

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
1	ascorbic acid	n/a	5/11/2009	Treatment of Charcot-Marie-Tooth disease type 1A.	Murigenetics SAS
2	budesonide	n/a	12/20/2006	Treatment of patients with eosinophilic esophagitis	Shire ViroPharma Incorporated
3	single chain urokinase plasminogen activator	n/a	9/11/2014	Treatment of empyema (pleural)	Lung Therapeutics, Inc.
4	(-)-(3aR,4S,7aR)-4-Hydroxy-4-m-tolylolethynyl-octahydro-indole-1-carboxylic acid methyl ester	n/a	10/12/2011	Treatment of Fragile X syndrome	Novartis Pharmaceuticals Corp.
5	(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-broethyl) diamidophosphate	n/a	6/5/2013	Treatment of pancreatic cancer	EMD Serono
6	(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate	n/a	3/9/2012	Treatment of soft tissue sarcoma	Threshold Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
7	(10R)-7-amino-12-fluoro-2,10,16-trimethyl-15 oxo-10,15,16,17-tetrahydro-2H-8,4-(metheno)pyrazolo[4,3-h][2,5,11]benzoxadiazacyclotetradecine-3-carbonitrile	n/a	6/23/2015	Treatment of anaplastic lymphoma kinase (ALK)-positive or ROS1-positive non-small cell lung cancer	Pfizer, Inc.
8	(1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexylpropanoate-	n/a	2/17/2009	Treatment of vaso-occlusive crisis in patients with sickle cell disease.	Pfizer, Inc.
9	(1S)-1-(9-deazahypoxanthin-9-yl)-1,4-dideoxy-1,4-imino-D-ribitol-hydrochloride	n/a	8/13/2004	Treatment of acute lymphoblastic leukemia	Mundipharma Research Limited
10	(1S)-1-(9-deazahypoxanthin-9-yl)-1,4-dideoxy-1,4-imino-D-ribitol-hydrochloride	n/a	1/29/2004	Treatment of T-cell non-Hodgkin's lymphoma	Mundipharma Research Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
11	(1S)-1-(9-deazahypoxanthin-9-yl)-1,4-dideoxy-1,4-imino-D-ribitol-hydrochloride	n/a	8/10/2004	Treatment of chronic lymphocytic leukemia and related leukemias to include prolymphocytic leukemia, adult T-cell leukemia, and hairy cell leukemia	Mundipharma Research Ltd.
12	(1S,3S)-3-amino-4-(difluoromethylene)cyclopentanecarboxylic acid hydrochloride, (1S,3S)-3-amino-4-(difluoromethylene)-1-cyclopentanoic acid hydrochloride	n/a	9/15/2010	Treatment of infantile spasms.	Catalyst Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
13	(2'R,3'S)-2'-hydroxy-N-carboxy-3'-amino-5'-methyl-hexanoic,N-tert-butyl ester, 13 ester 5B-20-epoxy-1B,2a,4a,7B,9a,10a,13a-heptahydroxy-4,10-diacetate-2-benzoate-(1"S)-7,9-acrolein acetal-11(15-1)-abeotaxane	n/a	6/23/2014	Treatment of progressive supranuclear palsy	Cortice Biosciences, Inc.
14	(2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1h-pyrazolo [4,3-c] pyridine-3,6(2h,5h)-dione)	n/a	10/14/2015	Treatment of systemic sclerosis.	Genkyotex Innovation SAS
15	(2E, 4E, 6Z, 8E)-3,7-dimethyl-9-(2,6,6-trimethylcyclohex-1-en-1-yl) nona-2,4,6,8-tetraen-1-yl acetate	n/a	12/2/2010	Treatment of retinitis pigmentosa	QLT Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
16	(2E,4E,6Z,8E)-3,7-dimethyl-9-(2,6,6-trimethylcyclohex-1-enyl)nona-2,4,6,8-tetraenyl acetate 9-cis-retinyl acetate (API)	n/a	12/2/2010	Treatment of Leber congenital amaurosis (LCA) due to inherited mutations in RPE65 (encoding the protein retinal pigment epithelial protein 65) or LRAT (encoding the enzyme lecithin:retinol acyltransferase) genes.	QLT, Inc.
17	(2S)-2-[[[(2R)-2-[[[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino]butanoic acid	n/a	10/31/2012	Treatment of progressive familial intrahepatic cholestatis	Albireo AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
18	(2S)-2-[[[(2R)-2-[[[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl]amino]-2-(4-hydroxyphenyl)acetyl]amino]butanoic acid	n/a	10/31/2012	Treatment of primary biliary cirrhosis	Albireo AB
19	(2Z)-2-cyano-3-hydroxy-N-[4-(trifluoromethyl)phenyl]-2-hepten-6-ynamide	Fk778	1/10/2005	Prevention of acute rejection following kidney, heart, and liver transplantation	Fujisawa Healthcare, Inc.
20	(3,5-Bis(trifluoromethyl)-N-[4-methyl-3-(4-pyridin-3-ylpyrimidin-2-ylamino)phenyl]benzamide	n/a	3/18/2011	Treatment of pancreatic cancer	NATCO Pharma Limited
21	(3,5-Bis(trifluoromethyl)-N-[4-methyl-3-(4-pyridin-3-ylpyrimidin-2-ylamino)phenyl]benzamide	n/a	3/18/2011	Treatment of chronic myelogenous leukemia.	NATCO Pharma Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
22	(3,5-Bis trifluoromethyl)- N-[4-methyl-3-(4- pyridin-3-yl- pyrimidin-2-yl amino) phenyl] benzamideNRC- AN-019	n/a	3/18/2011	Treatment of Glioma	NATCO Pharma Limited
23	(3E,5E)-3,5-bis[(4- fluoro-3- nitrophenyl)meth ylidene]-1-(prop- 2-enoyl)azepan-4- One	n/a	4/30/2014	Treatment of multiple myeloma	VivoLux AB
24	(3S)-1- azabicyclo[2.2.2] oct-3-yl {2-[2-(4- fluorophenyl)-1,3- thiazol-4- yl]propan-2- yl}carbamate	n/a	9/11/2014	Treatment of Gaucher disease	Genzyme
25	(3S)-1- azabicyclo[2.2.2]o ct-3-yl {2-[2-(4- fluorophenyl)-1,3- thiazol-4- yl]propan-2- yl}carbamate	n/a	8/26/2014	Treatment of Fabry's disease	Genzyme Corporation
26	(3S)-3-(4- trifluoromethoxy benzyloxy)-6- nitro-2H-3,4- dihydroimidazo[2 ,1-b]oxazine	n/a	7/5/2007	Treatment of tuberculosis	Global Alliance for TB Drug Development

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
27	(3S)-3-[(2S)-2-({N-[2-tert-butyl]phenyl]carbonyl}carbonylamino)propanoylamino]-4-oxo-5-(2,3,5,6-tetrafluorophenoxy) pentanoic acid	n/a	8/19/2003	Treatment of patients undergoing solid organ transplantatio n.	Pfizer Global Research and Development
28	(3S,4R)-3-ethyl-4-(3H-imidazo[1,2-a]pyrrolo[2,3-e]pyrazin-8-yl)-N-(2,2,2-trifluoroethyl)pyrrolidine-1-carboxamide (2R,3R)-2,3-dihydroxybutane dioate	n/a	9/18/2015	Treatment of pediatric (0 through 16 years of age) juvenile idiopathic arthritis (JIA) categories excluding systemic JIA	AbbVie, Inc.
29	(4R,5R)-1-[[4-[[4-[3,3-Dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy]methyl]phenyl]methyl]-4-aza-l-azoniabicyclo[2.2.2]octane Chloride	n/a	9/4/2013	Treatment of progressive familial intrahepatic cholestasis	Shire Human Genetic Therapies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
30	(4R,5R)-1-[[4-[[4-[3,3-Dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy)methyl]phenyl)methyl]-4-aza-l-azoniabicyclo[2.2.2]octane Chloride	n/a	9/4/2013	Treatment of primary sclerosing cholangitis	Shire Human Genetic Therapies, Inc.
31	(4R,5R)-1-[[4-[[4-[3,3-Dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy)methyl]phenyl)methyl]-4-aza-l-azoniabicyclo[2.2.2]octane Chloride	n/a	9/4/2013	Treatment of primary biliary cirrhosis	Shire Human Genetic Therapies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
32	(4R,5R)-1-[[4-[[4-[3,3-Dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy)methyl]phenyl)methyl]-4-aza-l-azoniabicyclo[2.2.2]octane Chloride	n/a	9/4/2013	Treatment of alagille syndrome	Shire Human Genetic Therapies, Inc.
33	(5R)-5-(4-{{2-fluorophenyl)methyl}oxy}phenyl)-L-prolinamide, hydrochloride	n/a	7/24/2013	Treatment of trigeminal neuralgia	Convergence Pharmaceuticals Ltd.
34	(6-[4-Deoxy-4-[[2E,4E)-tetradecadienoyl glycy] amino-L-glyceroB-L-manno-heptopyranosyl]amino-9H-purine)	n/a	2/21/2014	Parenteral treatment of painful, chronic, chemotherapy-induced peripheral neuropathy that is refractory to conventional analgesics	DARA BioSciences, Inc.
35	(6-maleimidocaproyl)hydrazone of doxorubicin	n/a	6/29/2011	Treatment of soft tissue sarcoma	CytRx Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
36	(6R,S)5,10-methylene-tetrahydrofolic acid	Cofactor	8/13/2004	For use in combination with 5-fluorouracil for the treatment of patients with pancreatic cancer	Adventrx Pharmaceuticals, Inc.
37	(6aR, 10aR)-3-(1',1'-dimethylheptyl)-delta8-tetrahydrocannabinol-9-carboxylic acid	n/a	10/13/2015	Treatment of cystic fibrosis.	Corbus Pharmaceuticals, Inc.
38	(6aR, 10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydrocannabinol-9-carboxylic acid	n/a	6/10/2015	Treatment of systemic sclerosis	Corbus Pharmaceuticals, Inc.
39	(9-[N-(3-morpholinopropyl)-sulfonyl]-5,6-dihydro-5-oxo-11H-indeno [1,2-c] isoquinoline methanesulfonic acid	n/a	12/8/2004	Prevention of post-operative complications of aortic aneurysm surgical repair	Inotek Pharmaceuticals Corporation
40	(E)-4-carboxystyryl-4-chlorobenzyl-sulfone, sodium salt	Ex-Rad(R)	6/1/2012	Prevention of acute radiation toxicity, also known as Acute Radiation Syndrome (ARS)	Onconova Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
41	(E)-4-carboxystyryl-4-chlorobenzyl-sulfone, sodium salt	Ex-Rad(R)	9/4/2012	Treatment of acute radiation syndrome	Onconova Therapeutics, Inc.
42	(N-[2,6-bis(1-methylethyl)-phey]-N'-[[1-4-dimethyl-amino)phenyl]cyclopentyl]methyl] urea, hydrochloride salt	n/a	3/9/2012	Treatment of adrenocortical carcinoma	Atterocor, Inc.
43	(R)-1-(2,2-difluorobenzo[d][1,3] dioxol-5-yl)-N-(1-(2,3-dihydroxypropyl)-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl) cyclopropanecarb oxamide	n/a	4/24/2014	Treatment of cystic fibrosis	Vertex Pharmaceuticals Inc.
44	(R)-1-phenylethyl-5-(4-biphenyl-4-cyclopropanecarboxylic acid)-3-methylisoxazole-4-yl carbamate sodium salt	n/a	4/15/2011	Treatment of idiopathic pulmonary fibrosis	Bristol-Myers Squibb Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
45	(R)-2-methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxy)methyl}-2,3-dihydroimidazo[2,1-b]oxazole	n/a	7/12/2007	Treatment of pulmonary tuberculosis.	Otsuka Pharmaceutical Company, Ltd
46	(R)-4-(3-morpholin-4-yl-1-phenylsulfanylmethyl-propylamino)-N-(4-{4-[2-(4-chlorophenyl)-5,5-dimethylcyclohex-1-enylmethyl]-piperazin-1-yl}-benzoyl)-3-trifluoromethanesulfonylbenzenesulfonamide bis-hydrochloride	n/a	12/19/2007	Treatment of small cell lung cancer.	AbbVie, Inc
47	(R)-N-[2-(6-Chloro-methoxy-1H-indol-3-yl)propyl]acetamide	n/a	7/3/2003	Treatment of neuroleptic-induced tardive dyskinesia in schizophrenia patients	Phase 2 Discovery, Inc.
48	(R)-N-[2-(6-chloro-5-methoxy-1H-indol-3-yl)propyl]acetamide	n/a	10/3/2001	Treatment of circadian rhythm sleep disorders in blind people with no light perception	Phase 2 Discovery, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
49	(RS)-baclofen, naltrexone and D-sorbitol	n/a	3/17/2014	Treatment of Charcot-Marie-Tooth disease type 1A	Pharnext SAS
50	(S)-1-(6,7-Dihydro-5H-benzo[6,7]cyclohepta[1,2-c]pyridazin-3-yl)-N3-(7-(pyrrolidin-1-yl)-6,7,8,9-tetrahydro-5H-benzo[7]annulen-2-yl)-1H-1,2,4-triazole-3,5-diamine	n/a	11/5/2014	Treatment of acute myeloid leukemia	BerGenBio AS
51	(S)-10-[[dimethylamino methyl]-4-ethyl-9hydroxy-4-O-[a-(2",4",5",7"-tetranitro-9"-fluorenylideneaminoxy)propionyl]-1H-pyrano[3',4',6',7',] indolizino [1,2-B]-quinoline-3,14-(4H, 12H)-dione, hydrochloride	Lipotecan	10/6/2010	Treatment of hepatocellular carcinoma	TLC Biopharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
52	(S)-2-(1-((6-amino-5-cyanopyrimidin-4-yl)amino)ethyl)-4-oxo-3-phenyl-3,4-dihydropyrrolo[2,1-f][1,2,4]triazine-5-carbonitrile	n/a	1/26/2015	Treatment of pemphigus vulgaris	Almirall S.A.
53	(S)-3-((3-(1-((6-((3,4-dimethoxyphenyl)pyrazin-2-yl)amino)ethyl)phenyl)carbonyl)-5-methylpyridin-1-ium	n/a	11/17/2014	Treatment of pulmonary arterial hypertension	Pulmokine, Inc.
54	(S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one	n/a	8/1/2013	Treatment of follicular lymphoma	Infinity Pharmaceuticals, Inc.
55	(S)-4,5-dihydro-2[2-hydroxy-3-(3,6,9-trioxadecyloxy)phenyl]-4-methyl-4-thiaxolecarboxylic acid	n/a	2/4/2009	Treatment of chronic iron overload in patients with transfusion-dependent anemias	Ferrokin BioSciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
56	(S)-4-(5-chloro-2-(isopropylamino)pyridin-4-yl)-N-(1-(3-chlorophenyl)-2-hydroxyethyl)-1H-pyrrole-2-carboxamide hydrochloride	n/a	6/24/2013	Treatment of Stage IIb through Stage IV BRAF mutant melanoma	BioMed Valley Discoveries, Inc.
57	(S)-4-(8-amino-3-(1-but-2-ynoylpyrrolidin-2-yl)-imidazo[1,5-a]pyrazin-1-yl)-N-(pyridin-2-yl)-benzamide	n/a	10/22/2015	Treatment of Waldenstrom macroglobulin emia.	Acerta Pharma BV
58	(S)-4-(8-amino-3-(1-but-2-ynoylpyrrolidin-2-yl)-imidazo[1,5-a]pyrazine-1-yl)-N-(pyridine-2-yl)-benzamide	n/a	9/21/2015	Treatment of mantle cell lymphoma.	Acerta Pharma BV
59	(S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride	n/a	11/17/2014	Treatment of inherited mitochondrial respiratory chain diseases	Khondrion BV
60	(S)-7-(1-(9H-purin-6-ylamino)ethyl)-6-(3-fluorophenyl)-3-methyl-5H-thiazolo[3,2-a]pyrimidin-5-one	n/a	4/29/2015	Treatment of Hodgkin lymphoma	Incyte Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	(S)-8-{2-Amino-6-[1-(5-chloro-biphenyl-2-yl)-(R)-2,2,2-trifluoroethoxy]-pyrimidin-4-yl}-2,8-diazaspiro[4.5]decane-3-carboxylic acid ethyl ester	n/a	10/6/2015	Treatment of pulmonary arterial hypertension (PAH).	KAROS Pharmaceuticals, Inc.
	(S)-N-(5-((R)-2-(2,5-difluorophenyl)pyrrolidin-1-yl)pyrazolo[1,5-a]pyrimidin-3-yl)-3-hydroxypyrrolidine-1-carboxamide hydrogen sulfate	n/a	8/31/2015	Treatment of soft tissue sarcoma.	Loxo Oncology, Inc.
	(S)-perillyl alcohol temozolomide	n/a	11/12/2014	Treatment of glioma	NeOnc Technologies, Inc.
	(UDU-stereoisomer of c-UJUun UNU-terminal UKUnhibitor)	n/a	3/28/2006	Treatment of acute sensorineural hearing loss	Auris Medical, Inc.
	(Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N-(pyrazin-2-yl)acrylohydrazide	n/a	5/14/2014	Treatment of diffuse large B-cell lymphoma	Karyopharm Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	(monomethoxy polyethylene glycol) recombinant adenosine deaminase	n/a	3/19/2015	Treatment of adenosine deaminase deficiency in patients with severe combined immunodeficiency	Sigma-Tau Pharmaceuticals, Inc.
	18-(p[131I]-iodophenyl)octadecyl phosphocholine	n/a	12/3/2014	Treatment of multiple myeloma	Collectar Biosciences, Inc.
	1'-{[5-(trifluoromethyl)-2-furyl]methyl}spiro[furo[2,3-f][1,3]benzodioxole-7,3'-indol]-2'(1'H)-one	n/a	11/19/2012	Treatment of erythromelalgia	Teva Pharmaceuticals
	1,2:5,6-Dianhydrogalactitol, NSC-132313	n/a	1/31/2012	Treatment of malignant gliomas	DelMar Pharmaceuticals (BC), Ltd.
	1,5-(Butylimino)-1,5 dideoxy,D-glucitol	n/a	5/12/1998	Treatment of Fabry's disease.	Oxford GlycoSciences
	1-(2-C-cyano-2-deoxy-B-D-arabino-pentafuranosyl)-N4-palmitoylcytosine	Sapacitabine	6/24/2010	Treatment of acute myelogenous leukemia	Cyclacel Limited
	1-(2-C-cyano-2-deoxy-B-D-arabino-pentafuranosyl)-N4-palmitoylcytosine	Sapacitabine	6/24/2010	Treatment of myelodysplastic syndrome	Cyclacel Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	1-(3-chloro-5-[[4-(4-chloro-2-thienyl)-5-(4-cyclohexylpiperazin-1-yl)-1,3-thiazol-2-yl]carbamoyl]-2-pyridyl) piperidine-4-carboxylic acid Monomaleate	n/a	9/1/2011	Treatment of idiopathic thrombocytopenic purpura	Eisai, Inc.
	1-(4-(benzylamino)-7,8-dihydro-5H-pyrano[4,3-d]pyrimidin-2-yl)-2-methyl-1H-indole-4-carboxamide	n/a	7/8/2015	Treatment of multiple myeloma	Cleave Biosciences, Inc.
	1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid	n/a	3/31/2015	Treatment of systemic sclerosis	Inventiva Pharma
	1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzimidazol-2-yl)-1H-pyrazol-4-yl]-urea	n/a	11/12/2009	Treatment of acute myeloid leukemia	Astex Therapeutics Ltd
	1-[(2-Chloro-4-methoxyphenoxy)methyl]-4-[(2,6-dichlorophenoxy)methyl]benzene	n/a	1/9/2012	Treatment of poliovirus infection.	ViroDefense, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	10 synthetic peptides targeting 5 tumor associated antigens	n/a	1/23/2013	Treatment of non-small cell lung cancer in patients expressing HLA-A2	Orphan Synergy Europe Pharma (OSE Pharma)
	111Indium pentetreotide	Somatother	6/10/1999	Treatment of somatostatin receptor positive neuroendocrine tumors.	Louisiana State University Medical Center Foundation
	12-A-p21 RAS(5-21). 12-C-p21 RAS(5-21). 12-D-p21 RAS(5-21). 12-Rp21 RAS(5-21). 12-S-p21 RAS(5-21). 12-V-p21 RAS(5-21). 13-D-p21 RAS(5-21)	n/a	6/7/2011	Treatment of pancreatic cancer	Oncos Therapeutics
	17 amino acid peptide	n/a	1/29/2010	Prevention of ischemia reperfusion injury in the lung during lung transplantation	Apeptico Forschung und Entwicklung GmbH
	17-a-hydroxyprogesterone caporate (oral formulation)	n/a	6/1/2015	Prevention of preterm birth in women with a singleton pregnancy	Lipocine, Inc.
	17-allylamino-17-demethoxygeldanamycin (17-AGG)	n/a	9/3/2004	Treatment of chronic myelogenous leukemia	Bristol-Myers Squibb Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	177-LU-DOTA-GlyGlyNle-CycMSHhex	n/a	4/16/2015	Treatment of Stage IIB through IV malignant melanoma	SolaranRx, Inc.
	177Lu-tetraxetan-tetulumab	Betalutin	5/14/2014	Treatment of Follicular Lymphoma	Nordic Nanovector AS
	18-(p-[124I]-iodophenyl)octadecyl phosphocholine	n/a	4/30/2014	Diagnostic for the management of glioma	Collectar Biosciences, Inc.
	18Fluorine-N-3-Fluoropropyl-2beta-carbomethoxy-3beta-(4-iodophenyl) Nortropane	n/a	1/26/2015	Diagnostic to be used in the management of multiple system atrophy (MSA)	Advanced Imaging Projects, LLC
	2 dimethylbutyrate	n/a	6/18/2008	Treatment of beta thalassemia	HemaQuest Pharmaceuticals, Inc.
	2'-O-methyl phosphorothioate 5'-GCUAGGUUUAC GGGACCUCU-3'	n/a	10/31/2012	Treatment of amyotrophic lateral sclerosis	LifeSplice Pharma LLC
	2'-deoxycytidine	n/a	9/9/1996	As a host-protective agent in the treatment of acute myelogenous leukemia.	Grant, Steven M.D.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2,2'-(2-[1R]-1-(((2,5-dichlorobenzoyl)amino)acetyl)amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid (ixazomib citrate)	n/a	3/9/2012	Treatment of systemic light chain (AL) amyloidosis.	Millennium Pharmaceuticals, Inc.
	2,5-dimethyl-3-[2-methyl-4-(methoxy)phenyl]-N-[(1S)-1-(3-methyl-1,2,4-oxadiazol-5-	n/a	1/15/2015	Treatment of congenital adrenal hyperplasia (CAH)	Neurocrine Biosciences, Inc.
	2-(2-chlorobenzylidene)hydrazinecarboximidamide acetate	n/a	9/10/2015	Treatment of Charcot-Marie Tooth disease.	InFlectis BioScience
	2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione	n/a	9/21/2010	Treatment of idiopathic pulmonary fibrosis.	GenKyoTex S.A.
	2-(2-phenylvinyl)-4-[-methylpiperazin-1-yl]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+)-tartrate salt	n/a	1/29/2010	Treatment of acute myeloid leukemia.	EntreMed, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt	n/a	7/1/2014	Treatment of hepatocellular carcinoma	CASI Pharmaceuticals, Inc.
	2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt	n/a	12/22/2008	Treatment of multiple myeloma.	EntreMed, Inc.
	2-(2-phenylvinyl)-4-[4-methylpiperazin-1-yl]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt	n/a	3/18/2009	Treatment of ovarian carcinoma	EntreMed, Inc.
	2-(3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-1-(1-(3-fluoro-2-(trifluoromethyl)isonicotinoyl)pipерidin-4-yl)azetid-3-yl)acetonitrile adipate	n/a	1/26/2015	Treatment of pancreatic cancer	Incyte Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-(3-Diethylaminopropyl)-8,8-dipropyl-2-azaspiro[4,5]decane dimaleate	Atiprimod	9/18/2006	Treatment of carcinoid tumors	Callisto Pharmaceuticals, Inc.
	2-(3-diethylaminopropyl)-8,8-dipropyl-2-azaspiro [4,5] decan dimaleate	Atiprimod	12/2/2003	Treatment of mulitple myeloma and associated bone resorption	Callisto Pharmaceuticals, Inc.
	2-(5-fluoro-2-methyl-1H-indol-3-yl)-1H-imidazo[4,5-f][1,10]phenanthroline	n/a	6/1/2015	Treatment of acute myeloid leukemia	Aptose Biosciences, Inc.
	2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide	n/a	5/13/2015	Treatment of cystic fibrosis	Flatley Discovery Lab
	2-O-Butyryl-1-O-octyl-myo-inositol 3,4,5,6-tetrakisphosphate	n/a	8/15/2003	Treatment of cystic fibrosis	Inologic, Inc.
	2-O-desulfated heparin	Aeropin	9/17/1993	Treatment of cystic fibrosis.	Kennedy & Hoidal, M.D.'s

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-Chloro-N6--(3-iodobenzyl)adenosine-5'-N-methyluronamide	n/a	2/17/2012	Treatment of hepatocellular carcinoma	Can-Fite BioPharma Ltd.
	2-Ethylbutyl (2S)-2-(((S)-{[(2R,3S,4R,5R)-5-(4-aminopyrrolo[2,1-f][1,2,4]triazin-7-yl)-5-cyano-3,4-dihydroxytetrahydrofuran-2-yl]methoxy}(phenoxy)phosphoryl)amino}propanoate	n/a	9/18/2015	Treatment of Ebola virus disease	Gilead Sciences, Inc.
	2-Pyrazinecarbonitrile, 5-[[5-2-(3-aminopropoxy)-6-methoxyphenyl]-1H-pyrazol-3-yl]amino] monomesylate monohydrate	n/a	4/9/2015	Treatment of anal cancer	Eli Lilly and Company
	2-[(3-methyl-4-(2,2,2-trifluoroethoxy)pyridin-2-yl)methylsulfinyl]-1H-benzimidazole	Prevonco (Tm)	8/27/2008	Treatment of hepatocellular carcinoma.	BioQuant, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-[(4S)-6-(4-chlorophenyl)-1,7,8-trimethylthiophenol[3,2-f]1,2,4-triazolo[4,3-a]1,4-diazepin-4-yl]-N-[3-(4-methylpiperazinyl)propyl]acetamide	n/a	9/29/2015	Treatment of nuclear protein in testis (NUT) midline carcinoma.	Tensha Therapeutics, Inc.
	2-[(6S)-4-(4-chlorophenyl)-2,3,9-trimethyl-6H-thienol[3,2,-f]-[1,2,4]triazolo[4,3-a][1,4]diazepin-6-yl]-N-(4-hydroxyphenyl)-acetamide dihydrate	n/a	7/16/2014	Treatment of acute myeloid leukemia	OncoEthix SA
	2-[(R)-2-methylpyrrolidin-2-yl]-1H-benzimidazole-4-carboxamide	n/a	12/18/2007	Treatment of malignant melanoma stages IIb through IV.	AbbVie, Inc.
	2-[4-(1-Methyl-4-pyridin-4-yl-1H-pyrazol-3-yl)-phenoxy-methyl]-quinoline succinic acid	n/a	6/2/2014	Treatment of Huntington's disease.	Pfizer Inc.
	2-[4-Methoxy-3-(2-m-tolylethoxy)-benzoylamino]-indian-2-carboxylic acid	n/a	5/14/2013	Treatment of patients with systemic sclerosis	Sanofi U. S., Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-[4-[[[(2-R)-2-ethoxy-3-[4-(trifluoromethyl)phenoxy]propyl]thio]-2-methylphenoxy]acetic acid (1:1) lysine dihydrate	n/a	4/15/2015	Treatment of patients with Frederickson Type I or V hyperlipoproteinemia	CymaBay Therapeutics, Inc.
	2-[4-[[[(2R)-2-ethoxy-3-[4-(trifluoromethyl)phenoxy]propyl]thio]-2-methylphenoxy]acetic acid (1:1) lysine dihydrate	n/a	3/18/2015	Treatment of homozygous familial hypercholesterolemia (HoFH)	CymaBay Therapeutics, Inc.
	2-[[[3-[[4-[[5-[[2-[[3-Fluorophenyl]amino]-2-oxoethyl]-1H-pyrazol-3-yl]amino]-quinazolin-7-yl]propyl](ethyl)amino]ethyl dihydrogen phosphate trihydrate	n/a	1/19/2010	Treatment of acute myeloid leukemia	AstraZeneca Pharmaceuticals LP
	2-amino-2-[2-[2-chloro-4-[[3-(phenylmethoxy)phenyl]thio]phenyl]ethyl]1,3-propanediol hydrochloride	n/a	10/6/2015	Prevention of graft versus host disease.	Novartis Pharmaceuticals Corporation
	2-chloroethyl-3-sarcosinamide-1-nitroso-urea	Sarmustine	11/15/2001	Treatment for malignant glioma	Pangene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-chloroethyl-3-sarcosinamide-1-nitrosourea	n/a	8/3/2001	Treatment for malignant gliomas	Lawrence Panasci, MD
	2-hydroxypropyl-β-cyclodextrin	Kleptose	2/18/2013	Treatment of Niemann Pick disease, type C.	Vtesse, Inc.
	2-iminobiotin	n/a	2/24/2009	Treatment of perinatal asphyxia.	Neurophyxia B.V.
	2-methoxyestradiol	Pulmolar	4/11/2005	Treatment of pulmonary arterial hypertension	PR Pharmaceuticals, Inc.
	2-{3-[(3R)-3-[[2-chloro-3-(trifluoromethyl)phenyl]methyl](2,2-diphenylethyl)amino]butoxy}phenyl}acetic acid	n/a	6/23/2015	Treatment of malignant melanoma stages IIB to IV.	Rgenix, Inc.
	2-{3-[(3R)-3-[[2-chloro-3-(trifluoromethyl)phenyl]methyl](2,2-diphenylethyl)amino]butoxy}phenyl}acetic acid	n/a	6/16/2015	Treatment of ovarian cancer.	Rgenix, Inc.
	2-{3-[(3R)-3-[[2-chloro-3-(trifluoromethyl)phenyl]methyl](2,2-diphenylethyl)amino]butoxy}phenyl}acetic acid	n/a	6/16/2015	Treatment of glioblastoma multiforme.	Rgenix, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	2-{{(1r,4r)-4-((4-chlorophenyl)(phenyl)carbamoxy)methyl)cyclohexy]methoxy} acetic acid	n/a	8/28/2014	Treatment of pulmonary arterial hypertension	Arena Pharmaceuticals, Inc.
	20-mer complementary to Akt mRNA	n/a	12/10/2004	Treatment of stomach cancer	Rexahn Corporation
	20-mer oligonucleotide complementary to Akt mRNA	n/a	12/8/2004	Treatment of glioblastoma	Rexahn Corporation
	20-mer oligonucleotide complementary to Akt mRNA	n/a	12/1/2004	Treatment of renal cell carcinoma	Rexahn Corporation
	20-mer oligonucleotide complementary to Akt mRNA	n/a	12/1/2004	Treatment of ovarian cancer	Rexahn Corporation
	20-mer oligonucleotide complementary to Akt mRNA	n/a	12/8/2004	Treatment of pancreatic cancer	Rexahn Corporation
	225Ac-lintuzumab	n/a	11/25/2014	Treatment of acute myelogenous leukemia	Actinium Pharmaceuticals, Inc.
	24,25 dihydroxycholecalciferol	n/a	2/27/1987	Treatment of uremic osteodystrophy.	Lemmon Company
	2S,4R ketoconazole	n/a	3/9/2012	Treatment of endogenous Cushing's syndrome	Cortendo AB (HQ address)

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	3,4-diaminopyridine and choline bitartrate	n/a	4/30/2010	Treatment of Lambert-Eaton myasthenic syndrome	MS Therapeutics Limited
	3,4-diaminopyridine	n/a	12/18/1990	Treatment of Lambert-Eaton myasthenic syndrome.	Jacobus Pharmaceutical Company
	3,5,3'-triiodothyroacetate	n/a	9/20/2000	Treatment of well-differentiated papillary, follicular or combined papillary/follicular carcinomas of the thyroid gland.	Elliot Danforth, Jr., M.D.
	3,5-diiodothyropropionic acid	n/a	5/14/2013	Treatment of Allan-Herndon-Dudley syndrome	Zarion Pharmaceuticals P/L
	3-(3,5-Dimethyl-1H-2ylmethylene)-1,3-dihydro-indol-2-one	n/a	3/23/2000	Treatment of von Hippel-Lindau disease.	Sugen, Inc.
	3-(3,5-dimethyl-1H-2ylmethylene)-1,3-dihydro-indol-2-one	n/a	9/11/1998	Treatment of Kaposi's sarcoma.	Sugen, Inc.
	3-(4-(1,5-Naphthyridine)-Imidazole[1,2-a]pyridine-7-(1-phenyl-4-(1-(4-methyl piperazine)	n/a	7/14/2015	Treatment of fibrodysplasia ossificans progressiva.	La Jolla Pharmaceutical Company, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	3-(4-(8-fluoroquinoline)-imidazole[1,2-a]pyridine-7-(1-phenyl-4-(1-(4-methylpiperazine)	n/a	7/14/2015	Treatment of fibrodysplasia ossificans progressiva.	La Jolla Pharmaceutical Company, Inc.
	3-(6-(1-(2,2-difluorobenzo[d][1,3]dioxyl-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid	n/a	3/2/2010	Treatment of cystic fibrosis	Vertex Pharmaceuticals Inc.
	3-[2-(4-carbamimidoyl-phenylcarbamoyl)-5-methoxy-4-vinyl-phenyl]-6-(cyclopropylmethyl-carbamoyl)-pyridine-2-carboxylic acid	n/a	12/23/2014	Prevention of acute attacks of angioedema in individuals with hereditary angioedema	BioCryst Pharmaceuticals, Inc.
	3-bromopyruvate	n/a	4/29/2014	Treatment of pancreatic cancer	PreScience Labs, LLC
	3-bromopyruvate	n/a	3/5/2013	Treatment of liver and intrahepatic bile duct cancer	Primocure Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	3-chloro-4-fluorophenyl-[4-fluoro-4-[[5-methylpyrimidin-2-yl(methyl)amino]methyl]piperidin-1yl]methanone	n/a	10/25/2013	Treatment of Rett syndrome	Neurolix, Inc.
	3-fluoro-5-[5-(2-menthyl-thiazol-4-ylethyl)pyridin-2-yl]benzotrile dihydrochloride	n/a	7/28/2008	Treatment of behavioral abnormalities associated with fragile X syndrome.	Seaside Therapeutics
	3-pentylbenzenetic acid sodium salt	n/a	2/11/2015	Treatment of idiopathic pulmonary fibrosis.	ProMetic Life Sciences, Inc.
	3-{3-[4-(1-aminocyclobutyl)phenyl]-5-phenyl-3H-imidazo[4,5-b]pyridin-2-yl}pyridin-2-amine bis-mesylate	n/a	11/9/2015	Treatment of Proteus Syndrome.	ArQule, Inc.
	4, 5 dibromorhodamine methyl ester	Theralux Photodynamic Therapy	10/30/2008	Treatment of follicular lymphoma	Kiadis Pharma Canada, Inc.
	4, 5 dibromorhodamine methyl ester	Theralux Photodynamic Therapy	10/30/2008	Treatment of diffuse large B-cell lymphoma	Kiadis Pharma Canada, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4,5 dibromorhodami ne methyl ester	Theralux Photodynamic Therapy	10/23/2007	For immune reconstitution and prevention of graft versus host disease following allogeneic hematopoietic stem cell transplantatio n.	Kiadis Pharma Netherlands B.V.
	4,5,6,7- tetrachloro- 2',4',5',7'- tetraiodofluoresc ein disodium salt	n/a	4/18/2011	Treatment of hepatocellular carcinoma.	Provectus Pharmaceuticals, Inc.
	4-(2- fluorophenyl)-6- methyl-2-(1- piperziny)	n/a	10/11/2006	Treatment of Chronic Functional Vomiting to include functional vomiting and cyclic vomiting syndrome.	Dynogen Pharmaceuticals, Inc.
	4-(3- Methanesulfonyl- phenyl)-1- propylpiperidine HCl	n/a	12/12/2005	Treatment of Huntington's disease.	Teva Branded Pharmaceutical Products R&D
	4-(4-Methoxy- phenylamino)-6- methylcarbamy- quinoline-3- carboxylic acid	n/a	3/24/2015	Prevention of scarring post ab externo glaucoma surgery	Clanotech AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4-(4-{[2-(4-chlorophenyl)-4,4-dimethylcyclohex-1-en-1-yl]methyl}piperazin-1-yl)-N-({3-nitro-4-[(tetrahydro-2H-pyran-4-ylmethyl)amino]phenyl)sulfonyl)-2-(1H-pyrrolo[2,3-b]pyridin-5-yloxy)benzamide	n/a	3/27/2014	Treatment of diffuse large B-cell lymphoma.	AbbVie
	4-(4-{[2-(4-chlorophenyl)-4,4-dimethylcyclohex-1-en-1-yl]methyl}piperazin-1-yl)-N-({3-nitro-4-[(tetrahydro-2H-pyran-4-ylmethyl)amino]phenyl)sulfonyl)-2-(1H-pyrrolo[2,3-b]pyridin-5-yloxy)benzamide	n/a	9/20/2012	Treatment of chronic lymphocytic leukemia	AbbVie, Inc
	4-(6-(4-(piperazin-1-yl)phenyl)pyrazolo[1,5-a]pyrimidin-3-yl)quinoline hydrochloride	n/a	4/15/2013	Treatment of fibrodysplasia ossificans progressiva	La Jolla Pharmaceutical Company, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4-(pyrimidin-2-ylmethyl)-7-[4-(trifluoromethoxy)phenyl]-3,4-dihydro-1,4-benzoxazepin-5(2H)-one	n/a	5/4/2015	Treatment of congenital long QT syndrome	Gilead Sciences, Inc.
	4-Amino-1-[5-O-[(2R, 4S)-2-oxido-4-(4-pyridinyl)-1,3,2-dioxaphosphorinane-2-yl]-b-D-arabinofuranosyl]-2(1H)-pyrimidinone	n/a	8/22/2007	Treatment of hepatocellular carcinoma.	Ligand Pharmaceuticals, Inc.
	4-Aminopyridine	n/a	12/14/2005	Treatment chronic functional motor and sensory deficits from Guillain-Barre syndrome	Acorda Therapeutics, Inc.
	4-Hydroxy-2,2,6,6-tetramethylpiperidine-N-oxyl	n/a	9/29/2015	Treatment of cerebral cavernous malformation.	REursion Pharmaceuticals, LLC
	4-[(7-Methoxy-2,3-dihydro-1,4-benzothiazepin-4(5H)-yl)methyl]benzoic acid, hemifumarate	n/a	11/18/2015	Treatment of patients with Duchenne Muscular Dystrophy.	ARMGO Pharma, Inc.
	4-[131I]iodo-L-phenylalanine	n/a	1/4/2011	Treatment of glioma.	Therapeia GmbH & Co KG

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4-[1]benzofuro[3,2-d]pyrimidin-4-yl-N-(1,3-benzodioxol-5-ylmethyl)piperazine-1-carbothioamide	n/a	7/30/2008	Treatment, in combination with radiotherapy and temozolomide chemotherapy, of patients with glioblastoma multiforme.	SuperGen, Inc.
	4-[2-(3-Propyl-[1,2,4]oxadiazol-5-yl)-vinyl]-benzene-1,2-diol	n/a	10/4/2011	Treatment of chronic myeloid leukemia	Piramal Enterprises Limited
	4-[4-[[[3-(1,1-dimethylethyl)-1-(6-quinolinyl)-1H-pyrazol-5-yl]amino]carbonyl]-amino]-3-fluorophenoxy]-N-methyl-2-pyridinecarboxamide, p-toluenesulfonate salt	n/a	9/3/2009	Treatment of Philadelphia chromosome positive chronic myeloid leukemia	Deciphera Pharmaceuticals, LLC
	4-[[9-(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol	n/a	9/23/2011	Treatment of idiopathic pulmonary fibrosis	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4-amino-1- [(1S,4R,5S)-2- fluoro-4,5- dihydroxy-3- (hydroxymethyl) cyclopent-2-en-1- yl] pyrimidin-2- one	n/a	9/23/2014	Treatment of pancreatic cancer.	Rexahn Pharmaceuticals, Inc.
	4-aminosalicylic acid	Pamisyl (P-D), Rezipas (Squibb)	12/13/1989	Treatment of mild to moderate ulcerative colitis in patients intolerant to sulfasalazine.	Beeken, Warren M.D.
	4-aminosalicylic acid	Paser Granules	4/26/2006	Treatment of acute flares in pediatric patients with ileo-cecal Crohn's disease	Jacobus Pharmaceutical Co., Inc.
	4-cyano-N-[2-(1- cyclohexen-1-yl)- 4-[1- [dimethylamino)a cetyl]-4- piperidinyl]pheny l]-1H-imidazole-2- carboxamide monohydrochlori de	n/a	7/20/2006	Treatment of acute myeloid leukemia	Johnson & Johnson Pharmaceutical Research & Dev,
	4-pyridinylmethyl 3(4- chlorophenyl)ada mantine carboxamide	n/a	11/2/2010	Treatment of pancreatic cancer.	Apogee Biotechnology Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	4-[[[(2R,3S,4R,5S)-4-(4-Chloro-2-fluoro-phenyl)-3-(3-chloro-2-fluoro-phenyl)-4-cyano-5-(2,2-dimethyl-propyl)-pyrrolidine-2-carbonyl]-amino]-3-methoxy-benzoic acid	n/a	5/19/2014	Treatment of Acute Myeloid Leukemia	Roche Genentech
	40K PEGylated recombinant factor IX	n/a	3/18/2013	Routine prophylactic administration for prevention of bleeding in patients with hemophilia B (Christmas disease)	Novo Nordisk, Inc.
	5'-CTATCTGUC*G1T TCTCTGU-3'.17 Na+	n/a	3/31/2015	Treatment of diffuse large B-cell lymphoma.	Idera Pharmaceuticals, Inc.
	5'-GCCATGGTTTTTT CTCAGG-3'	n/a	10/31/2012	Prophylaxis for patients following documented or suspected exposure to ebolavirus	Sarepta Therapeutics, Inc.
	5(S)-(2'-hydroxy ethoxy)-20(S)-Camptothecin	n/a	6/15/2007	Treatment of osteosarcoma (bone cancer)	Dr. Reddy's Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	5,5',5''-[Phosphinothioylidene-tris(imino-2,1-ethanediyl)]tris[5-methylchelonium]trihydroide hexahydrochloride	n/a	8/20/2003	Treatment of pancreatic cancer	Now Pharm AG
	5,6-dihydro-5-azacytidine	n/a	5/11/1992	Treatment of malignant mesothelioma.	ILEX Oncology, Inc.
	5,7-dichloro-2-dimethylaminoethyl-8-hydroxyquinoline hydrochloride	n/a	9/4/2014	Treatment of Huntington's disease	Prana Biotechnology Limited
	5,7-dihydroxy-3-(4-hydroxyphenyl)-chromen-4-one	n/a	6/18/2007	Prevention of acute radiation syndrome	Humanetics Corporation
	5-(3-ethyl-1,2,4-oxadiazol-5-yl)-1,4,5,6-tetrahydropyrimidine hydrochloride	n/a	4/18/2011	Treatment of progressive supranuclear palsy.	Mithridion, Inc.
	5-(4-Cyclopropyl-1H-imidazol-1-yl)-2-fluoro-N-(6-(4-isopropyl-4H-1,2,4-triazol-3-yl)pyridin-2-yl)-4-methylbenzamide	n/a	1/15/2015	Treatment pulmonary arterial hypertension	Gilead Sciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzop[d]oxazole	n/a	11/22/2011	Treatment of Duchenne Muscular Dystrophy	Summit Corporation plc
	5-[(E)-2-(4-hydroxyphenyl)-ethenyl] benzene-1,3 diol	Resveratrol	3/13/2008	Treatment of MELAS syndrome	Sirtris Pharmaceuticals, Inc.
	5-[1-(2,6-Dichlorobenzyl)-piperidin-4-yl]methoxyquinazoline-2,4-diamine	n/a	8/25/2009	Treatment of spinal muscular atrophy	Pfizer Incorporated
	5-[8-methyl-9-(1-methylethyl)-2-(4-morpholinyl)-9H-purin-6yl]-2-pyrimidinamine	n/a	2/10/2015	Treatment of malignant mesothelioma	Verastem, Inc.
	5-aminolevulinic acid	Gliolan	1/15/2013	Visualization of malignant tissue during surgery for malignant glioma (WHO grade III and IV)	NX Development Corporation
	5-aza-2'-deoxycytidine	n/a	8/3/1987	Treatment of acute leukemia.	SuperGen, Inc.
	5-hydroxymethyl-2-furfuraldehyde	n/a	5/26/2006	Treatment of sickle cell disease	Baxalta US, Inc.
	5-iodo-2-pyrimidinone-2'-deoxyribose	n/a	5/26/2006	Treatment of malignant glioma	Hana Biosciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	5'-(3'-phosphoryl)-2'-deoxyadenosine 3',5'-bisphosphate GAATATTAACAIA CTGACAAGTC- 3'-phosphoryl-2'-deoxyadenosine 3',5'-bisphosphate	n/a	10/31/2012	Prophylaxis following documented or suspected exposure to marburg virus	Sarepta Therapeutics, Inc.
	6,8-bis-benzylsulfanyl-octanoic acid	n/a	2/6/2006	Treatment of pancreatic cancer	Cornerstone Pharmaceuticals, Inc.
	6,8-bis-benzylsulfanyl-octanoic acid	n/a	9/26/2013	Treatment of myelodysplastic syndrome	Cornerstone Pharmaceuticals, Inc.
	6,8-bis-benzylsulfanyl-octanoic acid	n/a	8/22/2011	Treatment of acute myeloid leukemia.	Cornerstone Pharmaceuticals, Inc.
	6-((3S,4S)-4-Methyl-1-pyrimidin-2-ylmethyl-pyrrolidin-3-yl)-1-(tetrahydropyran-4-yl)-1,5-dihydro-1H-pyrazolo[3,4-d]pyrimidin-4-one	n/a	9/4/2014	Treatment of sickle cell disease	Pfizer, Inc.
	6-(1H-Indazol-6-yl)-N-(4-morpholinophenyl)imidazo-[1,2-alpha]pyrazin-8-amine bis-methanesulfonate	n/a	4/14/2014	Treatment of chronic lymphocytic leukemia	Gilead Sciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	6-[4-Deoxy-4- [(2E,4E)- tetradecadienoyl glycyl]amino-L- glycero-B-L- manno- heptopyranosyl]a mino-9H-purine	n/a	6/12/2014	Treatment of multiple myeloma	DARA BioSciences, Inc.
	6-alpha- ethylchenodeoxy cholic acid	n/a	4/9/2008	Treatment of primary sclerosing cholangitis	Intercept Pharmaceuticals, Inc.
	6-amino-5-chloro- N-[1R)-1-[5-[[[5- hloro-4- (trifluoromethyl)- 2pyridinyl]amino] carbonyl]-2- thiazoyl]ethyl]-4- pyrimidinecarbox amide	n/a	10/15/2013	Treatment of stage IIb-IV melanoma	Millennium Pharmaceuticals, Inc.
	6-ethoxy-7- methoxy-2-(2- methylsulfanylph enyl)-3,1- bensoxazin-4-one	n/a	6/18/2015	Treatment of Netherton syndrome	Sixera Pharma AB
	6- mercaptapurine oral liquid	n/a	12/7/2009	Treatment of acute lymphoblastic leukemia in the pediatric population	Orbona Pharma Ltd
	68G-OPS202, small somatostatin analog labeled with 68Gallium	n/a	9/24/2014	Management of gastroenterop ancreatic neuroendocrin e tumors.	OctreoPharm Sciences GmbH

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	7-B-Hydroxy Cholesteryl-3-B- Oleate-Ester	n/a	6/15/2011	Treatment of gliomas.	Intsel Chimos SA
	8-(1-Hydroxy- ethyl)-2-methoxy- 3-(4-methoxy- benzyloxy)- benzo[c]chromen- 6-one	n/a	1/21/2015	Treatment of glioblastoma multiforme	RestorGenex Corporation
	8-[4-(1- aminocyclobutyl) phenyl]-9-phenyl- 1,2,4-triazolo[3,4- f][1,6]naph-thyrin 3(2H)-one mono- hydrochloride	n/a	9/3/2009	Treatment of ovarian cancer.	Merck Sharp & Dohme Corp.
	8-methoxsalen	Uvadex	6/22/1993	For use in conjunction with the UVAR photopheresis to treat diffuse systemic sclerosis.	Therakos, Inc.
	9-nitro-20-(S)- camptothecin	Camvirex	5/15/2001	Treatment of pediatric HIV infection/AIDS	NovoMed Pharmaceuticals, Inc.
	9-nitro-20-(S)- camptothecin	n/a	9/16/1996	Treatment of pancreatic cancer.	SuperGen, Inc.
	90Y-hPAMA4	Pan-Cide	1/29/2004	Treatment of pancreatic cancer	Immunomedics, Inc.
	A-dmDT390- bisFv(UCHT1)	Resimmune	10/9/2014	Treatment of cutaneous T- cell lymphoma	Angimmune, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	A10 & AS2-1 Antineoplaston	n/a	9/3/2004	Treatment for patients with brain stem glioma	Burzynski Research Institute, Inc.
	AAV-G6Pase vector	n/a	3/11/2013	Treatment of glycogen storage disease type Ia	GlyGenix Therapeutics, Inc.
	ABCA4 DNA nanoparticles	n/a	10/28/2015	Treatment of Stargardt Macular Degeneration	Copernicus Therapeutics, Inc.
	ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS; HLA class I/II binding tumor associated peptides	n/a	9/28/2012	Treatment of renal cell carcinoma in HLA-A*2 positive patients	Immatics Biotechnologies GmbH
	ADXS11-001	n/a	8/12/2013	Treatment of HPV-positive associated anal cancer	Advaxis, Inc.
	AEZS-108 (LHRH-agonist linked to doxorubicin)	n/a	4/30/2010	Treatment of ovarian cancer	Aeterna Zentaris
	ALX-0081 nanobody, directed towards the human A1 domain of von Willebrand factor	n/a	4/14/2009	Treatment of thrombotic thrombocytopenic purpura	Ablynx NV
	ANA-conjugated dactinomycin nanoemulsion	n/a	7/7/2015	Treatment of Ewing sarcoma	NanoSmart Pharmaceuticals, Inc.
	AQ-13 (4-aminoquinoline analog)	n/a	9/12/2008	Treatment of malaria	Immtech Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Abetimus	n/a	7/28/2000	Treatment of lupus nephritis.	La Jolla Pharmaceutical Co.
	Ac-Ile-Cys-Val-Trp(1-Me)-Gln-Asp-Trp-Gly-Ala-His-Arg-Cys-Thr-AEEA-Lys-PEG 40KDa-Lys-AEEA-Thr-Cys-Arg-His-ALa-Gly-Trp-Asp-Gln-(1-Me)Trp-Val-Cys-Ile-Ac	n/a	4/20/2014	Treatment of paroxysmal nocturnal hemoglobinuria	Apellis Pharmaceuticals, Inc.
	Acetylcysteine	Mucomyst/Mucomyst 10 lv	8/13/1987	Intravenous treatment of patients presenting with moderate to severe acetaminophen overdose.	Bristol-Myers Squibb Company
	ActRIIB-IgG1)	n/a	8/16/2010	Treatment of Duchenne muscular dystrophy	Acceleron Pharma, Inc.
	Ad5/3-D24-granulocyte-macrophage colony stimulating factor (GMCSF)-encoding oncolytic adenovirus	n/a	3/17/2014	Treatment of ovarian cancer	Oncos Therapeutics
	Adeno-Associated Viral Vector Expressing Low-Density Lipoprotein Receptor	n/a	1/31/2012	Treatment of homozygous familial hypercholesterolemia	ReGenX Biosciences LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Adeno-associated vector expressing the human lipoprotein lipase protein	n/a	5/21/2007	Treatment of lipoprotein lipase deficiency	uniQure B.V.
	Adeno-associated viral vector containing the gene for human coagulation factor IX	Coagulin-B	6/13/2001	Intrahepatic treatment of patients with moderate to severe hemophilia	Avigen, Inc.
	Adeno-associated viral vector containing the gene for human coagulation factor IX	Coagulin-B	6/13/2001	Intramuscular treatment of patients with moderate to severe hemophilia	Avigen, Inc.
	Adeno-associated viral vector expressing human acid alpha glucosidase gene	n/a	3/20/2007	Treatment of Pompe disease	Applied Genetic Technologies Corporation
	Adeno-associated virus serotype rh10 vector encoding the human factor IX gene	n/a	8/25/2015	Treatment of hemophilia B.	Dimension Therapeutics

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Adenoassociated virus vector (AAV) carrying a modified AAV serotype 2 backbone and coding sequence of human thymidine phosphorylase preceded by a human thyroxin-binding globulin promoter	n/a	9/4/2014	For the treatment of Mitochondrial Neurogastrointestinal Encephalomyopathy (MNGIE)	Columbia University Medical Center
	Adenosine triphosphate type 1 competitive inhibitor of JAK2 V617F tyrosine kinase	n/a	3/3/2011	Treatment of myeloproliferative disorders (polycythemia vera, essential thrombocythemia, and myelofibrosis)	Eli Lilly and Company
	Adenoviral vector expressing Herpes simplex virus thymidine kinase gene	n/a	6/17/2005	Treatment of malignant brain tumors	Advantagene, Inc.
	Adenoviral vector-RheoSwitch Therapeutic System-human interleukin 12 + veledimex	n/a	7/23/2015	Treatment of malignant glioma.	Ziopharm Oncology, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Adenovirus serotype-5 (Ad5) vector that contains a modified non-oncogenic fused early 6 (E6) and early 7 (E7) gene of the human papillomavirus (HPV); (Ad5 [E1-, E2b-]-E6/E7)	n/a	9/4/2014	Treatment of human papillomavirus (HPV)-associated head and neck squamous cell carcinoma (HNSCC)	Etubics Corporation
	Adenovirus-based vector Factor VIII complementary DNA to somatic cells	Miniadviii	12/15/1999	Treatment of hemophilia A.	GenStar Therapeutics Corporation
	Albendazole	Albenza	1/17/1996	Treatment of hydatid disease (cystic echinococcosis due to E. granulosus larvae or alveolar echinococcosis due to E. multilocularis larvae).	Amedra Pharmaceuticals LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Albendazole	Albenza	1/18/1996	Treatment of neurocysticercosis due to Taenia solium as: 1) chemotherapy of parenchymal, subarachnoidal and racemose (cysts in spinal fluid) neurocysticercosis in symptomatic cases and 2) prophylaxis of epilepsy and other sequelae in asymptomatic neurocysticercosis.	Amedra Pharmaceuticals LLC
	Albuterol	n/a	3/12/2002	Prevention of paralysis due to spinal cord injury	MotoGen, Inc.
	Aldesleukin	Proleukin	11/24/1998	For the treatment non-Hodgkin's lymphoma.	Prometheus Laboratories, Inc.
	Aldesleukin	Proleukin	3/22/1989	Treatment of primary immunodeficiency disease associated with T-cell defects.	Prometheus Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Aldesleukin	Proleukin	9/14/1988	Treatment of metastatic renal cell carcinoma.	Chiron Corporation
	Aldesleukin	Proleukin	9/10/1996	Treatment of metastatic melanoma.	Chiron Corporation
	Alendronate disodium	Fosamax	2/13/2001	Treatment of the bone manifestations of Gaucher disease	Richard J. Wenstrup, M.D.
	Alfentanil	n/a	7/8/2005	Management of postherpetic neuralgia	Cinergen, LLC
	Alfentanil	n/a	8/9/2005	Treatment of painful HIV-associated neuropathy	Cinergen, LLC
	Alglucerase injection	Ceredase	3/11/1985	For replacement therapy in patients with Gaucher's disease type I.	Genzyme Corporation
	Alglucerase injection	Ceredase	7/21/1995	Replacement therapy in patients with Type II and III Gaucher's disease.	Genzyme Corporation
	Alitretinoin	Panretin	4/10/1992	Treatment of acute promyelocytic leukemia	Ligand Pharmaceuticals, Inc.
	Alitretinoin	Panretin	3/24/1998	Treatment of AIDS-related Kaposi's sarcoma.	Eisai, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Allogeneic T-cells cultured with anti-CD3 and IL-2; transduced with retroviral vector (SFCMM-3), expressing herpes simplex 1 virus-thymidine kinase (HSV-TK) and truncated low affinity nerve growth factor receptor; selected with anti-low affinity nerve gro	n/a	1/28/2005	Immunotherapy for acceleration of T-cell reconstitution in patients undergoing allogeneic hematopoietic stem cell transplantation	MolMed S.p.A.
	Allogeneic human aortic endothelial cells cultured in a porcine gelatin matrix	Vascugel (R)	4/3/2009	Prevention of arteriovenous fistula or arteriovenous graft failure in patients with end stage renal disease receiving hemodialysis or preparing for hemodialysis	Shire
	Allogeneic motor neuron progenitor cells derived from human embryonic stem cells	Motorgraft (Tm)	1/17/2014	Treatment of Amyotrophic Lateral Sclerosis	California Stem Cell Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Allogeneic peripheral blood mononuclear cells sensitized against patient alloantigens by mixed lymphocyte culture	Cytoimplant	6/13/1997	Treatment of pancreatic cancer	Applied Immunotherapeutics, LLC
	Allogeneic retinal epithelial cells transfected with plasmid vector expressing ciliary neurotrophic growth factor	n/a	9/1/2004	Treatment of retinitis pigmentosa	Neurotech USA, Inc.
	Allopurinol sodium	Aloprim For Injection	10/16/1992	Management of patients with leukemia, lymphoma, and solid tumor malignancies who are receiving cancer therapy which causes elevations of serum and urinary uric acid levels and who cannot tolerate oral therapy.	Catalytica Pharmaceuticals, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Alpha-galactosidase A	Replagal	6/22/1998	Long-term enzyme replacement therapy for the treatment of Fabry disease	Shire Human Genetic Therapies, Inc.
	Alpha-galactosidase A	Fabrase	7/20/1990	Treatment of Fabry's disease.	Desnick, Robert J. M.D.
	Alpha-galactosidase A	Cc-Galactosidase	6/17/1991	Treatment of alpha-galactosidase A deficiency (Fabry's disease).	David Calhoun, Ph.D.
	Alpha-melanocyte stimulating hormone	n/a	8/19/1997	Prevention and treatment of intrinsic acute renal failure due to ischemia.	National Institute of Diabetes, and Digestive and Kidney Diseases
	Alpha-tocopherol quinone	n/a	3/28/2006	Treatment of inherited mitochondrial respiratory chain diseases	Penwest Pharmaceuticals Company
	Alpha1-Proteinase Inhibitor (Human)	n/a	12/22/2004	Inhalation therapy for the treatment of congenial deficiency of alpha1-proteinase inhibitor.	Kamada Ltd.
	Alpha1-Proteinase Inhibitor (Human)	Arc-Api	9/1/2004	Treatment of cystic fibrosis	Kamada Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Alpha1-antitrypsin(human)	n/a	4/3/2008	Treatment of bronchiectasis	Kamada Ltd.
	Alpha1-proteinase inhibitor (human)	Prolastin	12/7/1984	For replacement therapy in the alpha-1-proteinase inhibitor congenital deficiency state.	Bayer Corporation
	Altretamine	Hexalen	2/9/1984	Treatment of advanced adenocarcinoma of the ovary.	Medimmune Oncology, Inc.
	Alvocidib	n/a	4/13/2007	Treatment of B-cell chronic lymphocytic leukemia (B-CLL) or prolymphocytic leukemia arising from CLL.	Tolero Pharmaceuticals, Inc.
	Amantadine HCl	n/a	7/20/2015	Treatment of levodopa-induced dyskinesia	Osmotica Pharmaceutical Corporation
	Amantadine hydrochloride	Nurelin	4/9/2015	Treatment of levodopa-induced dyskinesia	Adamas Pharmaceuticals, Inc.
	Amifostine	Ethiol	11/24/1998	For the reduction of the incidence and severity of toxicities associated with cisplatin administration.	Clinigen Group plc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Amifostine	Ethyol	5/30/1990	For use as a chemoprotective agent for cyclophosphamide in the treatment of advanced ovarian carcinoma.	Clinigen Group plc
	Amifostine	Ethyol	5/30/1990	For use as a chemoprotective agent for cisplatin in the treatment of metastatic melanoma.	Clinigen Group plc
	Amifostine	Ethyol	5/30/1990	For use as a chemoprotective agent for cisplatin in the treatment of advanced ovarian carcinoma.	Medimmune Oncology, Inc.
	Amifostine	Ethyol	10/4/1999	Treatment of myelodysplastic syndromes.	Clinigen Group plc
	Amifostine	Ethyol	5/12/1998	Reduction of the incidence of moderate to severe xerostomia in patients undergoing post-operative radiation treatment for head and neck cancer.	Medimmune Oncology, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Aminocaproic acid	Caprogel	1/6/1995	For the topical treatment of traumatic hyphema of the eye.	Eastern Virginia Medical School
	Aminosalicylic acid	Paser Granules	2/19/1992	Treatment of tuberculosis infections	Jacobus Pharmaceutical Company
	Aminosidine	Gabbromicina	5/14/1993	Treatment of tuberculosis.	Kanyok, Thomas P. Pharm.D.
	Aminosidine	Paromomycin	9/9/1994	Treatment of visceral leishmaniasis (kala-azar).	Kanyok, Thomas P. Pharm.D.
	Aminosidine	Gabbromicina	11/15/1993	Treatment of Mycobacterium avium complex.	Kanyok, Thomas P. Pharm.D.
	Amiodarone	Amio-Aqueous	8/17/1993	Treatment of incessant ventricular tachycardia.	Academic Pharmaceuticals, Inc.
	Amiodarone HCl	Cordarone	3/16/1994	For the acute treatment and prophylaxis of life-threatening ventricular tachycardia or ventricular fibrillation.	Wyeth-Ayerst Laboratories
	Ammonium tetrathiomolybdate	Coprexa	1/31/1994	Treatment of Wilson's disease.	Pipex Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Amphotericin B inhalation powder	n/a	12/15/2005	Prevention of pulmonary fungal infections in patients at risk for aspergillosis due to immunosuppressive therapy including those receiving organ or stem cell transplants, or treated with chemotherapy or radiation for hematologic malignancies	Novartis Pharmaceuticals Corporation
	Amphotericin B lipid complex	Abelcet	12/5/1991	Treatment of invasive fungal infections.	Liposome Company, Inc.
	Amyl nitrite, sodium nitrite, sodium thiosulfate	Cyanide Antidote Kit	12/18/2006	Treatment of cyanide poisoning	Akorn, Inc.
	Anagrelide	Agrylin	6/11/1985	Treatment of polycythemia vera.	Roberts Pharmaceutical Corp.
	Anagrelide	Agrylin	7/14/1986	Treatment of thrombocytosis in chronic myelogenous leukemia.	Roberts Pharmaceutical Corp.
	Anagrelide	Agrylin	1/27/1988	Treatment of essential thrombocythemia.	Roberts Pharmaceutical Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Ananain, comosain	Vianain	1/21/1992	For the enzymatic debridement of severe burns.	Genzyme Corporation
	Anatibant	n/a	4/15/2005	Treatment of patients having experienced a severe traumatic brain injury (Glasgow Coma Scale 3 to 8) in order to decrease early mortality and improve long-term functional and neurological outcome	Xytis, Inc.
	Ancrod	Viprinex	10/20/1989	To establish and maintain anticoagulation in heparin-intolerant patients undergoing cardiopulmonary bypass.	Knoll Pharmaceutical Company
	Angiotensin (1-7)	n/a	11/26/2013	Treatment of limb-girdle muscular dystrophy	US Biotest, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Angiotensin 1-7	n/a	2/16/2000	Treatment of neutropenia associated with autologous bone marrow transplantation.	Maret Pharmaceuticals
	Angiotensin 1-7	Marstem	8/3/2001	Treatment of myelodysplastic syndrome	Maret Pharmaceutical Corporation
	Angiotensin-(1-7)	n/a	9/13/2011	Treatment of pulmonary arterial hypertension.	US Biotest, Inc.
	Anti-Beta1 integrin monoclonal antibody	n/a	8/7/2014	Treatment of glioblastoma	OncoSynergy, Inc.
	Anti-T cell receptor murine monoclonal antibody	n/a	4/3/2009	Prophylaxis of acute rejection of solid organ transplantation	Tolera Therapeutics, Inc.
	Anti-eTau Humanized IgG4 Monoclonal Antibody	n/a	5/13/2015	Treatment of Progressive Supranuclear Palsy	Britol-Myers Squibb Company
	Anti-tenascin 81C6 monoclonal antibody labeled w/ I 131	Neuradiab	10/4/2005	Treatment of primary malignant brain tumors	Bradmer Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Anti-thymocyte serum	Nashville Rabbit Anti-Thymocyte Serum	6/2/1993	Treatment of allograft rejection, including solid organ (kidney, liver, heart, lung, and pancreas) and bone marrow transplantatio n.	Applied Medical Research
	Antibody drug conjugate consisting of a humanized IgG1 anti-NaPi2b monoclonal antibody and monomethyl auristatin E	n/a	7/29/2014	Treatment of ovarian cancer	Genentech, Inc.
	Antiepilepsirine	n/a	3/23/1989	Treatment of drug resistant generalized tonic-clonic epilepsy in children and adults.	Children's Hospital
	Antihemophilic factor (human)	Alphanate	1/5/1996	Treatment of von Willebrand's disease	Grifols Biologicals Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Antihemophilic factor (recombinant)	Kogenate	9/25/1989	Prophylaxis and treatment of bleeding in individuals with hemophilia A or for prophylaxis when surgery is required in individuals with hemophilia A.	Bayer Corporation
	Antihemophilic factor (recombinant)	Refacto, Xyntha	2/8/1996	For the control and prevention of hemorrhagic episodes and for surgical prophylaxis in patients with hemophilia A (congenital factor VIII deficiency or classic hemophilia).	Wyeth Pharmaceuticals, Inc.
	Antihemophilic factor/von Willebrand factor complex (human), dried, pasteurized	Humate-P	10/16/1992	Treatment of patients with von Willebrand's disease	CSL Behring

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Antisense 20-mer oligonucleotide complementary to R2 component of ribonucleotide reductase mRNA	n/a	4/15/2005	Treatment of acute myeloid leukemia	Lorus Therapeutics, Inc.
	Antisense oligonucleotide directed against connexin43	Nexagon	4/27/2009	Treatment of persistent corneal epithelial defects	CoDa Therapeutics, Inc.
	Antithrombin III (human)	Atnativ	2/8/1985	For the treatment of patients with hereditary antithrombin III deficiency in connection with surgical or obstetrical procedures or when they suffer from thromboembolism.	Pharmacia & Upjohn AB
	Antithrombin III (human)	Thrombate Iii	11/26/1984	For replacement therapy in congenital deficiency of AT-III for prevention and treatment of thrombosis and pulmonary emboli.	Bayer Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Antithrombin III human	Antithrombin lii Human	1/2/1986	Preventing or arresting episodes of thrombosis in patients with congenital AT-III deficiency and/or to prevent the occurrence of thrombosis in patients with AT-III deficiency who have undergone trauma or who are about to undergo surgery or parturition.	American National Red Cross
	Antivenin crotaline (pit-viper) equine immune F(ab)2	Antivipmyn	1/29/2004	Treatment of envenomation by Crotaline snakes	Rare Disease Therapeutics, Inc.
	Antivenin, crotalidae polyvalent immune Fab (ovine)	Crofab	1/12/1994	Treatment of envenomations inflicted by North American crotalid snakes.	Protherics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Antivenom (crotalidae) purified (avian)	n/a	2/12/1991	Treatment of envenomation by poisonous snakes belonging to the Crotalidae family.	Ophidian Pharmaceuticals, Inc.
	Apomorphine	n/a	7/17/1995	Treatment of the on-off fluctuations associated with late-stage Parkinson's disease.	Pentech Pharmaceuticals, Inc.
	Apomorphine HCl	Apokyn	4/22/1993	Treatment of the on-off fluctuations associated with late-stage Parkinson's disease.	US WorldMeds, LLC
	Apomorphine hydrochloride	n/a	5/23/2006	For the treatment of patients in a vegetative state or minimally conscious state for up to 12 months following a severe traumatic brain injury (traumatic or spontaneous)	NeuroHealing Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Aprotinin	Trasylol	11/17/1993	prophylactic use to reduce perioperative blood loss and the homologous blood transfusion requirement in patients undergoing cardiopulmonary bypass surgery in the course of repeat coronary artery bypass graft surgery, and in selected cases of primary coronary artery bypass graft surgery where the risk of bleeding is	Bayer Corporation
	Arenegy	n/a	8/22/2008	Treatment of malignant pleural mesothelioma	MolMed S.p.A.
	Arginine butyrate	n/a	4/7/1992	Treatment of beta-hemoglobinopathies and beta-thalassemia.	Perrine, Susan P., M.D.
	Arsenic trioxide	Trisenox	5/13/2003	Treatment of chronic lymphocytic leukemia	Teva Branded Pharmaceutical Products R&D, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Arsenic trioxide	Trisenox	3/4/2005	Treatment of malignant glioma	Teva Branded Pharmaceutical Products R&D, Inc.
	Arsenic trioxide	Trisenox	4/28/2000	Treatment of multiple myeloma.	TEVA Branded Pharmaceutical Products R & D, Inc.
	Arsenic trioxide	Trisenox	3/3/1998	Treatment of acute promyelocytic leukemia.	Cephalon
	Artesunate	n/a	7/19/1999	Treatment of malaria.	World Health Organization
	Artesunate	n/a	3/28/2006	Immediate treatment of malaria	US Army Medical Materiel Development Activity
	Ascorbic acid	Ascor L 500	8/31/2007	Treatment of scurvy	McGuff Pharmaceuticals Inc.
	Asfotase alfa	n/a	9/12/2008	Treatment of hypophosphatemia	Alexion Pharmaceuticals, Inc.
	Atovaquone	Mepron	8/14/1991	Prevention of Pneumocystis carinii pneumonia (PCP) in high-risk, HIV-infected patients defined by a history of one or more episodes of PCP and/or a peripheral CD4+ (T4 helper/inducer) lymphocyte count less than or equal to 200/mm <sup>3</sup> .	Glaxo Wellcome Research and Development

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Atovaquone	Mepron	9/10/1990	Treatment of AIDS associated Pneumocystis Carinii Pneumonia.	Glaxo Wellcome Inc.
	Attenuated autologous cancer cells and granulocyte macrophage colony stimulating factor in combination with activated autologous blood derived T-cells	n/a	4/27/2007	Treatment of primary central nervous system malignancies	TVAX Biomedical, LLC
	Autologous CD3+ T cells transduced with retroviral vector containing a chimeric antigen receptor directed against CD19 (Autologous CD3+ T cells containing CD19 chimeric antigen receptor)	n/a	11/13/2014	Treatment of acute lymphoblastic leukemia (ALL)	Juno Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Autologous CD34+ cells transfected with lentiviral vector containing the human WAS cDNA (Telethon 003)	n/a	4/30/2010	Treatment of Wiskott Aldrich syndrome	GlaxoSmithKline Intellectual Property Development
	Autologous DNP-conjugated tumor vaccine	M-Vax	2/23/1999	For adjuvant therapy in melanoma patients with surgically resectable lymph node metastasis (Stage III and limited Stage IV disease).	Avax Technologies, Inc.
	Autologous Engineered Skin Substitute	Permaderm	6/1/2012	Treatment of hospitalized patients with deep partial and full thickness burns requiring grafting.	Amarantus BioScience Holdings, Inc.
	Autologous T Cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	n/a	1/31/2014	For the treatment of Acute Lymphoblastic Leukemia	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Autologous cultured endothelial cells on a donor human corneal disk	n/a	6/1/2007	Treatment of Fuch's dystrophy, pseudophakic bullous keratopathy, and bullous keratopathy	Cellular Bioengineering, Inc.
	Autologous dendritic cells pulsed with autologous antigens from primary malignant brain tumor cells	Dcvax-Brain	11/29/2002	Treatment of primary brain malignant cancer	Northwest Biotherapeutics, Inc.
	Autologous dendritic cells pulsed with recombinant human fusion protein (mucin1-glutathione S transferase) coupled to oxidized polymannose	Cvac(TM)	9/13/2010	Treatment of ovarian cancer.	Prima Biomed Ltd.
	Autologous incubated macrophage	n/a	9/3/2004	Therapy to improve the motor and sensory neurological outcome in acute cases of spinal cord injury	Proneuron Biotechnologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Autologous lymphoma-derived immunoglobulin idiotype antigen vaccine conjugated to keyhole limpet hemocyanin	Biovaxid	10/18/2011	Treatment of Waldenstrom's macroglobulin emia	Biovest International, Inc.
	Autologous olfactory neural progenitors	Rhinocytes	2/1/2008	Treatment of spinal cord injury patients with ASIA Impairment grades A, B, or C	RhinoCyte, Inc.
	Autologous or allogeneic limbal epithelial stem cells expanded ex vivo on human amniotic membrane	n/a	7/14/2005	Treatment of ocular surface diseases that are characterized by total limbal stem cell deficiency	TissueTech, Inc.
	Autolymphocyte therapy	n/a	7/12/1994	Treatment of renal cell carcinoma.	Cytogen Corporation
	Azacitidine	Vidaza	6/18/2008	Treatment of acute myeloid leukemia	Celgene Corporation
	Azathioprine	Imuran	9/14/1999	Treatment of oral manifestations of graft-versus-host disease.	Oral Solutions, Inc.
	BF2.649 (Pitolisant)	n/a	5/17/2010	Treatment of narcolepsy	Bioprojet Pharma

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Bacillus Calmette-Guerin vaccine	n/a	8/9/2006	Treatment of stage IIb through IV metastatic melanoma	OncoVac Corporation
	Bacitracin	Altracin	3/13/1984	Treatment of antibiotic-associated pseudomembranous enterocolitis caused by toxins A and B elaborated by Clostridium difficile.	A. L. Laboratories, Inc.
	Baclofen	Lioresal Intrathecal	9/26/1994	Treatment of spasticity associated with cerebral palsy.	Medtronic, Inc.
	Balsalazide disodium	Colazal	8/12/2005	Treatment of pediatric patients with ulcerative colitis	Salix Pharmaceuticals, Inc.
	Bardoxolone	n/a	8/6/2008	Treatment of pancreatic cancer	Reata Pharmaceuticals, Inc.
	Basiliximab	Simulect	12/12/1997	Prophylaxis of solid organ rejection.	Novartis Pharmaceuticals Corporation
	Beclomethasone dipropionate	n/a	3/27/1998	For oral administration in the treatment of intestinal graft-versus-host disease.	Soligenix, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Belinostat	Beleodaq	9/3/2009	Treatment of peripheral T-cell lymphoma (PTCL).	Spectrum Pharmaceuticals, Inc.
	Bendamustine hydrochloride	Treanda	8/17/2007	Treatment of chronic lymphocytic leukemia	Cephalon, Inc.
	Bendamustine oral doseage formulation	n/a	11/5/2015	Treatment of chronic lymphocytic leukemia	Exinda Theapeutics LLC
	Benzoate and phenylacetate	Ucephan	1/21/1986	For adjunctive therapy in the prevention and treatment of hyperammone mia in patients with urea cycle enzymopathy due to carbamylphosp hate synthetase, ornithine, transcarbamy lase, or argininosuccin ate synthetase deficiency.	ImmunexImmunex

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Benzophenone-3, octylmethoxycinnamate, avobenzone, titanium dioxide, zinc oxide	Total Block VI Spf 75	8/13/2001	For the prevention of visible light induced skin photosensitivity as a result of porfimer sodium photodynamic therapy	Fallien Cosmeceuticals Ltd.
	Benzydamine hydrochloride	Tantum	5/18/1998	Prophylactic treatment of oral mucositis resulting from radiation therapy for head and neck cancer.	Angelini Pharmaceuticals, Inc.
	Benzylpenicillin, benzylpenicilloic, benzylpenilloic acid	Pre-Pen/Mdm	9/28/1987	Assessing the risk of administrating penicillin when it is the preferred drug of choice in adult patients who have previously received penicillin and have a history of clinical sensitivity.	AllerQuest LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Beractant	Survanta Intratracheal Suspension	12/20/1993	Treatment of full-term newborn infants with respiratory failure caused by meconium aspiration syndrome, persistent pulmonary hypertension of the newborn, or pneumonia and sepsis.	Ross Laboratories
	Beractant	Survanta Intratracheal Suspension	2/5/1986	Treatment of neonatal respiratory distress syndrome	Ross Laboratories
	Beraprost	n/a	4/29/1999	Treatment of pulmonary arterial hypertension associated with any New York Heart Association classification (Class I, II, III, or IV).	LungRx, Inc.
	Beta alethine	Betathine	3/24/1997	Treatment of metastatic melanoma.	Dovetail Technologies, Inc.
	Beta alethine	Betathine	3/24/1997	Treatment of multiple myeloma.	Dovetail Technologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Betahistine dihydrochloride	n/a	11/8/2007	Treatment of obesity associated with Prader Willi syndrome	Floyd R. Sallee, M.D., Ph.D.
	Betaine	Cystadane	5/16/1994	Treatment of homocystinuria.	Jazz Pharmaceuticals
	Betulinic acid	n/a	8/9/2007	Topical treatment of metastatic melanoma	Advanced Life Sciences, Inc. (ALS)
	Bexarotene	Targretin	6/18/1999	Treatment of cutaneous T-cell lymphoma.	Eisai, Inc.
	Bindarit	n/a	2/3/1998	Treatment of lupus nephritis.	Angelini Pharmaceuticals, Inc.
	Bio-engineered oral mucosal tissue	n/a	4/27/2006	For use as a graft for restoring a cornea-like epithelial phenotype to substitute for the normal corneal epithelium that is lost in patients due to total limbal stem cell deficiency	TissueTech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Bioartificial liver system utilizing xenogenic hepatocytes in a hollow fiber bioreactor cartridge (BAL)	n/a	2/11/2002	Treatment of patients with acute liver failure presenting with encephalopathy deteriorating beyond Parson's grade 2	Excorp Medical, Inc.
	Bis(4-fluorophenyl)phenylacetamide	n/a	3/2/2000	Treatment of sickle cell disease.	ICAgen Inc.
	Bleomycin	n/a	12/20/2010	Treatment of pancreatic cancer.	CIRJ Company Ltd.
	Bleomycin	Blenoxane	2/9/1999	Treatment of pancreatic cancer.	Genetronics, Inc.
	Bleomycin sulfate	Blenoxane	9/17/1993	Treatment of malignant pleural effusion.	Bristol-Myers Squibb Pharmaceutical Research Institute
	Blinatumomab	n/a	5/16/2008	Treatment of chronic lymphocytic leukemia	Amgen, Inc.
	Bone marrow-derived mononuclear cells	n/a	5/17/2010	Treatment of thromboangiitis obliterans (Buerger's disease)	t2cure GmbH
	Bosentan	Tracleer	10/6/2000	Treatment of pulmonary arterial hypertension.	Actelion Life Sciences Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Botulinum toxin type A	Botox	3/22/1984	Treatment of strabismus and blepharospasms	Allergan, Inc.
	Botulinum toxin type A	Botox	8/20/1986	Treatment of cervical dystonia.	Allergan, Inc.
	Botulinum toxin type A	Dysport	3/23/1989	Treatment of essential blepharospasm.	Porton International, Inc.
	Botulinum toxin type A	n/a	9/15/1992	Treatment of synkinetic closure of the eyelid associated with VII cranial nerve aberrant regeneration.	Botulinum Toxin Research Associates, Inc.
	Botulinum toxin type A	Dysport	10/20/1999	Treatment of dynamic muscle contractures in pediatric cerebral palsy patients.	Ipsen Limited
	Botulinum toxin type A	Dysport	8/12/1998	Treatment of spasmodic torticollis (cervical dystonia).	Ipsen Biopharm Limited
	Botulinum toxin type A	Botox	12/6/1991	Treatment of dynamic muscle contracture in pediatric cerebral palsy patients.	Allergan, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Botulinum toxin type B	Myobloc	1/16/1992	Treatment of cervical dystonia.	Soltice Neurosciences, LLC
	Botulinum toxin type F	n/a	10/24/1991	Treatment of spasmodic torticollis (cervical dystonia).	Ipsen Limited
	Botulism immune globulin	Babybig	1/31/1989	Treatment of infant botulism.	California Department of Health Services
	Bovine colostrum	n/a	11/19/1990	Treatment of AIDS-related diarrhea.	Hastings, Donald DVM
	Bovine immunoglobulin concentrate, Cryptosporidium parvum	Sporidin-G	3/1/1994	Treatment and symptomatic relief of Cryptosporidium parvum infection of the gastrointestinal tract in immunocompromised patients.	GalaGen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Bovine whey protein concentrate	Immuno-C	9/30/1993	Treatment of cryptosporidiosis caused by the presence of <i>Cryptosporidium parvum</i> in the gastrointestinal tract of patients who are immunodeficient/immunocompromised or immunocompetent.	Biomune Systems, Inc.
	Branched chain amino acids	n/a	12/23/1988	Treatment of amyotrophic lateral sclerosis.	Mount Sinai Medical Center
	Brilliant Blue G	Dorc IIm-Blue	7/31/2012	To selectively stain the thickened internal limiting membrane, which has formed onto the inner side of the retina in vitreo-retinal disorders	Dutch Ophthalmic Research Center
	Brimonidine	Alphagan	2/7/2000	Treatment of anterior ischemic optic neuropathy.	Allergan, Inc.
	Brivaracetam	n/a	10/5/2005	Treatment of symptomatic myoclonus	UCB Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Broxuridine	Broxine/Neomark	9/18/1995	Radiation sensitizer in the treatment of primary brain tumors.	NeoPharm, Inc.
	Bruton's Tyrosine Kinase (Btk) Inhibitor	n/a	5/13/2015	Treatment of chronic lymphocytic leukemia (CLL).	Acerta Pharma BV
	Bucillamine	n/a	10/22/2015	Treatment of cystinuria	Revive Therapeutics, Ltd.
	Buffered Ursodeoxycholic Acid	Ursocarb	9/3/2004	Treatment of pruritus in patients with Alagille Syndrome	Digestive Care, Inc.
	Buffered intrathecal electrolyte/dextrose injection	Elliotts B Solution	8/24/1994	For use as a diluent in the intrathecal administration of methotrexate and cytarabine for the prevention or treatment of meningeal leukemia and lymphocytic lymphoma	Lukare Medical, LLC
	Buprenorphine hydrochloride	Subutex	6/15/1994	Treatment of opiate addiction in opiate users.	Reckitt Benckiser Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Busulfan	Spartaject	4/21/1994	For use as preparative therapy for malignancies treated with bone marrow transplantatio n.	Sparta Pharmaceuticals, Inc.
	Busulfan	Spartaject	7/7/1997	Treatment of primary brain malignancies.	SuperGen, Inc.
	Busulfan	Busulfex	7/28/1994	As preparative therapy in the treatment of malignancies with bone marrow transplantatio n.	Otsuka Pharmaceutical Company
	Buthionine sulfoxamine	n/a	10/5/2005	Use as a modulator of chemotherapy for the treatment of pediatric patients with primary malignant brain tumors	USC-CHLA Institute for Pediatric Clinical Research
	Buthionine sulfoxamine	n/a	10/5/2005	For use in children as a modulator of chemotherapy for the treatment of pediatric patients with neuroblastoma	USC-CHLA Institute for Pediatric Clinical Research

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Butyrylcholinesterase	n/a	3/25/1992	For the reduction and clearance of toxic blood levels of cocaine encountered during a drug overdose.	Shire Laboratories Inc.
	Butyrylcholinesterase	n/a	9/30/1992	Treatment of post-surgical apnea.	Shire Laboratories Inc.
	C1 esterase inhibitor (human)	n/a	8/21/1996	Treatment and prevention of angioedema caused by C1-esterase inhibitor deficiency.	Alpha Therapeutic Corporation
	C1 esterase inhibitor (human)	Cinryze(R)	7/16/2004	Treatment of angioedema	Shire
	C1-esterase inhibitor (recombinant)	Ruconest	2/23/1999	Treatment of (acute attacks of) angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency.	Santarus, Inc.
	C1-esterase-inhibitor, human, pasteurized	Berinerst	10/16/1992	Prevention and/or treatment of acute attacks of hereditary angioedema.	CSL Behring LLC
	C20-D3-retinyl acetate	n/a	9/16/2010	Treatment of Stargardt's disease	Alkeus pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	C21H27O10P	Minnelide	3/30/2015	Treatment of gastric cancer.	Minneamrita Therapeutics, LLC
	C24H28N8O7S2	n/a	4/2/2015	Treatment of diffuse large B-cell lymphoma (DLBCL).	Curis, Inc.
	C66H100N6O27	n/a	3/11/2013	Treatment of of hepatocellular carcinoma	GenSpera, Inc.
	CD40/CD80/CD86 modified autologous dendritic cell therapy	n/a	12/20/2013	Treatment Type 1 diabetes mellitus patients with residual beta cell function	DiaVacs, Inc.
	CNDO-109-activated allogeneic natural killer cells	n/a	6/18/2012	Treatment of acute myeloid leukemia	Coronado Biosciences, Inc.
	Caffeine	Cafcit	9/20/1988	Treatment of apnea of prematurity.	O.P.R. Development, L.P.
	Calcitonin-human for injection	Cibacalcin	1/20/1987	Treatment of symptomatic Paget's disease (osteitis deformans).	Novartis Pharmaceutical Corporation
	Calcium acetate	Phos-Lo	12/22/1988	Treatment of hyperphosphatemia in end stage renal failure.	Fresenius Medical Care North America
	Calcium acetate	n/a	6/27/1989	Treatment of hyperphosphatemia in end stage renal disease.	Pharmedic Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Calcium carbonate	R & D Calcium Carbonate/600	6/6/1990	Treatment of hyperphosphatemia in patients with end stage renal disease.	R & D Laboratories, Inc.
	Calcium gluconate	Calgonate	11/20/1997	For use as a wash for hydrofluoric acid spills on human skin.	Calgonate Corp.
	Calcium gluconate gel	H-F Gel	5/21/1991	For use in the emergency topical treatment of hydrogen fluoride (hydrofluoric acid) burns.	Calgonate Corporation
	Calcium gluconate gel 2.5%	n/a	9/10/1990	Emergency topical treatment of hydrogen fluoride (hydrofluoric acid) burns.	Paddock Laboratories, Inc.
	Capsaicin	n/a	8/3/2005	Treatment of postherpetic neuralgia.	TheraQuest Biosciences, LLC
	Capsaicin	n/a	9/29/2006	Treatment of intermetatarsal neuroma (Morton's Neuroma) that does not respond to conservative treatment and requires either neurectomy or neurolysis	Centrexion Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Carmustine	n/a	7/3/2000	Treatment of intracranial malignancies.	Direct Therapeutics, Inc.
	Cascara sagrada fluid extract	n/a	3/21/1989	Treatment of oral drug overdose to speed lower bowel evacuation.	Intramed Corporation
	Catumaxomab	Removab	6/9/2006	Treatment of ovarian cancer	Neoviibiotech North America, Inc.
	Ceftriaxone sodium	Rocephin	3/28/2006	Treatment of amyotrophic lateral sclerosis	Mass General Hospital
	Cells produced using the AastromReplicell e System and SC-I Therapy Kit	n/a	7/10/2002	For use in patients receiving high dose chemotherapy who are unable to generate an acceptable dose of peripheral blood stem cells and who have a sufficient bone marrow aspirate without morphological evidence of tumor	Aastrom Biosciences Incorporated
	Centruroides immune F(ab)2	Anascorp	6/12/2000	Treatment of scorpion envenomations requiring medical attention.	Rare Disease Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Ceramide trihexosidase/alpha-galactosidase A	Fabrazyme	1/19/1988	Treatment of Fabry's disease.	Genzyme Corporation
	Chelating agent delivering Holmium-166	n/a	2/10/1999	Treatment of multiple myeloma.	NeoRx Corporation
	Chenodeoxycholic acid	Chenofalk	1/29/2004	Treatment of cerebrotendinous xanthomatosis	Dr. Falk Pharma GmbH
	Chimeric monoclonal antibodies, c-alphaStx2	n/a	10/4/2005	For treatment of shiga-toxin producing bacterial infection	Thallion Pharmaceuticals, Inc.
	Chimeric monoclonal antibody to mesothelin	n/a	10/31/2006	Treatment of pancreatic cancer	Morphotek, Inc.
	Chimeric, humanized monoclonal antibody to staphylococcus	n/a	8/3/2000	Prophylaxis of Staphylococcus epidermidis sepsis in low birth weight (1500 grams or less) infants.	Biosynexus, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Chlorhexidine gluconate mouthrinse	Peridex	8/18/1986	For use in the amelioration of oral mucositis associated with cytoreductive therapy used in conditioning patients for bone marrow transplantatio n therapy.	Procter & Gamble Company
	Chlorotoxin	n/a	12/18/2007	Treatment of malignant glioma	Morphotek, Inc.
	Choline chloride	Intrachol	2/10/1994	Treatment of choline deficiency, specifically the choline deficiency, hepatic steatosis, and cholestasis, associated with long-term parenteral nutrition.	QOL Therapeutics EU Ltd
	Choline chloride	Intrachol	7/20/2006	Prevention and/or treatment of choline deficiency in patients on long-term parenteral nutrition	QOL Therapeutics EU Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Chondrocyte-alginate gel suspension	n/a	12/1/1997	For use in correcting vesicoureteral reflux in the pediatric population.	Curis, Inc.
	Ciliary neurotrophic factor	n/a	1/30/1992	Treatment of amyotrophic lateral sclerosis.	Regeneron Pharmaceuticals Inc
	Cintredekin Besudotox	n/a	4/30/2010	Treatment of idiopathic pulmonary fibrosis	Insys Therapeutics, Inc.
	Cisplatin ChemoThin Wafer	n/a	11/3/2015	Treatment of anatomically accessible oral cancers (lip, tongue, gum, floor of mouth, salivary gland, and other oral cavity)	Privo Technologies
	Cisplatin in liposomal formulation	Slit Cisplatin For Inhalation	3/20/2007	Treatment of osteosarcoma	Eleison Pharmaceuticals LLC
	Cisplatin/epinephrine	Intradose	4/3/2000	Treatment of squamous cell carcinoma of the head and neck.	Matrix Pharmaceutical, Inc.
	Cisplatin/epinephrine	Intradose	9/7/2000	Treatment of metastatic malignant melanoma.	Matrix Pharmaceutical, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Citric acid, glucono-delta-lactone and magnesium carbonate	Renacidin Irrigation	8/28/1989	Treatment of renal and bladder calculi of the apatite or struvite variety.	United-Guardian, Inc.
	Cladribine	Leustatin Injection	11/15/1990	Treatment of hairy cell leukemia.	R. W. Johnson Pharmaceutical Research Institute
	Cladribine	Mylinax	4/19/1994	Treatment of the chronic progressive form of multiple sclerosis.	Johnson & Johnson Pharmaceutical R & D, LLC
	Clazosentan	Erajat	2/16/2006	Treatment of cerebral vasospasm following subarachnoid hemorrhage	Actelion Pharmaceuticals Ltd.
	Clindamycin	Cleocin	10/28/1988	Prevention of Pneumocystis carinii pneumonia in AIDS patients.	Pfizer Inc.
	Clindamycin	Cleocin	10/28/1988	Treatment of Pneumocystis carinii pneumonia associated with AIDS patients.	Pharmacia & Upjohn
	Clindamycin hydrochloride	n/a	8/9/2006	Treatment of sarcoidosis	Autoimmunity Research Foundation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Clofazimine	Lamprene	6/11/1984	Treatment of lepromatous leprosy, including dapsone-resistant lepromatous leprosy and lepromatous leprosy complicated by erythema nodosum leprosum.	Novartis Pharmaceutical Corporation
	Clonazepam	Klonopin	8/4/1994	Treatment of hyperekplexia (startle disease).	Hoffmann-La Roche, Inc.
	Clonazepam Intranasal Spray	n/a	12/19/2007	Treatment of recurrent acute repetitive seizures	Jazz Pharmaceuticals, Inc.
	Clonidine	Duraclon	1/24/1989	For continous epidural administration as adjunctive therapy with intraspinal opiates for the treatment of pain in cancer patients tolerant to, or unresponsive to, intraspinal opiates.	Roxane Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Clotrimazole	n/a	3/13/2006	Treatment of Huntington's disease	EnVivo Pharmaceuticals, Inc.
	Clotrimazole	n/a	6/14/2005	Topical treatment of children and adults with pouchitis	AesRx, LLC
	Clotrimazole	n/a	4/24/1995	Treatment of sickle cell disease.	Brugnara, Carlo M.D.
	Coagulation Factor IX (human)	Alphanine	7/5/1990	For use as replacement therapy in patients with hemophilia B for the prevention and control of bleeding episodes, and during surgery to correct defective hemostasis.	Alpha Therapeutic Corporation
	Coagulation Factor IX (recombinant)	Benefix	10/3/1994	Treatment of hemophilia B.	Genetics Institute, Inc.
	Coagulation Factor VIIa (Recombinant)	Novoseven	7/16/2004	Treatment of bleeding episodes in patients with acquired inhibitors to Factor VIII or Factor IX	Novo Nordisk, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Coagulation factor IX	Mononine	6/27/1989	Replacement treatment and prophylaxis of the hemorrhagic complications of hemophilia B.	Armour Pharmaceutical Company
	Coagulation factor IX (recombinant), Fc fusion protein	Alprolix	10/30/2008	Control and prevention of hemorrhagic episodes in patients with hemophilia B (congenital factor IX deficiency or Christmas disease)	Biogen Idec Inc.
	Coagulation factor VIIa (recombinant)	Novoseven	9/10/2004	Treatment of bleeding episodes in patients with congenital factor VII deficiency	Novo Nordisk, Inc.
	Coagulation factor VIIa (recombinant)	Novoseven	6/18/2004	Prevention of bleeding episodes in patients with hemophilia A or B, with or without inhibitors	Novo Nordisk, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Coagulation factor VIIa (recombinant)	Novoseven	6/6/1988	Treatment of bleeding episodes in hemophilia A or B patients with inhibitors to Factor VIII or Factor IX.	Novo Nordisk, Inc.
	Coagulation factor VIIa (recombinant)	Novoseven	7/21/2004	Prevention of bleeding episodes in patients with acquired inhibitors to Factor VIII or Factor IX	Novo Nordisk, Inc.
	Coagulation factor VIIa (recombinant)	Novoseven	6/18/2004	Prevention of bleeding episodes in Glanzmann's thrombasthenia	Novo Nordisk, Inc.
	Coagulation factor VIIa (recombinant)	Novoseven	9/10/2004	Prevention of bleeding episodes in patients with congenital Factor VII deficiency	Novo Nordisk, Inc.
	Coenzyme Q10	n/a	3/5/2001	Treatment of Huntington's disease	Integrative Therapeutics, Inc.
	Colchicine	n/a	9/25/2007	Treatment of Behcet's Syndrome	AR Scientific, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Colfosceril palmitate, cetyl alcohol, tyloxapol	Exosurf Neonatal For Intratracheal Suspension	10/20/1989	of hyaline membrane disease, also known as respiratory distress syndrome, in infants born at 32 weeks gestation or less 2. prevention of hyaline membrane disease (HMD), also known as respiratory distress syndrome (RDS), in infants born at 32 weeks gestation or less and in the treatment of established HMO at all	Glaxo Wellcome Inc.
	Combination of nivolumab and ipilimumab	n/a	10/9/2014	Treatment of Stage IIb to Stage IV melanoma	Bristol-Myers Squibb Company
	Combretastatin A4 Phosphate	n/a	7/23/2003	Treatment of anaplastic thyroid cancer, medullary thyroid cancer, and stage IV papillary or follicular thyroid cancer	OXiGENE, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Combretastatin A4 phosphate	n/a	5/8/2006	Treatment of ovarian cancer	OXiGENE, Inc.
	Conjugate of a dengue virus specific small chemical ligand and an amphiphilic PEG based polymer	n/a	8/6/2013	Treatment of dengue fever (includes dengue hemorrhagic fever and dengue shock syndrome)	NanoViricides Incorporated
	Contulakin-G	n/a	7/7/2005	Intrathecal treatment of neuropathic pain associated with spinal cord injury	Cognetix, Inc.
	Cordycepin	n/a	7/5/2007	Treatment of TdT-positive acute lymphocytic leukemia	OncoVista, Inc.
	Corticotropin-releasing factor, human	Acthrel	11/24/1989	For use in differentiating pituitary and ectopic production of ACTH in patients with ACTH-dependent Cushings syndrome.	Ferring Laboratories, Inc.
	Corticotropin-releasing factor, human	Xerecept	4/6/1998	Treatment of peritumoral brain edema.	Neurobiological Technologies, Inc.
	Coumarin	Onkolox	12/22/1994	Treatment of renal cell carcinoma.	Drossapharm LTD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Coxsackievirus A21	Cavatak	12/15/2005	Treatment of stage II (T4), stage III, and stage IV melanoma	Viralytics Limited
	Creatine	Creapure	10/11/2005	Treatment of Huntington's disease	Marathon Pharmaceuticals, LLC
	Cromolyn sodium	Gastrocrom	3/8/1984	Treatment of mastocytosis.	Fisons Corporation
	Cromolyn sodium 4% ophthalmic solution	Opticrom 4% Ophthalmic Solution	7/24/1985	Treatment of vernal keratoconjunctivitis.	Fisons Corporation
	Cryptosporidium hyperimmune bovine colostrum IgG concentrate	n/a	12/30/1991	Treatment of diarrhea in AIDS patients caused by infection with Cryptosporidium parvum.	ImmuCell Corporation
	Cyclo {{{(E,Z)-(2S,3R,4R)-3-hydroxy-4-methyl-2-(methylamino)nona-6,8-dienoyl}-L-2-aminobutyl-N-methyl-glycyl-N-methyl-L-leucyl-L-valyl-N-methyl-L-leucyl-L-alanyl-D-alanyl-N-methyl-L-leucyl-N-methyl-L-leucyl-N-methyl-L-valyl}}	n/a	12/20/2006	Treatment and chronic control of non-infectious posterior, intermediate and pan-uveitis	Lux Biosciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Cyclosporin A	Mitogard	10/29/2004	Treatment of amyotrophic lateral sclerosis and its variants	Maas Biolab,LLC
	Cyclosporine	n/a	11/25/2003	Prophylaxis of organ rejection in patients receiving allogeneic lung transplant	APT Pharmaceuticals, Inc.
	Cyclosporine	Pluminiq	11/25/2003	Treatment of acute rejection in patients requiring allogeneic lung transplants	APT Pharmaceuticals, Inc.
	Cyclosporine 2% ophthalmic ointment	n/a	8/1/1991	Treatment of patients at high risk of graft rejection following penetrating keratoplasty	Allergan, Inc.
	Cyclosporine A	n/a	5/4/2007	Treatment of vernal keratoconjunctivitis	Santen SAS
	Cyclosporine in combination with omega-3 polyunsaturated fatty acids	n/a	12/6/2000	Prevention of solid organ graft rejection.	RTP Pharma Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Cyclosporine ophthalmic	Optimmune	11/9/1988	Treatment of severe keratoconjunctivitis sicca associated with Sjogren's syndrome.	University Of Georgia
	Cys-His-Ala-Val-Cys	n/a	2/14/2008	For use in conjunction with melphalen for the treatment of malignant melanoma, AJCC stages IIB, IIC, III and IV	Adherex Technologies, Inc.
	Cysteamine	Lynovex	9/11/2014	Treatment of cystic fibrosis	NovaBiotics Ltd.
	Cysteamine	Cystagon	1/25/1991	Treatment of nephropathic cystinosis.	Mylan Laboratories, Inc.
	Cysteamine	n/a	5/1/1986	Treatment of nephropathic cystinosis.	Thoene, Jess G., M.D.
	Cysteamine hydrochloride	Cystaran	8/19/1997	Treatment of corneal cystine crystal accumulation in cystinosis patients.	Sigma-Tau Pharmaceuticals, Inc.
	Cystic fibrosis Tr gene therapy (recombinant adenovirus)	Adgvcftr.10	3/9/1995	Treatment of cystic fibrosis.	GenVec, Inc.
	Cystic fibrosis transmembrane conductance regulator gene	n/a	1/8/1993	Treatment of cystic fibrosis.	Genetic Therapy, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Cytarabine liposomal	Depocyt	6/2/1993	Treatment of neoplastic meningitis.	Pacira Pharmaceuticals, Inc.
	Cytarabine:daunorubicin liposome injection	n/a	8/22/2008	Treatment of acute myeloid leukemia	Celator Pharmaceuticals, Inc.
	Cytomegalovirus DNA Vaccine w/Copolymer/Benzalkonium Chloride	n/a	9/23/2006	For use in prevention of clinically significant CMV viremia, CMV disease and associated complications in at-risk hematopoietic cell transplant and solid organ transplant populations	Vical Inc.
	Cytomegalovirus DNA vaccine with plasmids expressing pp65 and gB genes	n/a	6/3/2005	Prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in at-risk hematopoietic cell transplant and solid transplant populations	Astellas Pharma Global Development, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Cytomegalovirus immune globulin (human)	Cytogam	8/3/1987	Prevention or attenuation of primary cytomegalovirus disease in immunosuppressed recipients of organ transplants.	CSL Behring LLC
	Cytomegalovirus immune globulin intravenous (human)	n/a	1/28/1991	For use in conjunction with ganciclovir sodium for the treatment of cytomegalovirus pneumonia in bone marrow transplant patients.	Bayer Corporation
	D-mannitol and L-proline	Prodarsan	4/20/2009	Treatment of Cockayne syndrome	DNage B.V.
	D-peptide	Nerofe	2/4/2011	Treatment of acute myelogenous leukemia	Immune System Key, Ltd
	D-peptide of the sequence AKRHHGYKRKFH - NH2	Pulmadex	10/23/2002	Treatment of cystic fibrosis	Demegen, Inc.
	DCVAC OvCa	n/a	5/14/2013	Treatment of ovarian cancer	SOTIOS a.s.
	DEAE-rebeccamycin	n/a	3/1/2004	Treatment of bile duct tumors	Helsinn Healthcare SA

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	DNA plasmid expressing diphtheria toxin triggered by the presence of oncofetal gen H19	n/a	8/20/2009	Treatment of ovarian cancer	BioCancell Therapeutics Ltd.
	DNA plasmid vector (pCK-HGFX7) expressing human hepatocyte growth factor	n/a	2/6/2014	Treatment of amyotrophic lateral sclerosis	ViroMed Co., Ltd.
	DNA plasmid vector expressing eIF5Ak50	n/a	12/13/2010	Treatment of multiple myeloma.	Senesco Technologies, Inc.
	DNA plasmid vector expressing eIF5Ak50R protein, siRNA directed against the native eIF5A mRNA, and PEI (polyethyleneimine)	n/a	7/24/2012	Treatment of diffuse large B cell lymphoma	Senesco Technologies, Inc.
	DNA plasmid vector expressing eIF5Ak50R protein, siRNA directed against the native eIF5A mRNA, and PEI (polyethyleneimine)	n/a	7/24/2012	Treatment of mantle cell lymphoma	Senesco Technologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	DNA plasmid vector expressing human IL-12 gene	n/a	4/4/2005	Treatment of ovarian cancer.	Celsion Corporation
	DNA-lipid complex (DMRIE/DOPE)/plasmid vector (VCL-1102, Vical) expressing human interleukin-2	Leuvectin	4/28/2000	Treatment of renal cell carcinoma.	Vical Incorporated
	DNP-Modified autologous tumor vaccine	O-Vax	9/21/2000	Adjuvant therapy for the treatment of ovarian cancer	AVAX Technologies, Inc.
	DOXO-EMCH	n/a	4/18/2011	Treatment of adenocarcinoma of the pancreas.	CytRx Corporation
	DPX-Survivac	n/a	7/14/2015	Treatment of ovarian cancer.	Immunovaccine Technologies, Inc.
	Daclizumab	Zenapax	3/5/1993	Prevention of acute renal allograft rejection.	Hoffmann-La Roche, Inc.
	Dapsone	n/a	11/7/1994	Prophylaxis of toxoplasmosis in severely immunocompromised patients with CD4 counts below 100.	Jacobus Pharmaceutical Company, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Dapsone USP	Dapsone	1/8/1992	For the combination treatment of Pneumocystis carinii pneumonia in conjunction with trimethoprim.	Jacobus Pharmaceutical Company
	Dapsone USP	Dapsone	12/24/1991	Prophylaxis for Pneumocystis carinii pneumonia.	Jacobus Pharmaceutical Company
	Darinaparsin	n/a	9/13/2010	Treatment of peripheral T-cell lymphoma.	Solasia Pharma K.K.
	Dasatinib	Sprycel	11/28/2005	Treatment of chronic myelogenous leukemia	Bristol-Myers Squibb Company
	Dasatinib	Sprycel	11/18/2005	Treatment of Philadelphia-positive acute lymphoblastic leukemia	Bristol-Myers Squibb Company
	Daunorubicin citrate liposome injection	Daunoxome	5/14/1993	Treatment of patients with advanced HIV-associated Kaposi's sarcoma.	NeXstar Pharmaceuticals, Inc.
	Daunorubicin liposomal	n/a	9/5/2008	Treatment of acute myeloid leukemia	Diatos USA, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Deferasirox	Exjade	11/21/2002	Treatment of chronic iron overload in patients with transfusion-dependent anemias	Novartis Pharmaceuticals Corporation
	Defibrotide	n/a	7/5/1985	Treatment of thrombotic thrombocytopenic purpura.	Crinos International
	Dehydroepiandrosterone (DHEA)	Fidelin	8/19/2003	Replacement therapy in individuals with adrenal insufficiency	Paladin Labs, Inc.
	Dehydroepiandrosterone sulfate sodium	n/a	1/29/1997	Treatment of serious burns requiring hospitalization.	Pharmadigm, Inc.
	Dehydroepiandrosterone sulfate sodium	n/a	1/28/1997	To accelerate the re-epithelialization of donor sites in those hospitalized burn patients who must undergo autologous skin grafting.	Pharmadigm, Inc.
	Dermagraft	n/a	12/13/2010	Treatment of epidermolysis bullosa	Shire Regenerative Medicine, Inc.
	Deslorelin	Somagard	11/5/1987		Roberts Pharmaceutical Corp.
	Desmoglein 3 synthetic peptide (PI-0824)	n/a	10/26/2004	Treatment of pemphigus vulgaris	Peptimmune, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Desmopressin acetate	n/a	1/22/1991	Treatment of mild hemophilia A and von Willebrand's disease.	Aventis Behring L.L.C.
	Dexrazoxane	Zinecard	12/17/1991	For the prevention of cardiomyopathy associated with doxorubicin administration.	Pharmacia & Upjohn
	Dextran 70	Dehydrex	3/5/1990	Treatment of recurrent corneal erosion unresponsive to conventional therapy.	Holles Laboratories, Inc.
	Dextran and deferoxamine	Bio-Rescue	3/8/1991	Treatment of acute iron poisoning.	Biomedical Frontiers, Inc.
	Dextran sulfate (inhaled, aerosolized)	Uendex	10/5/1990	For use as an adjunct to the treatment of cystic fibrosis.	Kennedy & Hoidal, M.D.'s
	Dextran sulfate sodium	n/a	11/19/1987	Treatment of acquired immunodeficiency syndrome.	Ueno Fine Chemicals Industry, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Dianeal peritoneal dialysis solution with 1.1% amino acids	Nutrineal (Peritoneal Dialysis Solution With 1.1% Amino Acid	6/11/1992	For use as a nutritional supplement for the treatment of malnourishment in patients undergoing continuous ambulatory peritoneal dialysis.	Baxter Healthcare Corporation
	Diazepam viscous solution for rectal administration	n/a	2/25/1992	For the management of selected, refractory, patients with epilepsy, on stable regimens of antiepileptic drugs (AEDs), who require intermittent use of diazepam to control bouts of increased seizure activity.	Valeant Pharmaceuticals
	Diethyldithiocarbamate	Imuthiol	4/3/1986	Treatment of AIDS.	Connaught Laboratories

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Diethylenetriami nepentaacetic acid (DTPA)	n/a	4/28/2004	Treatment of patients with known or suspected internal contamination with plutonium, americium, or curium to increase the rates of elimination.	Hameln Pharmaceuticals gmbh
	Digoxin immune FAB (Ovine)	Digibind	11/1/1984	Treatment of potentially life threatening digitalis intoxication in patients who are refractory to management by conventional therapy.	Glaxo Wellcome Inc.
	Digoxin immune fab(ovine)	Digidote	3/11/1985	Treatment of life- threatening acute cardiac glycoside intoxication manifested by conduction disorders, ectopic ventricular activity and (in some cases) hyperkalemia.	Boehringer Mannheim Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Dihydrotestosterone	Androgel -Dht	2/5/1996	Treatment of weight loss in AIDS patients with HIV-associated wasting.	Besins Internaitonal, US Inc.
	Dimerizing drug that binds to mutated Fas protein/drug-binding domain fusion protein (FKBP)	n/a	11/24/1999	Treatment of acute graft-versus-host disease in patients undergoing bone marrow transplantatio n.	Bellicum Pharmaceuticals, Inc.
	Dimethyl sulfoxide	n/a	11/22/1994	Treatment of increased intracranial pressure in patients with severe, closed-head injury, also known as traumatic brain coma, for whom no other effective treatment is available.	Abela Pharmaceuticals, Inc.
	Dimethylsulfoxide	n/a	4/6/1998	Treatment of palmar-plantar erythrodyseth esia syndrome.	Cancer Technologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Dimethylsulfoxide	n/a	4/15/1997	Topical treatment for the prevention of soft tissue injury following extravasation of cytotoxic drugs.	Cancer Technologies, Inc.
	Dipalmitoylphosphatidylcholine /phosphatidylglycerol	Alec	7/28/1988	Prevention and treatment of neonatal respiratory distress syndrome.	Forum Products, Inc.
	Diphenylcyclopropenone gel	Samcyprone (Tm)	4/15/2015	Treatment of malignant melanoma stage IIB to IV	RXi Pharmaceuticals Corp.
	Disaccharide tripeptide glycerol dipalmitoyl	Immther	3/1/1990	Treatment of pulmonary and hepatic metastases in patients with colorectal adenocarcinoma.	ImmunoTherapeutics, Inc.
	Disodium clodronate	n/a	6/16/1993	Treatment of hypercalcemia of malignancy.	Discovery Experimental & Development, Inc.
	Disodium clodronate tetrahydrate	Bonefos	3/5/1990	Treatment of increased bone resorption due to malignancy.	Anthra Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Doripenem	n/a	7/16/2004	Treatment of bronchopulmonary infection in patients with cystic fibrosis who are colonized with Pseudomonas aeruginosa or Burkholderia cepacia.	Shionogi, Inc.
	Dornase alfa	Pulmozyme	1/16/1991	To reduce mucous viscosity and enable the clearance of airway secretions in patients with cystic fibrosis.	Genentech, Inc.
	Double-stranded DNA plasmid carrying the gene for the diphtheria toxin A (DT-A) chain	n/a	8/6/2010	Treatment of pancreatic cancer	BioCancell Therapeutics Israel Ltd.
	Doxorubicin HCl with pluronic L-61 and pluronic F-127	n/a	10/7/2005	Treatment of esophageal carcinoma	Supratek Pharma Inc.
	Doxorubicin liposome	Doxil	11/4/1998	Treatment of ovarian cancer.	Alza Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Dronabinol	Marinol	1/15/1991	For the stimulation of appetite and prevention of weight loss in patients with a confirmed diagnosis of AIDS.	Unimed Pharmaceuticals, Inc.
	Duramycin	n/a	12/11/1997	Treatment of cystic fibrosis.	Lantibio, Inc.
	Dynamine	n/a	2/5/1990	Treatment of Lambert Eaton myasthenic syndrome.	Mayo Foundation
	Dynamine	n/a	10/16/1991	Treatment of hereditary motor and sensory neuropathy type I (Charcot-Marie-Tooth disease).	Mayo Foundation
	E-selectin antagonist	n/a	5/13/2015	Treatment of acute myeloid leukemia (AML)	GlycoMimetics, Inc.
	Eculizumab	n/a	9/21/2000	Treatment of dermatomyositis	Alexion Pharmaceuticals, Inc.
	Edaravone	n/a	5/12/2015	Treatment of amyotrophic lateral sclerosis	Mitsubishi Tanabe Pharma Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Edotreotide	Onalta(Tm)	7/28/2005	Treatment of somatostatin receptor-positive neuroendocrine gastroenteropancreatic tumors	Molecular Insight Pharmaceuticals, Inc. (Progenics Subsidiary)
	Efdispo (tm)	Efdispo (Tm)	1/15/2013	Treatment of Ewings Sarcoma.	TDP Biotherapeutics, Inc.
	Eflornithine	n/a	9/29/2006	Treatment of anaplastic glioma	Orbus Therapeutics, Inc.
	Eflornithine HCl	Ornidyl	4/23/1986	Treatment of Trypanosoma brucei gambiense infection (sleeping sickness).	Hoechst Marion Roussel
	Elcatonin	n/a	9/25/1995	Intrathecal treatment of intractable pain.	Innapharma, Inc.
	Elesclomol	n/a	12/18/2007	Treatment of metastatic melanoma in combination with paclitaxel	Synta Pharmaceuticals Corporation
	Enadoline hydrochloride	n/a	1/28/1997	Treatment of severe head injury.	Warner-Lambert Company
	Encapsulated porcine islet preparation	Betarx	7/5/1995	Treatment of type I diabetic patients who are already on immunosuppression.	VivoRx

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Engineered variant of recombinant human fibroblast growth factor 19	n/a	2/6/2014	Treatment of primary biliary cirrhosis	NGM Biopharmaceuticals, Inc.
	Enzastaurin	n/a	9/19/2005	Treatment of glioblastoma multiforme	Denovo Biopharma LLC
	Epidermal growth factor (human)	n/a	10/5/1987	For acceleration of corneal epithelial regeneration and the healing of stromal tissue in the condition of non-healing corneal defects.	Chiron Vision
	Epirubicin	Ellence	9/14/1999	Treatment of breast cancer.	Pharmacia & Upjohn Company
	Epitalon	n/a	9/2/2010	Treatment of retinitis pigmentosa	BioDiem Ltd
	Epoetin alfa	Epogen	4/10/1986	Treatment of anemia associated with end stage renal disease.	Amgen, Inc.
	Epoetin alfa	n/a	12/20/1993	Treatment of myelodysplastic syndrome.	Johnson & Johnson Pharmaceutical Research & Dev.,

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Epoetin alfa	Epogen	7/1/1991	Treatment of anemia associated with HIV infection or HIV treatment.	Amgen, Inc.
	Epoetin alpha	Procrit	3/7/1989	Treatment of HIV associated anemia related to HIV infection or HIV treatment.	R. W. Johnson Pharmaceutical Research Institute
	Epoetin alpha	Procrit	8/27/1987	Treatment of anemia associated with end stage renal disease.	R. W. Johnson Pharmaceutical Research Institute
	Epoetin beta	Marogen	10/22/1987	Treatment of anemia associated with end stage renal disease.	Chugai-USA, Inc.
	Epoprostenol	Flolan	9/25/1985	Treatment of primary pulmonary hypertension.	Glaxo Wellcome Inc.
	Eprodinate	Kiacta(Tm)	4/6/1999	Treatment of secondary amyloidosis	C. T. Development America, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Epstein-Barr virus-specific autologous cytotoxic T lymphocytes	n/a	6/12/2014	Treatment of Epstein-Barr virus positive head and neck cancers (which includes nasopharyngeal carcinoma)	FFCanVac Pte Ltd
	Erlotinib Hydrochloride	Tarceva	7/18/2003	Treatment of malignant gliomas	Genentech, Inc
	Erwinia L-asparaginase	Erwinase	7/30/1986	Treatment of acute lymphocytic leukemia.	Jazz Pharmaceuticals, Inc.
	Erythropoietin (recombinant human)	n/a	8/19/1987	Treatment of anemia associated with end stage renal disease.	McDonnell Douglas Corp
	Ethanolamine oleate	Ethamolin	3/22/1984	Treatment of patients with esophageal varices that have recently bled, to prevent rebleeding.	QOL Medical
	Ethyl eicosapentaenoate	n/a	4/6/2000	Treatment of Huntington's disease.	Laxdale Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Etidronate disodium	Didronel	3/21/1986	Treatment of hypercalcemia of malignancy inadequately managed by dietary modification and/or oral hydration.	MGI Pharma, Inc.
	Etiocholanedione	n/a	11/3/1995	Treatment of aplastic anemia.	SuperGen, Inc.
	Etiocholanedione	n/a	5/7/1996	Treatment of Prader-Willi syndrome.	SuperGen, Inc.
	Ex vivo cultured adult human mesenchymal stem cells	Prochymal(R)	12/14/2005	Treatment of acute graft versus host disease	Mesoblast, Inc.
	Exemestane	Aromasin	9/19/1991	Treatment of advanced breast cancer in postmenopausal women whose disease has progressed following tamoxifen therapy.	Pharmacia & Upjohn
	Exisulind	n/a	2/14/1994	For the suppression and control of colonic adenomatous polyps in the inherited disease adenomatous polyposis coli.	OSI Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Exon 44 specific phosphorothioate oligonucleotide	n/a	11/5/2009	Treatment of Duchenne Muscular Dystrophy in patients who have a mutation correctable by skipping of exon 44 of the dystrophin gene.	Prosensa Therapeutics B.V.
	Exon 45 specific phosphorothioate oligonucleotide	n/a	1/23/2013	Treatment of Duchenne Muscular Dystrophy patients bearing mutations that can be corrected by skipping exon 45	Prosensa Therapeutics B.V.
	Exon 51 specific phosphorothioate oligonucleotide	n/a	8/25/2009	Treatment of Duchenne Muscular Dystrophy.	Prosensa Therapeutics BV
	Exon 52 specific phosphorothioate oligonucleotide	n/a	1/23/2013	Treatment of Duchenne Muscular Dystrophy patients bearing mutations that can be corrected by skipping exon 52	Prosensa Therapeutics B V

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Exon 55 specific phosphorothioate oligonucleotide	n/a	1/23/2013	Treatment of Duchenne Muscular Dystrophy patients bearing mutations that can be corrected by skipping exon 55	Prosensa Therapeutics B.V.
	Extract of sea cucumber, sea sponge, shark fin, sea urchin, and sargassum grass	n/a	5/14/2012	Treatment of multiple myeloma	Unicorn Pacific Corporation
	FTV1+GDP-fucose	n/a	3/18/2011	To improve homing to bone (treatment of myeloablation) in patients receiving hematopoietic stem cell transplantation.	America Stem Cell, Inc.
	Facilitated DNA Plasmid Vaccine	n/a	3/8/1995	Treatment of cutaneous T cell lymphoma.	Wyeth-Lederle Vaccines and Pediatrics
	Factor XIII [A2] homodimer, recombinant DNA origin	n/a	5/21/2003	Treatment of congenital FXIII deficiency	Novo Nordisk Pharmaceuticals, INC.
	Fampridine	Neurelan	6/2/1997	Treatment of chronic, incomplete spinal cord injury.	Acorda Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Felbamate	Felbatol	1/24/1989	Treatment of Lennox-Gastaut syndrome.	Wallace Laboratories
	Fenobam hydrochloride	n/a	11/20/2006	Treatment of fragile X syndrome	Neuropharm, Ltd.
	Fenretinide	n/a	10/5/2005	Treatment of neuroblastoma	USC-CHLA Institute for Pediatric Clinical Research
	Fenretinide	n/a	4/7/2010	Treatment of Pseudomonas aeruginosa lung infections in cystic fibrosis patients	McGill University
	Fibrin-based agent containing a N-terminally modified parathyroid hormone fragment TGpPTH1-34	n/a	2/1/2007	Treatment of solitary (unicameral) bone cysts	Kuros Biosurgery AG
	Fibrinogen (human)	n/a	8/23/1995	For the control of bleeding and prophylactic treatment of patients deficient in fibrinogen.	Alpha Therapeutic Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Fibronectin (human plasma derived)	n/a	9/5/1988	Treatment of non-healing corneal ulcers or epithelial defects which have been unresponsive to conventional therapy and the underlying cause has been eliminated.	Melville Biologics, Inc.
	Fibronectin Peptide	n/a	6/29/2011	Prevention of burn injury progression of acute, deep dermal burns in hospitalized patients.	NeoMatrix Formulations, Inc.
	Filgrastim	Neupogen	10/1/1990	Treatment of neutropenia associated with bone marrow transplants.	Amgen, Inc.
	Filgrastim	Neupogen	11/7/1990	Treatment of patients with severe chronic neutropenia (absolute neutrophil count less than 500/mm <sup>3</sup> ).	Amgen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Filgrastim	Neupogen	11/7/1996	Reduction in the duration of neutropenia, fever, antibiotic use, and hospitalization, following induction and consolidation treatment for acute myeloid leukemia.	Amgen, Inc.
	Filgrastim	Neupogen	7/17/1995	For use in the mobilization of peripheral blood progenitor cells for collection in patients who will receive myeloablative or myelosuppressive chemotherapy.	Amgen, Inc.
	FliC Flagellin Deletion Variant TLR5 Agonist	n/a	11/23/2010	Prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster	Cleveland BioLabs, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Floxuridine, FUDR	n/a	12/22/2004	Intraperitoneal treatment of gastric cancer.	Franco Muggia, M.D.
	Fludarabine phosphate	Fludara	4/18/1989	Treatment of chronic lymphocytic leukemia (CLL), including refractory CLL.	Berlex Laboratories, Inc.
	Fludarabine phosphate	Fludara	4/18/1989	Treatment and management of patients with non-Hodgkins lymphoma.	Berlex Laboratories, Inc.
	Fludarabine phosphate oral tablets	n/a	12/18/2007	Treatment of B-cell chronic lymphocytic leukemia	Sanofi-Aventis U.S., Inc.
	Flunarizine	Sibelium	1/6/1986	Treatment of alternating hemiplegia.	Janssen Research Foundation
	Fluocinolone	Retisert	7/31/2000	Treatment uveitis involving the posterior segment of the eye.	Bausch & Lomb Pharmaceuticals, Inc.
	Fluorouracil	n/a	10/27/1989	For use in combination with interferon alpha-2a, recombinant, for the treatment of esophageal carcinoma.	Hoffmann-La Roche, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Fluorouracil	n/a	4/18/1990	For use in combination with interferon alpha-2a, recombinant, for the treatment of advanced colorectal carcinoma.	Hoffmann-La Roche, Inc.
	Fluorouracil	n/a	6/29/2000	Treatment of glioblastoma multiforme.	Ethypharm SA
	Fluoxetine	Prozac	4/30/1999	Treatment of autism.	Neuropharm, Ltd.
	Fluoxetine	Prozac	4/14/2004	Treatment of body dysmorphic disorder in children and adolescents	Hollander, Eric MD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Follitropin alfa, recombinant	Gonal-F	12/21/1998	initiation and re-initiation of spermatogenesis in adult males with reproductive failure due to hypothalamic or pituitary dysfunction, hypogonadotropic hypogonadism. AMENDED indication 6/27/00: For the induction of spermatogenesis in men with primary and secondary hypogonadotropic hypogonadism in whom the cause of	EMD Serono, Inc.
	Fomepizole	Antizole	12/22/1988	Treatment of methanol or ethylene glycol poisoning.	Jazz Pharmaceuticals
	Fosphenytoin	Cerebyx	6/4/1991	For the acute treatment of patients with status epilepticus of the grand mal type.	Warner-Lambert Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Full Phosphorothioate Antisense Oligonucleotide against EWS-Fli-1 nanoparticles	n/a	9/22/2008	Treatment of Ewing's sarcoma	The Cure Our Children Foundation
	Fully human anti-interferon gamma monoclonal antibody	Ni-0501	3/26/2010	Treatment of hemophagocytic lymphohistiocytosis	NovImmune S.A.
	Fusion protein consisting of human immunoglobulin G1 constant region Fc region fused to the human receptor binding domain of ectodysplasin-A1	n/a	1/11/2006	Treatment of X-linked hypohidrotic ectodermal dysplasia	Edimer Pharmaceuticals, Inc.
	G17DT Immunogen	Gastrimmune(TM)	7/10/2002	Treatment of adenocarcinoma of the pancreas	Cancer Advances, Inc.
	G17DT Immunogen	Gastrimmune(TM)	7/18/2002	Treatment of gastric cancer	Cancer Advances, Inc.
	GNE Lipoplex	n/a	11/13/2008	Treatment of hereditary inclusion body myopathy-2	Gradalis, Inc.
	GNE plasmid(H001)	n/a	3/26/2010	Treatment of hereditary inclusion body myopathy type 2	HIBM Research Group

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	GVAX melanoma	n/a	12/23/2010	Treatment of stage IIb to IV melanoma	Aduro BioTech, Inc.
	Gallium (68Ga) edotreotide	n/a	7/1/2015	Diagnostic for the clinical management of neuroendocrine tumors.	Advanced Accelerator Applications, USA
	Gallium [Ga-68]-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide	n/a	12/31/2013	Diagnostic for the Management of Gastro-Enteropancreatic Neuroendocrine Tumors	Advanced Accelerator Applications
	Gallium-68 (DOTA0-Phe1-Tyr3)octreotide	n/a	10/25/2013	The management of neuroendocrine tumors	University of Iowa Hospital & Clinics

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Gamma hydroxybutyrate	n/a	12/3/1987	Treatment of narcolepsy and the auxiliary symptoms of cataplexy, sleep paralysis, hypnagogic hallucinations and automatic behavior.	Biocraft Laboratories, Inc.
	Gammalinolenic acid	n/a	7/27/1994	Treatment of juvenile rheumatoid arthritis.	Zurier, Robert B. M.D.
	Ganaxolone	n/a	5/25/1994	Treatment of infantile spasms.	Marinus Pharmaceuticals, Inc.
	Ganciclovir intravitreal implant	Vitrasert Implant	6/7/1995	Treatment of cytomegalovirus retinitis.	Bausch & Lomb Surgical, Chiron Vision Products
	Gastrin 17C Diphtheria Toxoid Immunogen	n/a	7/7/2009	Treatment of pancreatic cancer	Astrimmune Ltd.
	Gemtuzumab zogamicin	Mylotarg	11/24/1999	Treatment of CD33-positive acute myeloid leukemia.	Wyeth-Ayerst Laboratories
	Genetically engineered human recombinant IgG4 monoclonal antibody directed against human TNF alpha	n/a	12/11/1997	Treatment of Crohn's disease.	Celltech Chiroscience Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Geneticin	n/a	6/6/2005	Treatment of amoebiasis.	ProcesScience, Inc (PSI)
	Gentamicin impregnated PMMA beads on surgical wire	Septopal	1/31/1991	Treatment of chronic osteomyelitis of post-traumatic, postoperative, or hematogenous origin.	Lipha Pharmaceuticals, Inc.
	Gentamicin liposome injection	Maitec	7/10/1990	Treatment of disseminated Mycobacterium avium-intracellulare infection.	Liposome Company, Inc.
	Glatiramer acetate	Copaxone	11/9/1987	Treatment of multiple sclerosis.	Teva Pharmaceuticals USA
	Glatiramer acetate for injection	Copaxone	6/5/2001	Treatment of primary-progressive multiple sclerosis	Teva Pharmaceuticals USA
	Glutamine	Nutrestore	3/6/1995	For use with human growth hormone in the treatment of short bowel syndrome (nutrient malabsorption from the gastrointestinal tract resulting from an inadequate absorptive surface).	Emmaus Medical, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Glutathione pegylated liposomal doxorubicin hydrochloride	n/a	8/16/2010	Treatment of glioma	to-BBB technologies BV
	Glycerol tri (4-phenylbutyrate)	n/a	5/24/2005	Treatment of spinal muscular atrophy	Ucyclyd Pharma, Inc
	Glycerol trioleate and glycerol trierucate	n/a	2/14/1995	Treatment of adrenoleukodystrophy.	Moser, Hugo W. M.D.
	Glyceraldehyde-3-phosphate dehydrogenase	Proenzy	4/30/2010	Treatment of pediatric multiple sclerosis	BPT Pharmaceuticals, Inc.
	Gonadorelin acetate	Lutrepulse	4/22/1987	For induction of ovulation in women with hypothalamic amenorrhea due to a deficiency or absence in the quantity or pulse pattern of endogenous GnRH secretion.	Ferring Laboratories, Inc.
	Gossypol	n/a	10/22/1990	Treatment of cancer of the adrenal cortex.	Reidenberg, Marcus M. M.D.
	Gp100 adenoviral gene therapy	n/a	3/25/1997	Treatment of metastatic melanoma.	Genzyme Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Growth hormone releasing factor	n/a	8/7/1989	For the long-term treatment of children who have growth failure due to a lack of adequate endogenous growth hormone secretion.	Valeant Pharmaceuticals North America
	H-(D)p-Benzoylphenylalanyl-(D)seryl-(D)tryptophanyl-(D)seryl-(D)pentafluorophenylalanyl-(D)cyclohexylalanyl-(D)arginyl-(D)arginyl-(D)arginyl-(D)glutaminy-(D)arginyl-(D)arginine acetate salt	n/a	12/22/2011	For use in combination with cisplatin and pemetrexed for the treatment of patients with mesothelioma	CanBas Company, Ltd.
	H-Leu-Pro-Pro-Ser-Arg-OH	n/a	9/20/2012	Treatment of Kaposi sarcoma	Immuno Tech, Inc.
	H-Tyr-Gly-Arg-Lys-Lys-Arg-Arg-Gln-Arg-Arg-Arg-Lys-Leu-Ser-Ser-Ile-Glu- Ser-Asp-Val-OH	n/a	4/11/2014	Treatment of acute ischemic stroke patients presenting within 3 hours of symptom onset	NoNO, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	H-Tyr-Gly-Arg-Lys- Lys-Arg-Arg-Gln- Arg-Arg-Arg-allys- aleu-Ser-Ser-Ile- Glu-Ser-Asp-Val- OH	n/a	5/14/2013	Treatment of subarachnoid hemorrhage	NoNO, Inc.
	HIRMAb-IDS	n/a	5/15/2013	Treatment of mucopolysacc haridosis Type II (Hunter Syndrome)	ArmaGen Technologies, Inc.
	HLA-B7/Beta2M DNA Lipid (DMRIE/DOPE) Complex	Allovectin-7	9/30/1999	Treatment of invasive and metastatic melanoma (Stages II, III, and IV).	Vical Incorporated
	HPV-16 cancer therapeutic trojan peptide vaccine	n/a	1/12/2009	Treatment of HPV-16 expressing head and neck squamous cell carcinoma.	Gliknik, Inc.
	HPV16 E6/E7 synthetic long peptides vaccine	n/a	9/1/2011	Treatment of epithelial neoplasias of the vulva positive for human papilloma virus type 16.	ISA Therapeutics BV
	Halofantrine	Halfan	11/4/1991	Treatment of mild to moderate acute malaria caused by susceptible strains of P. falciparum and P. vivax.	SmithKline Beecham Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Halofuginone	Stenorol	2/7/2000	Treatment of systemic sclerosis.	Collgard Biopharmaceuticals Ltd.
	Hanferon	Hanferon	3/9/2012	Treatment of Behcet's disease	HanAll BioPharma Co., Ltd.
	Hantaan virus and Puumala virus DNA vaccines	n/a	11/13/2012	Prevention of hemorrhagic fever with renal syndrome caused by Hantaan virus and Puumala virus.	Surgeon General of the U. S. Army
	Heat Shock Protein (hsp60) antigen	Diapep277	5/21/2012	For use in type 1 diabetic mellitus patients with residual beta-cell function	Andromeda Biotech, LTD
	Heat Shock Protein 70	n/a	3/18/2011	Treatment of amyotrophic lateral sclerosis	ALS Biopharma, LLC
	Heme arginate	Normosang	3/10/1988	Treatment of symptomatic stage of acute porphyria.	Orphan Europe SARL
	Heme arginate	Normosang	3/1/1994	Treatment of myelodysplastic syndromes.	Orphan Europe

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Hemin	Panhematin	3/16/1984	Amelioration of recurrent attacks of acute intermittent porphyria (AIP) temporarily related to the menstrual cycle in susceptible women and similar symptoms which occur in other patients with AIP, porphyria variegata and hereditary coproporphyrin a.	Abbott Laboratories
	Hemin and zinc mesoporphyrin	Hemex	12/20/1993	Treatment of acute porphyric syndromes.	Bonkovsky, Herbert L. M.D.
	Hemoximer (pyridoxalated hemoglobin polyoxyethylene)	n/a	12/18/2007	Treatment of cardiogenic shock	Apex Bioscience, Inc.
	Heparin sodium	n/a	6/29/2006	Treatment of cystic fibrosis	Ockham Biotech Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Heparin-binding epidermal growth factor-like growth factor	n/a	9/18/2006	Prevention and treatment of necrotizing enterocolitis (NEC) in preterm infants with birth weight less than 1,500 grams	Trillium Therapeutics, Inc.
	Hepatitis B immune globulin intravenous (human)	Nabi-Hb	3/8/1995	Prophylaxis against hepatitis B virus reinfection in liver transplant patients.	Biotest Pharmaceuticals Corporation
	Hepatitis C virus immune globulin (human)	Civacir(Tm)	11/14/2002	Prophylaxis of hepatitis C infection in liver transplant recipients.	Biotest Pharmaceuticals Corporation
	Heptadecasodium salt of an 18-base residue phosphorothioate oligonucleotide	n/a	4/18/2011	Treatment of spinal muscular atrophy	Isis Pharmaceuticals, Inc.
	Herpes simplex virus gene	n/a	10/16/1992	Treatment of primary and metastatic brain tumors.	Genetic Therapy, Inc.
	His-His-Ile-Tyr-Leu-Gly-Ala-Val-Asn-Tyr-Ile-Tyr	n/a	3/11/2013	Treatment of retinal detachment	ONL Therapeutics, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Histamine	Maxamine	2/1/2000	For use as an adjunct to cytokine therapy in the treatment of malignant melanoma.	EpiCept Corporation
	Histamine	Ceplene	12/15/1999	Adjunct to cytokine therapy in the treatment of acute myeloid leukemia.	EpiCept Corporation
	Histrelin	n/a	5/3/1991	Treatment of acute intermittent porphyria, hereditary coproporphyrinuria, and variegate porphyria.	Anderson, Karl E., M.D.
	Histrelin	Supprelin La	11/18/2005	Treatment of central precocious puberty	Endo Pharmaceuticals Solutions, Inc.
	Histrelin acetate	Supprelin Injection	8/10/1988	Treatment of central precocious puberty.	Roberts Pharmaceutical Corp.
	Hu1D10, humanized monoclonal antibody	Remitogen	11/28/2001	For use in the treatment of 1D10+ B cell non-Hodgkin's lymphoma	PDL BioPharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Human Allogeneic HLA-matched, Umbilical Cord Blood Hematopoietic Progenitor Cells Ex Vivo Expanded on Allogeneic Mesenchymal Precursor Cells	n/a	9/12/2008	Treatment of insufficient hematopoietic stem cell production in patients with hematologic malignancies who have failed treatment with conventional chemotherapy.	Mesoblast, Inc.
	Human Hemin	n/a	8/6/2013	Prevention of ischemia reperfusion injury in patients undergoing solid organ transplantation	Borders Technology Management Ltd
	Human Monoclonal Antibody against Epidermal Growth Factor Receptor Linked to Monomethylauristatin F	n/a	5/29/2014	Treatment of glioblastoma multiforme	AbbVie, Inc.
	Human T-lymphotropic virus type III Gp160 antigens	Vaxsyn Hiv-1	11/20/1989	Treatment of AIDS.	MicroGeneSys, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Human alpha 2,6 sialyltransferase adenoviral gene therapy	n/a	4/4/2005	Treatment of patients with invasive (malignant) brain and central nervous system tumors lacking alpha 2,6 sialyltransferase.	Falk Center for Molecular Therapeutics
	Human anti-CD30 monoclonal antibody	n/a	9/27/2004	Treatment of Hodgkin's disease	Bristol-Myers Squibb, Inc.
	Human autologous bone-forming cell derived from bone marrow stem cells	n/a	3/24/2008	Treatment of osteonecrosis	Bone Therapeutics S.A.
	Human cytomegalovirus immunoglobulin	n/a	12/20/2006	Prevention of congenital cytomegalovirus (CMV) infection following primary CMV infection in pregnant women	Biotest Pharmaceuticals Corporation
	Human factor X	n/a	11/8/2007	Treatment of hereditary factor X deficiency	Bio Products Laboratory
	Human immune globulin	Flebogamma(R) 5% Dif	3/29/2006	Treatment of post-polio syndrome	Instituto Grifols, S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Human immunoglobulin anti-CD30 monoclonal antibody	n/a	1/10/2006	Treatment of CD30+ T-cell lymphoma	Bristol-Myers Squibb
	Human monoclonal antibody against platelet-derived growth factor D	n/a	11/2/2004	To slow the progression of IgA nephropathy and delay kidney failure in patients affected by the disease.	CuraGen Corporation
	Human monoclonal antibody directed against serotype 011 Pseudomonas aeruginosa	Aerumab 11	9/18/2006	Treatment of hospital acquired pneumonia caused by serotype 011 positive Pseudomonas aeruginosa	Kenta Biotech Limited
	Human monoclonal antibody inhibitor of mannan-binding lectin-associated serine protease-2 (MASP-2)	n/a	12/16/2013	Prevention (inhibition) of complement-mediated thrombotic microangiopathy	Omeros Corporation
	Human monoclonal antibody targeting CC-chemokine ligand 2 (CNTO 888)	n/a	4/30/2010	Treatment of ovarian cancer	Ortho Biotech

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Human plasma coagulation Factor VIII and human plasma von Willebrand Factor	Wilate	4/18/2007	Treatment of von Willebrand disease except for surgical and/or invasive procedures in patients with von Willebrand disease in whom desmopressin is either ineffective or contraindicated	Octapharma USA, Inc.
	Human plasma derived conagulation protein-Factor XI	n/a	4/30/2014	For the treatment of congenital Factor XI deficiency	Cambryn Biologics
	Human prothrombin complex concentrate	Octaplex	2/1/2008	Reversal of anticoagulation therapy in patients needing treatment of serious or life threatening bleeding and/or needing urgent surgery or invasive procedures	Octapharma USA, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Human telomerase reverse transcriptase peptide vaccine	n/a	7/20/2006	Treatment of pancreatic cancer	GemVax A/S
	Humanized 3F8-IgG1 monoclonal antibody	n/a	6/24/2013	Treatment of neuroblastoma	Memorial Sloan-Kettering Cancer Center
	Humanized anti-CD3 monoclonal antibody	n/a	9/29/2006	Treatment of recent-onset Type I diabetes	MacroGenics, Inc.
	Humanized anti-tac	Zenapax	3/5/1993	Prevention of acute graft-vs-host disease following bone marrow transplantation.	Hoffmann-La Roche, Inc.
	Humanized monoclonal antibody to TumorEndothelial Marker-1	n/a	4/29/2011	Treatment of soft tissue sarcoma	Morphotek

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Hydralazine	n/a	4/9/2004	Treatment of severe intrapartum hypertension (diastolic blood pressure greater than or equal to 110 or systolic blood pressure greater than or equal to 160) associated with severe preeclampsia/eclampsia of pregnancy	Bioniche Pharma USA LLC
	Hydroxocobalamin	n/a	9/22/2000	Treatment of acute cyanide poisoning	Jazz Pharmaceuticals
	Hydroxocobalamin	Cyanokit	11/25/2003	Treatment of acute cyanide poisoning	SERB, S.A.
	Hydroxy-Propyl-Beta-Cyclodextrin	Trappsol	5/17/2010	Treatment of Niemann Pick Disease, Type C	Sphingo Biotech, LLC
	Hydroxyurea	Droxia	10/1/1990	Treatment of patients with sickle cell anemia as shown by the presence of hemoglobin S.	Bristol-Myers Squibb Pharmaceutical Research Institute

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Hypericin	n/a	2/7/2000	Treatment of cutaneous T-cell lymphoma.	Soligenix, Inc.
	Hypericin	n/a	8/3/2000	Treatment of glioblastoma multiforme.	HyBiopharma, Inc.
	Hypothiocyanite and lactoferrin	Meveol	11/5/2009	Treatment of cystic fibrosis	Alaxia Biotechnologies Sante
	I(131)-TM-601 (chlorotoxin)	n/a	2/14/2002	Treatment of malignant glioma	Morphotek, Inc.
	IDUA-HIRMAb fusion protein	Agt-181	1/10/2008	Treatment of mucopolysaccharidosis Type 1 (MPS)	ArmaGen Technologies, Inc.
	IL-12 secreting dendritic cells loaded with autologous tumor lysate	n/a	6/24/2013	Treatment of malignant glioma	Activartis Biotech GmbH
	IL-4 Pseudomonas Toxin Fusion Protein (IL-4(38-37)-PE38KDEL)	n/a	4/6/2000	Treatment of astrocytic glioma.	Medicenna Therapeutics, Inc.
	Ibuprofen i.v. solution	Salprofen	10/29/1996	Prevention of patent ductus arteriosus.	Farmacon-IL, LLC
	Ibuprofen lysine	Neoprofen	10/29/1996	Treatment of patent ductus arteriosus	Lundbeck, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Icodextrin 7.5% with Electrolytes Peritoneal Dialysis Solution	Extraneal (With 7.5% Icodextrin) Peritoneal Dialysis Solutio	7/18/1997	Treatment of those patients having end stage renal disease and requiring peritoneal dialysis treatment.	Baxter Healthcare Corporation
	Idarubicin HCl for injection	Idamycin	7/25/1988	Treatment of acute myelogenous leukemia, also referred to as acute nonlymphocytic leukemia.	Adria Laboratories, Inc.
	Idoxuridine	n/a	4/8/1996	Treatment of nonparenchymatous sarcomas.	NeoPharm, Inc.
	Ifosfamide	Ifex	1/20/1987	Treatment of testicular cancer.	Bristol-Myers Squibb Pharmaceutical Research Institute
	IgG1 chimeric monoclonal antibody	n/a	6/22/2011	Treatment of multiple myeloma.	Immune System Therapeutics Ltd
	Iloprost inhalation solution	Ventavis	8/17/2004	Treatment of pulmonary arterial hypertension	CoTherix, Inc.
	Imatinib	Gleevec	1/31/2001	Treatment of chronic myelogenous leukemia	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Imatinib mesylate	Gleevec	8/25/2005	Treatment of idiopathic hypereosinophilic syndrome including acute and chronic eosinophilic leukemia	Novartis Pharmaceuticals Corporation
	Imatinib mesylate	Gleevec	10/5/2005	Treatment of myeloproliferative disorders/myelodysplastic syndromes associated with platelet-derived growth factor gene rearrangements	Novartis Pharmaceuticals Corporation
	Imatinib mesylate	Gleevec	9/9/2005	Treatment of systemic mastocytosis without the D816V c-kit mutation	Novartis Pharmaceuticals Corporation
	Imciromab pentetate	Myoscint	1/25/1989	Detecting early necrosis as an indication of rejection of orthotopic cardiac transplants.	Centocor, Inc.
	Imexon	n/a	11/8/1996	Treatment of multiple myeloma.	AmpliMed Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Imiglucerase	Cerezyme	11/5/1991	Replacement therapy in patients with types I, II, and III Gaucher's disease.	Genzyme Corporation
	Imiquimod	n/a	12/3/2014	Treatment of carcinoma in situ (CIS) of the urinary bladder	Telormedix SA
	Immortalized human liver cells found in the extracorporeal liver assist device	Elad	7/16/2004	Treatment of fulminant hepatic failure (acute liver failure)	Vital Therapies, Inc.
	Immune Globulin (Human)	Gamunex(R)-C	7/27/2004	Treatment of chronic inflammatory demyelinating polyneuropathy	Grifols Therapeutics, Inc.
	Immune Globulin (Human) containing high titers of West Nile virus antibodies	Omr-Igg-Am (Tm) 5% (Wnv)	3/17/2004	Treatment of the West Nile virus infection	OMRIX Biopharmaceuticals, Ltd.
	Immune Globulin Intravenous (human)	Carimune Nf	5/4/2004	Treatment for Guillain Barre Syndrome	ZLB Bioplasma AG

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Immune Globulin Subcutaneous (Human), 20% Liquid	Hizentra	8/18/2014	Treatment of chronic inflammatory demyelinating polyneuropathy (CIDP)	CSL Behring
	Immune globulin intravenous, human	Gamimune N	2/18/1993	Infection prophylaxis in pediatric patients affected with the human immunodeficiency virus.	Bayer Corporation
	Immunotherapeutic vaccine consisting of (PAM)2-Lys-Ser-Ser-Gln-Tyr-Ile-Lys-Ala-Asn-Ser-Lys-Phe-Ile-Gly-Ile-Thr-Glu-Ala-Ala-Ala-Phe-Leu-Pro-Ser-Asp-Phe-Phe-Pro-Ser-Val	n/a	3/16/1994	Treatment of chronic active hepatitis B infection in HLA-A2 positive patients.	Cytel Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Imported fire ant venom, allergenic extract	n/a	5/12/1992	For skin testing of victims of fire ant stings to confirm fire ant sensitivity and if positive, for use as immunotherapy for the prevention of IgE-mediated anaphylactic reactions.	ALK Laboratories, Inc.
	Inalimarev and falimarev	n/a	1/10/2006	Treatment of adenocarcinoma of the pancreas	Therion Biologics Corporation
	Indium-111 pentetretotide	Neuroendomedix	6/16/2006	Treatment of neuroendocrine tumors	Radiolotope Therapy of America (RITA) Foundation
	Inecalcitol	n/a	5/14/2014	Treatment of chronic lymphocytic leukemia	Hybrigenics, S.A.
	Infliximab	Remicade	5/21/2003	Treatment of chronic sarcoidosis	Centocor, Inc.
	Infliximab	Remicade	11/12/2003	Treatment of pediatric (0 to 16 years of age) Crohn's Disease	Centocor, Inc.
	Inhibitor of Tissue Factor Pathway Inhibitor (TFPI)	n/a	1/29/2010	Treatment of hemophilia A and hemophilia B.	Baxter Healthcare Corporation, Baxter BioScience

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Inhibitor of primary and secondary KIT and PDGFR alpha kinase mutants found in GIST	n/a	10/2/2014	Treatment of gastrointestinal stromal tumors (GIST)	Deciphera Pharmaceuticals, LLC
	Inosine pranobex	Isoprinosine	9/20/1988	Treatment of subacute sclerosing panencephalitis.	Newport Pharmaceuticals
	Interferon alfa-2a	Roferon A	6/6/1989	Treatment of chronic myelogenous leukemia.	Hoffmann-La Roche, Inc.
	Interferon alfa-2a (recombinant)	Roferon-A	5/11/1990	For the treatment of metastatic malignant melanoma in combination with Teceleukin.	Hoffmann-La Roche, Inc.
	Interferon alfa-2a (recombinant)	Roferon-A	4/18/1988	Treatment of renal cell carcinoma.	Hoffmann-La Roche, Inc.
	Interferon alfa-2a (recombinant)	Roferon-A	12/14/1987	Treatment of AIDS related Kaposi's sarcoma.	Hoffmann-La Roche, Inc.
	Interferon alfa-2a (recombinant)	Roferon-A	10/27/1989	For use in combination with fluorouracil for the treatment of esophageal carcinoma.	Hoffmann-La Roche, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Interferon alfa-2a (recombinant)	Roferon-A	5/2/1990	For the concomitant administration with Teceleukin for the treatment of metastatic renal cell carcinoma.	Hoffmann-La Roche, Inc.
	Interferon alfa-2a (recombinant)	Roferon-A	5/14/1990	For the concomitant administration with fluorouracil for the treatment of advanced colorectal cancer.	Hoffmann-La Roche, Inc.
	Interferon alfa-2b (recombinant)	Intron A	6/24/1987	Treatment of AIDS-related Kaposi's sarcoma.	Schering Corporation
	Interferon beta-1a	Avonex	12/16/1991	Treatment of multiple sclerosis.	Biogen, Inc.
	Interferon beta-1a	Rebif	3/11/1996	Treatment of patients with secondary progressive multiple sclerosis.	EMD Serono, Inc.
	Interferon beta-1a (recombinant human)	n/a	7/24/1992	Treatment of acute non-A, non-B hepatitis.	Biogen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Interferon beta-1a (recombinant)	Rebif	12/2/1992	Treatment of symptomatic patients with AIDS including all patients with CD4 T-cell counts less than 200 cells per mm <sup>3</sup> .	EMD Serono, Inc.
	Interferon beta-1b	Betaseron	11/17/1988	Treatment of multiple sclerosis.	Chiron Corp. & Berlex Laboratories
	Interferon gamma	n/a	10/11/2007	Treatment of idiopathic pulmonary fibrosis	mondoBIOTECH Laboratories AG
	Interferon gamma 1-b	Actimmune	9/30/1988	Treatment of chronic granulomatous disease.	Horizon Pharma Ireland Limited
	Interferon gamma-1b	Actimmune	12/4/1995	Treatment of renal cell carcinoma.	Horizon Pharma Ireland Limited
	Interferon gamma-1b	Actimmune	9/30/1996	Delaying time to disease progression in patients with severe, malignant osteopetrosis.	Horizon Pharma Ireland Limited
	Interferon-alfa-1b	n/a	4/17/2001	Treatment of multiple myeloma	Ernest C.Borden
	Interleukin-1 Trap	n/a	4/4/2005	Treatment of Still's disease including juvenile rheumatoid arthritis and adult-onset Still's disease	Regeneron Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Interleukin-1 receptor antagonist, human recombinant	Antril	10/16/1992	Prevention and treatment of graft versus host disease in transplant recipients.	Swedish Orphan Biovitrum AB (publ) (SOBI)
	Interleukin-1 receptor antagonist, human recombinant	Antril	9/23/1991	Treatment of juvenile rheumatoid arthritis.	Swedish Orphan Biovitrum AB (publ) (SOBI)
	Interleukin-2	Teceleukin	2/5/1990	Treatment of metastatic renal cell carcinoma.	Hoffmann-La Roche, Inc.
	Interleukin-2	Teleleukin	2/6/1990	Treatment of metastatic malignant melanoma.	Hoffmann-La Roche, Inc.
	Interleukin-2	Teceleukin	5/11/1990	In combination with interferon alfa-2a for the treatment of metastatic malignant melanoma.	Hoffmann-La Roche, Inc.
	Interleukin-2	Teceleukin	5/3/1990	In combination with interferon alfa-2a for the treatment of metastatic renal cell carcinoma.	Hoffmann-La Roche, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Intraoral fluoride releasing system	Ifrs	7/31/2001	Prevention of dental caries due to radiation-induced xerostomia in patients with head and neck cancer	Digestive Care, Inc.
	Intravenous immune globulin	Vitigam	7/26/2007	Treatment of Stage IIB to IV malignant melanoma	GammaCan Ltd.
	Intravenous immune globulin (human) 10%	Octagam(R)	7/31/2008	Treatment of stiff-person syndrome	Octapharma USA, Inc.
	Iobenguane I 123	Adreview	12/1/2006	For the diagnosis of neuroblastomas	GE Healthcare, Inc.
	Iobenguane I 123	Adreview	12/1/2006	For the diagnosis of pheochromocytomas	GE Healthcare, Inc.
	Iobenguane I 131	Azedra Ultratrace	1/18/2006	Treatment of neuroendocrine tumors	Molecular Insight Pharmaceuticals
	Iobenguane Sulfate I-123	Omaclear	10/17/2005	For scintigraphic detection, localization and staging of neuroblastomas.	Brogan Pharmaceuticals, LLC
	Iodine 131 6B-iodomethyl-19-norcholesterol	n/a	8/1/1984	For use in adrenal cortical imaging.	David E. Kuhl, M.D.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Iodine I 123 murine monoclonal antibody to hCG	n/a	11/7/1988	Detection of hCG producing tumors such as germ cell and trophoblastic cell tumors.	Immunomedics, Inc.
	Iodine I 131 bis(indium-diethylenetriamin epentaacetic acid)tyrosyllysine /hMN-14 x m734 F(ab')2 bispecific monoclonal antibody	Pentacea	2/22/2000	Treatment of small-cell lung cancer.	IBC Pharmaceuticals, L.L.C.
	Iodine I-131 radiolabeled chimeric MAb tumor necrosis treatment (TNT-1B)	131ichtnt-1	2/12/1999	Treatment of glioblastoma multiforme and anaplastic astrocytoma.	Peregrine Pharmaceuticals, Inc.
	lpatasertib	n/a	4/30/2014	For the treatment of gastric cancer including cancer of the gastro-esophageal junction.	Genentech, Inc.
	Isobutyramide	n/a	5/25/1994	Treatment of sickle cell disease and beta thalassemia.	Alpha Therapeutic Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Isobutyramide	Isobutyramide Oral Solution	12/18/1992	Treatment of beta-hemoglobinopathies and beta-thalassemia syndromes.	Perrine, Susan P., M.D.
	Itraconazole suspension	n/a	10/30/2008	Topical treatment of fungal otitis externa (otomycosis)	Fairfield Clinical Trials, LLC
	Japanese encephalitis vaccine (live, attenuated)	n/a	5/19/1999	Prevention of Japanese encephalitis.	Glovax Co., Ltd.
	Japanese encephalitis vaccine, inactivated, adsorbed	Ixiaro	9/25/2012	Prevention of Japanese encephalitis virus in pediatric patients.	Intercell AG
	Kre-Celazine	n/a	4/1/2013	Treatment of juvenile rheumatoid arthritis joint and related tissue inflammation in the pediatric population	All American Pharmaceutical & Natural Foods Corpor
	L-2-oxothiazolidine-4-carboxylic acid	Procysteine	6/14/1994	Treatment of adult respiratory distress syndrome.	Transcend Therapeutics, Inc.
	L-2-oxothiazolidine-4-carboxylic acid	Procysteine	7/30/1996	Treatment of amyotrophic lateral sclerosis.	Transcend Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	L-5 Hydroxytryptophan	n/a	11/1/1984	Treatment of postanoxic intention myoclonus.	Watson Laboratories, Inc.
	L-5-hydroxytryptophan	n/a	1/20/1999	Treatment of tetrahydrobiopterin deficiency.	Watson Laboratories, Inc.
	L-amino acids, vitamins and minerals combined with omega-3 fatty acids	n/a	1/12/2011	Treatment of patients with pediatric Crohn's disease	Immunopath Profile, Inc.
	L-aminocarnityl-succinyl-leucyl-argininal-diethylacetal	n/a	1/18/2006	Treatment of Duchenne and Becker muscular dystrophy	CepTor Corporation
	L-arginyl-L-isoleucyl-L-valyl-L-prolyl-L-alanine-amide	n/a	9/11/2013	Treatment of acute radiation syndrome	Soligenix, Inc.
	L-asparaginase	n/a	3/27/2014	Treatment of acute myeloid leukemia	ERYTECH Pharma S.A.
	L-asparaginase encapsulated in red blood cells	Graspa	1/6/2010	Treatment of acute lymphoblastic leukemia	Erytech Pharma
	L-asparaginase encapsulated in red blood cells	Graspa	7/6/2012	Treatment of pancreatic cancer.	ERYTECH Pharma
	L-baclofen	Neuralgon	1/30/1992	Treatment of intractable spasticity in children with cerebral palsy.	Osmotica Pharmaceutical Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	L-baclofen	n/a	12/17/1991	Treatment of spasticity associated with spinal cord injury or multiple sclerosis.	Osmotica Pharmaceutical Corp.
	L-baclofen	n/a	7/13/1990	Treatment of trigeminal neuralgia.	Fromm, Gerhard M.D.
	L-baclofen	n/a	1/6/1998	Treatment of trigeminal neuralgia	Osmotica Pharmaceutical Corp.
	L-cycloserine	n/a	8/1/1989	Treatment of Gaucher's disease.	Lev, Meir M.D.
	L-cysteine	n/a	5/16/1994	For the prevention and lessening of photosensitivity in erythropoietic protoporphyria.	Brigham and Women's Hospital
	L-glutamine	n/a	8/1/2001	Treatment of sickle cell disease	Emmaus Medical, Inc.
	L-glutamyl-L-tryptophan	n/a	10/20/1999	Treatment of AIDS-related Kaposi's sarcoma.	Implicit Bioscience Pty Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	L-ornithine phenylacetate	n/a	4/7/2010	Treatment of hyperammonemia and resultant hepatic encephalopathy (HE) in patients with acute liver failure or acute on chronic liver disease	Ocera Therapeutics, Inc.
	L-pyr-L-glu-L-gln-L-leu-L-glu-L-arg-L-ala-L-leu-L-asn-L-ser-L-ser	n/a	9/18/2009	Prevention of delayed graft function following renal transplant	Araim Pharmaceuticals, Inc.
	L-pyr-L-glu-L-gln-L-leu-L-glu-L-arg-L-ala-L-leu-L-asn-L-ser-L-ser	n/a	9/13/2011	Treatment of neuropathic pain in patients with sarcoidosis.	Araim Pharmaceuticals, Inc.
	L-threonyl-L-prolyl-L-prolyl-L-threonine	n/a	4/26/2005	Treatment of neuropathic pain associated with spinal cord injury	Nyxis Neurotherapies, Inc.
	L-tyrosine-L-serine-L-leucine	Cms-024	9/10/2004	Treatment of hepatocellular carcinoma.	CMS Peptides Patent Holding Company Limited
	L. reuteri	n/a	8/1/2013	Prevention of necrotizing enterocolitis in preterm infants with birth weight less than or equal to 1,500 grams	Infant Bacterial Therapeutics

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	LOAd703, a modified adenovirus 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand (TMZ-CD40L) and full length 4-1BBL	n/a	5/4/2015	Treatment of pancreatic cancer	Lokon Pharma AB
	Lactic acid	Aphthaid	6/29/1999	Treatment of severe aphthous stomatitis in severely, terminally immunocompromised patients.	Frontier Pharmaceutical, Inc.
	Lactobacillus acidophilus and Bifidobacterium animalis subsp. lactis	n/a	3/24/2015	Prevention of necrotizing enterocolitis (NEC) in premature infants with very low birth weight less than or equal to 1500 grams	Sigma-Tau Pharmaceuticals, Inc.
	Lamotrigine	Lamictal	8/23/1995	Treatment of Lennox-Gastaut syndrome.	Glaxo Wellcome Research and Development
	Lanreotide	Somatuline Depot	9/11/2000	Treatment for acromegly	IPSEN, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Laromustine	Cloretazine	3/27/2014	Treatment of acute myelogenous leukemia	Nanotherapeutics, Inc.
	Latrodectus immune F(ab)2	Analatro	6/18/2001	Treatment of black widow spider envenomations	Instituto Bioclon, S.A. de C.V.
	Leflunomide	n/a	10/18/1996	Prevention of acute and chronic rejection in patients who have received solid organ transplants.	Williams, MD, James W.
	Lentiviral vector containing the human ABCA4 gene	Stargen (Tm)	4/30/2010	Treatment of Stargardt disease	Sanofi US Services, Inc.
	Lentiviral vector containing the human MYO7A gene	Ushstat(Tm)	5/17/2010	Treatment of retinitis pigmentosa associated with Usher syndrome 1B gene defect.	Sanofi US Services, Inc.
	Lentiviral vector encoded with a human beta-globin gene plasmid	Thalagen	1/11/2006	Treatment of beta-thalassemia major and beta-thalassemia intermedia	Memorial Sloan-Kettering Cancer Center
	Lepirudin	Refluden	2/13/1997	Treatment of heparin-associated thrombocytopenia type II.	Hoechst Marion Roussel

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Lestaurtinib	n/a	3/24/2006	Treatment of acute myeloid leukemia	Teva Branded Pharmaceutical Products R&D, Inc.
	Leucovorin	Leucovorin Calcium	12/8/1986	For use in combination with 5-fluorouracil for the treatment of metastatic colorectal cancer.	Immunex Corporation
	Leucovorin	Leucovorin Calcium	8/17/1988	For rescue use after high dose methotrexate therapy in the treatment of osteosarcoma.	Immunex Corporation
	Leupeptin	n/a	9/18/1990	For use as an adjunct to microsurgical peripheral nerve repair.	Neuromuscular Adjuncts, Inc.
	Leuprolide acetate	Lupron Injection	7/25/1988	Treatment of central precocious puberty	Tap Pharmaceuticals, Inc.
	Levocarnitine	Carnitor	2/28/1984	Treatment of genetic carnitine deficiency.	Sigma-Tau Pharmaceuticals, Inc.
	Levocarnitine	Carnitor	7/26/1984	Treatment of primary and secondary carnitine deficiency of genetic origin.	Sigma-Tau Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Levocarnitine	Carnitor	9/6/1988	Treatment of manifestations of carnitine deficiency in patients with end stage renal disease who require dialysis.	Sigma-Tau Pharmaceuticals, Inc.
	Levocarnitine	Carnitor	4/7/1997	Treatment of zidovudine-induced mitochondrial myopathy.	Sigma-Tau Pharmaceuticals, Inc.
	Levocarnitine	Carnitor	11/22/1993	Treatment of pediatric cardiomyopathy.	Sigma-Tau Pharmaceuticals, Inc.
	Levodopa and carbidopa	Duodopa	1/18/2000	Treatment of late stage Parkinson's disease	AbbVie, Inc.
	Levofloxacin	n/a	2/27/2008	Treatment of pulmonary infections due to Pseudomonas aeruginosa and other bacteria in patients with cystic fibrosis patients	Forest Research Institute, Inc.
	Levoleucovorin	Fusilev	8/1/1991	For use in conjunction with high-dose methotrexate in the treatment of osteosarcoma.	Spectrum Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Levomethadyl acetate hydrochloride	Orlaam	1/24/1985	Treatment of heroin addicts suitable for maintenance on opiate agonists.	Biodevelopment Corporation
	Lidocaine patch 5%	Lidoderm Patch	10/24/1995	For relief of allodynia (painful hypersensitivity), and chronic pain in postherpetic neuralgia.	Teikoku Pharma USA, Inc.
	Liothyronine sodium injection	Triostat	7/30/1990	Treatment of myxedema coma/precoma.	SmithKline Beecham Pharmaceuticals
	Liposomal Glutathione	n/a	4/30/2010	Support of glutathione deficiency in individuals with inborn errors of metabolism of glutathione (IEMG)	Your Energy Systems, LLC
	Liposomal N-Acetylglucosminyl-N-Acetylmuramyl-L-Ala-D-isoGln-L-Ala-glycerolipalmitoyl	Immther	6/10/1998	Treatment of osteosarcoma.	Endorex Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Liposomal N-Acetylglucosminyl-L-N-Acetylmuramyl-L-Ala-D-isoGln-L-Ala - glycerolipalmitoyl	Immther	6/10/1998	Treatment of Ewing's sarcoma.	Endorex Corp.
	Liposomal amphotericin B	Ambisome	12/10/1996	Treatment of cryptococcal meningitis.	Fujisawa USA, Inc.
	Liposomal amphotericin B	Ambisome	12/6/1996	Treatment of visceral leishmaniasis.	Fujisawa USA, Inc.
	Liposomal amphotericin B	Ambisome	12/10/1996	Treatment of histoplasmosis.	Fujisawa USA, Inc.
	Liposomal annamycin	n/a	6/17/2005	Treatment of acute lymphoblastic leukemia	Callisto Pharmaceuticals, Inc.
	Liposomal annamycin	n/a	6/17/2005	Treatment of acute myeloid leukemia	Callisto Pharmaceuticals, Inc.
	Liposomal ciprofloxacin for inhalation	n/a	4/19/2006	Management of cystic fibrosis	Aradigm Corporation
	Liposomal ciprofloxacin for inhalation	n/a	12/27/2006	Management of bronchiectasis	Aradigm Corporation
	Liposomal cisplatin	Lipova-Pt	5/23/2006	Treatment of ovarian cancer	Eleison Pharmaceuticals LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Liposomal cyclosporin A	Cyclospire	4/30/1998	For aerosolized administration in the prevention and treatment of lung allograft rejection and pulmonary rejection events associated with bone marrow transplantation.	Vernon Knight, M.D.
	Liposomal doxorubicin hydrochloride	Sarcodoxome	12/27/2006	Treatment of soft tissue sarcomas	GP-Pharm SA
	Liposomal nystatin	Nyotran	6/13/2000	Treatment of invasive fungal infections.	The University of Texas
	Liposome encapsulated recombinant interleukin-2	n/a	11/25/1991	Treatment of brain and CNS tumors.	Oncothyreon Canada, Inc.
	Liposome encapsulated recombinant interleukin-2	n/a	6/20/1994	Treatment of cancers of the kidney and renal pelvis	Oncothyreon Canada, Inc.
	Lisofylline	n/a	6/10/1999	Treatment of patients undergoing induction therapy for acute myeloid leukemia.	Cell Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Live-attenuated Listeria monocytogenes actA/inlB/human mesothelin (hMeso) Strain hMeso38	Not Determined	3/24/2015	Treatment of mesothelioma.	Aduro BioTech, Inc.
	Lodoxamide tromethamine	Alomide Ophthalmic Solution	10/16/1991	Treatment of vernal keratoconjunctivitis.	Alcon Laboratories, Inc.
	Lonafarnib	n/a	11/19/2013	Treatment of Hepatitis Delta Virus (HDV)infection	Eiger Biopharmaceuticals, Inc.
	Lucinactant	Surfaxin	7/17/1995	Treatment of acute respiratory distress syndrome in adults.	Discovery Laboratories, Inc.
	Lucinactant	Surfaxin(R), Aerosurf(R)	10/21/2010	Treatment of cystic fibrosis.	Discovery Laboratories, Inc.
	Lucinactant	Surfaxin	10/21/2005	Treatment of bronchopulmonary dysplasia in premature infants.	Discovery Laboratories, Inc.
	Lucinactant	Surfaxin	5/23/2006	Prevention of bronchopulmonary dysplasia in premature infants	Discovery Laboratories, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Lucinactant	Surfaxin	10/18/1995	Treatment of respiratory distress syndrome in premature infants.	Discovery Laboratories, Inc.
	MA09-hRPE cells	n/a	2/2/2010	Treatment of Stargardt's macular dystrophy.	Advanced Cell Technology, Inc.
	MAGE-A3 cancer therapeutic Trojan peptide vaccine	n/a	11/24/2008	Treatment of MAGE-A3 expressing head and neck squamous cell carcinoma	Gliknik, Inc.
	MLN4924- Inhibitor of Nedd8-activating enzyme (NAE)	n/a	2/4/2011	Treatment of acute myelogenous leukemia.	Millennium Pharmaceuticals
	MLN4924- Inhibitor of Nedd8-activating enzyme (NAE)	n/a	2/4/2011	Treatment of Myelodysplastic syndrome	Millennium Pharmaceuticals, Inc.
	MN14 monoclonal antibody to carcinoembryonic antigen	Cea-Cide	9/18/1998	Treatment of small cell lung cancer	Immunomedics, Inc.
	MN14 monoclonal antibody to carcinoembryonic antigen	Cea-Cide	11/24/1998	Treatment of pancreatic cancer.	Immunomedics, Inc.
	MOD-423 hGH analogue	n/a	9/29/2010	Treatment of growth hormone deficiency	OPKO Biologics Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	MTC-DOX for Injection	n/a	1/3/2001	Treatment of hepatocellular carcinoma	FeRx Incorporated
	MV-NB-02, its bivalent ganglioside vaccine consisting of GD2-lactone and GD3-lactone each covalently conjugated to keyhole hemocyanin	n/a	9/23/2014	Treatment of Neuroblastoma	MabVax Therapeutics, Inc.
	Mafenide acetate solution	Sulfamylon Solution	7/18/1990	For use as an adjunctive topical antimicrobial agent to control bacterial infection when used under moist dressings over meshed autografts on excised burn wounds.	Mylan Laboratories, Inc.
	Mafosfamide	n/a	1/21/2003	Treatment of neoplastic meningitis	Baxter Healthcare Corporation
	Manganese superoxide dismutase mimetic	n/a	2/27/2008	Prevention of radiation- or chemotherapy-induced oral mucositis in cancer patients	Galera Therapeutics, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Mannitol	Bronchitol	2/11/2005	For use to facilitate clearance of mucus in patients with bronchiectasis and in patients with cystic fibrosis at risk for bronchiectasis	Pharmaxis Ltd.
	Marijuana	n/a	5/25/1999	Treatment of HIV-associated wasting syndrome.	Multidisciplinary Association for Psychedelic Studies, Inc.
	Marizomib	n/a	12/13/2013	Treatment of multiple myeloma	Triphase Research and Development I Corp.
	Matrix metalloproteinase inhibitor	Galardin	12/5/1991	Treatment of corneal ulcers.	Glycomed, Inc
	MaxAdFVIII	n/a	3/3/2003	Treatment of Hemophilia A	GenStar Therapeutics Corporation
	Mazindol	Sanorex	12/8/1986	Treatment of Duchenne muscular dystrophy.	Collipp, Platon J. M.D.
	Mecamylamine	Inversine	10/14/1998	Treatment of Tourette's syndrome.	Targacept, Inc.
	Mecasermin	Increlex	12/12/1995	Treatment of growth hormone insensitivity syndrome.	Ipsen Biopharmaceuticals, Inc.
	Medroxyprogesterone acetate	Hematrol	2/22/2001	Treatment of immune thrombocytopenic purpura.	ZaBeCor Pharmaceutical Company, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Mefloquine HCl	Mephaquin	7/22/1987	Treatment and prevention of chloroquine-resistant Falciparum malaria	Mepha AG
	Megestrol acetate	Megace	4/13/1988	Treatment of patients with anorexia, cachexia, or significant weight loss (= $>$ 10% of baseline body weight) and confirmed diagnosis of AIDS.	Bristol-Myers Squibb Pharmaceutical Research Institute
	Melanoma autologous dendritic cell vaccine	n/a	9/6/2006	Treatment of stage IIIb through IV metastatic melanoma	Caladrius Biosciences
	Melanoma cell vaccine	Canvaxin	10/13/1994	Treatment of invasive melanoma.	CancerVax Corporation
	Melanoma peptide vaccine	n/a	3/29/2005	Treatment of HLA-A2+ patients with stage IIB, IIC, III, and IV malignant melanoma	Bristol-Myers Squibb Research Inst
	Melanoma vaccine	Melacine	12/20/1989	Treatment of stage III - IV melanoma.	Ribi ImmunoChem Research, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Melatonin	n/a	11/15/1993	Treatment of circadian rhythm sleep disorders in blind people with no light perception.	Sack, Robert, M.D.
	Melatonin	Circadin	7/9/2004	Treatment of non-24-hour sleep-wake disorder in blind individuals without light perception	Neurim Pharmaceuticals, Ltd.
	Melphalan	Alkeran For Injection	2/24/1992	Treatment of patients with multiple myeloma for whom oral therapy is inappropriate.	Glaxo Wellcome Inc.
	Menadione Sodium Bisulfite	n/a	5/14/2014	For the treatment of autosomal dominant polycystic kidney disease	IC-MedTech Corporation
	Mesna	Mesnex	11/14/1985	For use as a prophylactic agent in reducing the incidence of ifosfamide-induced hemorrhagic cystitis.	Degussa Corporation
	Methionine/L-methionine	n/a	8/21/1996	Treatment of AIDS myelopathy.	Genopia USA, Inc.



**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Metronidazole (topical)	Metrogel	10/22/1987	Treatment of acne rosacea.	Galderma Laboratories, Inc.
	Metronidazole 10% ointment	n/a	11/8/2007	Topical treatment of active perianal Crohn's disease	SLA Pharma (UK) Ltd
	Microbubble contrast agent	Filmix Neurosonographic Contrast Agent	11/16/1990	Intraoperative aid in the identification and localization of intracranial tumors.	Cav-Con, Inc.
	Microvesiculated modified glycosylated tissue factor	n/a	1/25/2007	Treatment of non-life threatening, mild to severe bleeding episodes due to cutaneous injuries or dental procedures in hemophiliac patients	Thrombotargets Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Microvesiculated modified glycosylated tissue factor	n/a	10/11/2007	Treatment of non-life threatening, mild to severe bleeding episodes due to cutaneous injuries or dental procedures in patients with von Willebrand disease.	Thrombotargets Corp.
	Midodrine HCl	Amatine	6/21/1985	Treatment of patients with symptomatic orthostatic hypotension.	Shire
	Midostaurin	Rydapt	4/30/2010	Treatment of mastocytosis	Novartis Oncology
	Midostaurin	n/a	7/7/2009	Treatment of acute myeloid leukemia	Novartis Pharmaceuticals Corporation
	Mifepristone	n/a	2/7/2005	Treatment of Cushing's syndrome secondary to ectopic ACTH secretion	HRA Pharma
	Minnelide	Minnelide (Tm)	2/18/2013	Treatment of pancreatic cancer	Minneamrita Therapeutics, LLC
	Minocycline hydrochloride	n/a	3/28/2006	Treatment of sarcoidosis	Autoimmunity Research Foundation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Mitoguazone	Apep	3/18/1994	Treatment of diffuse non-Hodgkin's lymphoma, including AIDS-related diffuse non-Hodgkin's lymphoma.	ILEX Oncology, Inc.
	Mitolactol	n/a	7/12/1995	As adjuvant therapy in the treatment of primary brain tumors.	Targent, Inc.
	Mitolactol	n/a	1/23/1989	Treatment of invasive carcinoma of the uterine cervix	Targent, Inc..
	Mitomycin-C	n/a	8/20/1993	Treatment of refractory glaucoma as an adjunct to ab externo glaucoma surgery.	IOP Inc.
	Mitoxantrone	Novantrone	8/21/1996	Treatment of hormone refractory prostate cancer.	Serono
	Mitoxantrone	Novantrone	8/13/1999	Treatment of progressive-relapsing multiple sclerosis.	Serono, Inc.
	Mitoxantrone	Novantrone	8/13/1999	Treatment of secondary-progressive multiple sclerosis.	Serono, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Mitoxantrone HCl	Novantrone	7/13/1987	Treatment of acute myelogenous leukemia, also referred to as acute nonlymphocytic leukemia.	Lederle Laboratories
	Mixture of 2 anti-rabies humanized monoclonal immunoglobulin (Ig)G1k antibodies	n/a	10/7/2015	Post-exposure prophylaxis against rabies virus infection	Synermore Biologics Co., Ltd
	Mocetinostat	n/a	6/12/2014	Treatment of myelodysplastic syndrome	Mirati Therapeutics, Inc.
	Modafinil	Provigil	3/15/1993	Treatment of excessive daytime sleepiness in narcolepsy.	Cephalon, Inc.
	Monoclonal antibody for immunization against lupus nephritis	n/a	1/7/1993	Treatment of lupus nephritis.	VivoRx Autoimmune, Inc.
	Monoclonal antibody-B43.13	Ovarex Mab-B43.13	11/25/1996	Treatment of epithelial ovarian cancer.	Quest PharmaTech, Inc.
	Monolaurin	Glylorin	4/29/1993	Treatment of congenital primary ichthyosis.	Glaxo Wellcome Inc.
	Mononuclear enriched fraction of human umbilical cord blood	U-Cord-Cell(R)	9/15/2015	Treatment of amyotrophic lateral sclerosis (ALS).	Saneron CCEL Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Monoctanoin	Moctanin	5/30/1984	For dissolution of cholesterol gallstones retained in the common bile duct.	Ethitek Pharmaceuticals, Inc.
	Morphine sulfate concentrate (preservative free)	Infumorph	7/12/1990	For use in microinfusion devices for intraspinal administration in the treatment of intractable chronic pain.	Elkins-Sinn, Inc.
	Multi-ligand somatostatin analog	n/a	7/27/2004	Treatment of patients with functional gastroenteropancreatic (GEP) neuroendocrine tumors (specifically, carcinoid, insulinoma, gastrinoma, somatostatinoma, GRFoma, VIPoma and glucagonoma.	Novartis Pharmaceuticals Corporation
	Multi-peptide cancer vaccine	n/a	6/23/2013	Treatment of multiple myeloma	OncoPep, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Multi-vitamin infusion (neonatal formula)	n/a	12/12/1989	For establishment and maintenance of total parenteral nutrition in very low birth weight infants.	Astra Pharmaceuticals, L.P.
	Multistem(r)	n/a	9/15/2010	Prophylaxis of graft vs host disease	Athersys, Inc.
	Murine MAb (Lym-1) and Iodine 131-I radiolabeled murine MAb (Lym-1) to human B-cell lymphoma	Oncolym	11/27/1998	Treatment of B-cell non-Hodgkin's lymphoma.	Peregrine Pharmaceuticals, Inc.
	Murine MAb to polymorphic epithelial mucin, human milk fat globule 1	Theragyn	3/22/1999	Adjuvant treatment of ovarian cancer.	Antisoma plc
	Mx-dnG1	Rexin-G(R)	6/24/2008	Treatment of soft tissue sarcoma	Epeius Biotechnologies Corporation
	Mx-dnG1	Rexin-G	6/24/2008	Treatment of osteosarcoma.	Epeius Biotechnologies Corporation
	Mx-dnG1 or Rexin-G retroviral vector	Rexin-G	8/15/2003	Treatment of pancreatic cancer	Epeius Biotechnologies Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Mycobacterium avium sensitin RS-10	n/a	10/11/1995	For use in the diagnosis of invasive Mycobacterium avium disease in immunocompetent individuals.	Statens Seruminstitut
	N'-(7-Chloroquinolin-4-yl)-N,N-diethylpropane-1,3-diamine	n/a	10/29/2015	Treatment of malaria.	Donald J. Krogstad, MD
	N,N'-bis(2-mercaptoethyl)isophthalamide (NBMI)	n/a	4/6/2012	Treatment of mercury toxicity	CTI Science Limited
	N-(2-((4Z,7Z,10Z,13Z,16Z,19Z)-docosa-4,7,10,13,16,19-hexaenamido)ethyl)-2-hydroxybenzamide	n/a	11/17/2014	Treatment of Duchenne muscular dystrophy	Catabasis Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-(2-amino-2-oxoethyl)-2-(2-((4-fluorophenethyl)amino)-N-isobutylacetamido)-N-(3-(2-oxopyrrolidin-1-yl)propyl)acetamide N-({Carbamoylmethyl-[3-(2-oxopyrrolidin-1-yl)propyl]-carbamoyl}-methyl)-2-[2-(2-fluorophenyl)-ethylamino]-N-isobutylacetamide	n/a	11/17/2014	Treatment of optic neuritis	Bionure Farma SL
	N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl]benzamide dihydrobromide	n/a	2/1/2008	Treatment of acute myeloid leukemia	Mirati Therapeutics, Inc.
	N-(2-amino-phenyl)-4-[(4-pyridin-3-yl-pyrimidin-2-ylamino)-methyl]benzamide dihydrobromide	n/a	8/8/2007	Treatment of Hodgkin's lymphoma	Mirati Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-(2-{2-dimethylaminoethyl-methylamino}-4-methoxy-5-[[4-(1-methylindol-3-yl)pyrimidin-2-yl]amino}phenyl)prop-2-enamide mesylate salt	n/a	9/4/2014	Treatment of epidermal growth factor receptor mutation-positive non-small cell lung cancer	AstraZeneca Pharmaceuticals LP
	N-(3,4-dihydroxyphenyl)-3,4-dihydroxybenzamide	n/a	10/15/2014	Treatment of AL Amyloidosis	ProteoTech, Inc.
	N-(3-fluorobenzyl)-2-(5-(4-morpholinophenyl)pyridin-2-yl)acetamide Besylate	n/a	11/26/2013	Treatment of gliomas	Kinex Pharmaceuticals, Inc.
	N-(4-Fluorobenzoyl)-L-arginyl-L-arginyl-[L-3-(naphthyl-alanyl)-L-cysteinyl-L-tyrosyl-L-citrullinyl-L-lysyl-D-lysyl-L-prolyl-L-trosyl-L-arginyl-L-citrullinyl-L-cysteinyl-L-arginine amide, cyclic (4-13)-disulfide	n/a	7/6/2012	For use in combination with G-CSF to mobilize HSCs from the marrow to peripheral blood for collection for autologous or allogeneic transplantation	BioLineRx, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-(4-{3-(2-aminopyrimidin-4-yl)pyridin-2-yl}oxy)phenyl)-4-(4-methylthiophen-2-yl)phthalazin-1-amine bis-mesylate dihydrate	n/a	10/21/2014	Treatment of ovarian cancer	Amgen, Inc.
	N-(5-tert-Butylisoxazol-3-yl)-N'-(4-[7-(2-(morpholin-4-yl)ethoxy)imidazo[2,1-b][1,3]benzothiazol-2-yl]phenyl)urea dihydrochloride salt	n/a	3/18/2009	Treatment of acute myeloid leukemia	Ambit Biosciences Corporation
	N-(9-methoxynonyl)-1-deoxynojirimycin hydrochloride	n/a	11/17/2014	Treatment of acute dengue illness (inclusive of acute dengue illness, dengue fever, dengue hemorrhagic fever, and dengue shock syndrome)	Unither Virology, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-(cyanomethyl)-4-(2-{{4-(morpholin-4-yl(phenyl)amino)pyrimidin-4-yl}benzamide	n/a	8/5/2010	Treatment of myelofibrosis	Gilead Sciences, Inc.
	N-(methyl-diazacyclohexylmethylbenzamide)-azaphenylaminothiopyrrole) mesylate	n/a	10/17/2005	Treatment of multiple myeloma.	AB Science
	N-Acetylmannosamine (ManNAc)	n/a	4/5/2010	Treatment of hereditary inclusion body myopathy type 2	Altamira Bio, Inc. (ABIO)
	N-Hydroxy-4-(3-methyl-2-(S)phenylbutyrylamino)benzamide	n/a	1/13/2012	Treatment of meningioma	Arno Therapeutics, Inc.
	N-Hydroxy-4-(3-methyl-2-(S)phenylbutyrylamino)benzamide	n/a	1/13/2012	Treatment of Schwannoma of the central nervous system	Arno Therapeutics, Inc.
	N-Methanocarbamidine	n/a	10/15/2014	Treatment of neonatal herpes	N & N Pharmaceuticals, Inc.
	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride	n/a	1/29/2010	Treatment of pancreatic cancer	EMD Serono, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride	n/a	1/12/2011	Treatment of acute myeloid leukemia (AML).	EMD Serono, Inc.
	N-[2,6-bis(1-methylethyl)phenyl]-N'-[[1-[4-(dimethylamino)phenyl]cyclopentyl]methyl]urea, hydrochloride salt	n/a	1/7/2015	For the treatment of Cushing's Syndrome	Atterocor, Inc.
	N-[3-(4',5'-bipyrimidin-2-ylamino)-4-methylphenyl]-4-[[{(3S)-3-(dimethylamino)pyrrolidin-1-yl]methyl}-3-(trifluoromethyl)benzamide	n/a	12/27/2006	Treatment of Philadelphia chromosome-positive chronic myelogenous leukemia.	CytRx Corporation
	N-[4-(trifluoromethyl)phenyl] 5-methylisoxazole-4-carboxamide	n/a	5/25/1995	Treatment of malignant glioma.	Sugen, Inc.
	N-[4-(trifluoromethyl)phenyl]-5-methylisoxazole-4-carboxamide	n/a	3/12/1996	Treatment of ovarian cancer.	Sugen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4-methylpiperazin-1yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide	n/a	2/3/2015	Treatment of TrkA-positive, TrkB-positive, TrkC-positive ROS1-positive or ALK-positive non-small cell lung cancer	Ignyta, Inc.
	N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4-methylpiperazin-1yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide	n/a	2/12/2015	Treatment of TrkA-positive, TrkB-positive, TrkC-positive, ROS1-positive, or ALK-positive colorectal cancer	Ignyta, Inc.
	N-acetyl cysteine amide	n/a	12/31/2013	Treatment of retinitis pigmentosa	Brighton Biotech, Inc.
	N-acetyl-glucosamine thiazoline	n/a	2/6/2006	Treatment of adult Tay-Sachs disease	ExSAR Corporation
	N-acetyl-procainamide	n/a	12/10/1996	Prevention of life-threatening ventricular arrhythmias in patients with documented procainamide-induced lupus.	NAPA of the Bahamas

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-acetylcysteinate Lysine	Nacystelyn Dry Powder Inhaler	12/27/2000	For the management of cystic fibrosis	Galephar Pharmaceutical Research, Inc.
	N-acetylcysteine	n/a	9/9/2002	Treatment of acute liver failure	Cumberland Pharmaceuticals, Inc.
	N-acetylcysteine	n/a	7/19/2012	Prevention of ototoxicity caused by platinum-based chemotherapeutic agents used to treat pediatric cancers	Edward A. Neuwelt, MD
	N-acetylcysteine and sodium thiosulfate	n/a	8/31/2015	Prevention of platinum-induced toxicities in pediatric patients (0 through 16 years of age).	Edward A. Neuwelt, MD
	N-acetylgalactosamine-4-sulfatase, recombinant human	Naglzyme	2/17/1999	Treatment of mucopolysaccharidosis Type VI (Maroteaux-Lamy syndrome).	BioMarin Pharmaceutical, Inc.
	N-adamantanyl-N'-Geranylethylenediamine	n/a	10/16/2007	Treatment of tuberculosis.	Sequella, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-methyl-4-({4- [[{3- methyl(methylsul fonyl)amino]pyra zin-2- yl}methyl)amino]- 5- (trifluoromethyl) pyrimidin-2- yl}amino)benzam ide hydrochloride	n/a	7/18/2013	Treatment of mesothelioma	Verastem, Inc.
	N-t- butylhydroxylami ne	n/a	5/12/2015	Treatment of infantile neuronal ceroid lipofuscinosis.	Andrew Lim
	N-tert-butyl-3-[(5- methyl-2-[[4-(2- pyrrolidin-1- ylethoxy)phenyl] amino}pyrimidin- 4-yl)amino] benzenesulfonam ide dihydrochloride monohydrate	n/a	3/21/2013	Treatment of polycythemia vera	sanofi-aventis U.S. LLC
	N-tert-butyl-3-[5- methyl-2-[[4-(2- pyrrolidin-1- ylethoxy)phenyl] amino}pyrimidin- 4-yl-amino] benzenesulfonam ide dihydrochloride monohydrate	n/a	5/18/2009	Treatment of secondary and primary myelofibrosis	Sanofi-Aventis US, LLC a Sanofi company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	N-{{(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl}methyl}acetamide	n/a	2/4/2011	Treatment of tuberculosis.	Sequella, Inc.
	N1,N14,-diethyl-3,12,-dihydroxyhomospermine	n/a	8/7/2014	Treatment of pancreatic cancer	Sun BioPharma, Inc.
	N2'-Deacetyl-N2'-[4-methyl-4-(oxobuthyldithio)-1-oxopentyl]-maytansine-chimerized anti-CD138 IgG4 Monoclonal Antibody	n/a	2/27/2008	Treatment of multiple myeloma	Biotest Pharmaceuticals Corporation
	N2'-deacetyl-N2'-(3-mercapto-1-oxopropyl)-Maystansine-Conjugated Humanized C242 Monoclonal Antibody	n/a	12/7/2000	For pancreatic cancer	SmithKline Beecham Pharmaceuticals
	NDX-peptides	n/a	12/14/2010	Treatment of amyotrophic lateral sclerosis	NeoDiagnostic Research Ltd
	Nafarelin acetate	Synarel Nasal Solution	7/20/1988	Treatment of central precocious puberty.	Syntex (USA), Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Naltrexone HCl	Trexan	3/11/1985	For blockade of the pharmacological effects of exogenously administered opioids as an adjunct to the maintenance of the opioid-free state in detoxified formerly opioid-dependent individuals.	DuPont Pharmaceuticals
	NanoDTPA(tm)	n/a	6/17/2011	Treatment of known or suspected cases of internal contamination with plutonium, americium, or curium	Nanotherapeutics, Inc.
	Natural human lymphoblastoid interferon-alpha	n/a	8/10/2000	Treatment of papillomavirus warts in the oral cavity of HIV positive patients.	Amarillo Biosciences, Inc.
	Natural human lymphoblastoid interferon-alpha	n/a	1/18/2000	Treatment of Behcet's disease	Amarillo Biosciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Nebacumab	Centoxin	10/1/1986	Treatment of patients with gram-negative bacteremia which has progressed to endotoxin shock.	Centocor, Inc.
	Neurotrophin-1	n/a	9/13/1994	Treatment of motor neuron disease/amyotrophic lateral sclerosis.	Ericsson, Arthur Dale, M.D.
	Neutrophil-endothelial interaction inhibitor	Cylexin	12/22/1993	Treatment of post-ischemic pulmonary reperfusion edema following surgical treatment for chronic thromboembolic pulmonary hypertension.	Cytel Corporation
	Nifedipine	n/a	6/13/1991	Treatment of interstitial cystitis.	Fleischmann, Jonathan M.D.
	Nikkomycin Z	n/a	2/14/2006	Treatment of coccidioidomycosis	Valley Fever Center for Excellence (1-111 INF)
	Nimotuzumab	n/a	11/17/2004	Treatment of glioma	InnoKeys PTE Ltd.
	Nine amino acid polypeptide derived from proteinase 3	n/a	9/3/2004	Treatment of acute myelogenous leukemia	The Vaccine Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Nine amino acid polypeptide derived from proteinase 3	n/a	9/3/2004	Treatment of myelodysplastic syndromes requiring therapy	The Vaccine Company
	Nine amino acid polypeptide derived from proteinase 3	n/a	9/3/2004	Treatment of chronic myelogenous leukemia.	The Vaccine Company
	Nitazoxanide	Alinia	12/12/1996	Treatment of cryptosporidiosis.	Romark Laboratories, L.C.
	Nitisinone	Orfadin	10/19/2001	Treatment of alkaptonuria	Swedish Orphan AB
	Nitisinone	Orfadin	5/16/1995	Treatment of tyrosinemia type 1.	Swedish Orphan Biovitrum AB (PUBL)
	Nitric oxide	n/a	2/16/2005	Diagnosis of sarcoidosis	SensorMedics Corporation
	Nitric oxide	n/a	9/11/2013	Treatment of cystic fibrosis	Novoteris, LLC
	Nitric oxide	Inomax	9/27/2004	To reduce the risk of chronic lung disease in premature neonates	INO Therapeutics
	Nitric oxide	Inomax	6/22/1993	Treatment of persistent pulmonary hypertension in the newborn.	INO Therapeutics, Inc.
	Nitric oxide	n/a	7/10/1995	Treatment of acute respiratory distress syndrome in adults.	INO Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Nitroprusside	n/a	2/21/2001	Treatment and prevention of cerebral vasospasm following subarachnoid hemorrhage.	Thomas, MD, Jeffrey Evan
	NorLeu3-Angiotensin(1-7)) [NorLeu3-A(1-7)]	n/a	12/31/2013	Treatment of leakage from the surgical site following penetrating keratoplasty	US Biotest, Inc.
	NorLeu3-Angiotensin(1-7) [NorLeu3-A(1-7)]	n/a	1/31/2014	Treatment of dermal injury due to nuclear/radiation incident	US Biotest, Inc.
	Nucleic acid aptamer binding to tumor cell nucleolin	n/a	9/10/2009	Treatment of acute myeloid leukemia	Antisoma, Inc.
	Nucleic acid aptamer binding to tumor cell nucleolin	n/a	8/17/2004	Treatment of pancreatic cancer	Antisoma Research Ltd.
	Nucleic acid aptamer binding to tumor cell nucleon	n/a	7/28/2005	Treatment of renal cell carcinoma	Antisoma Inc.
	O-(3-piperidino-2-hydroxyl-1-propyl)-nicotinic acid amidoxime hydrochloride	n/a	1/15/2013	Treatment of Duchenne Muscular Dystrophy	N-Gen Research Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	O2-(2,4-dinitrophenyl) 1-[(4-ethoxycarbonyl) piperazin-1-yl] diazen-1-ium-1,2-diolate	n/a	3/16/2012	Treatment of acute myeloid leukemia	JSK Therapeutics, Inc.
	O2-(2,4-dinitrophenyl) 1-[(4-ethoxycarbonyl) piperazin-1-yl] diazen-1-ium-1,2-diolate	n/a	12/13/2013	Treatment of multiple myeloma	JSK Therapeutics, Inc.
	OTL38; conjugate of pteroi acid and S0456 near infrared dye	n/a	12/23/2014	Detecting folic receptor alpha positive lesions in ovarian cancer patients (diagnostic for the management of ovarian cancer)	On Target Laboratories, LLC
	Octreotide	Sandostatin Lar	8/24/1998	Treatment of acromegaly.	Novartis Pharmaceuticals Corporation
	Octreotide	Sandostatin Lar	8/24/1998	Treatment of diarrhea associated with vasoactive intestinal peptide tumors (VIPoma).	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Octreotide	Sandostatin Lar	8/24/1998	Treatment of severe diarrhea and flushing associated with malignant carcinoid tumors.	Novartis Pharmaceuticals Corporation
	Ofloxacin	Ocuflox Ophthalmic Solution	4/18/1991	Treatment of bacterial corneal ulcers.	Allergan, Inc.
	Omega-3 (n-3) polyunsaturated fatty acid with all double bonds in the cis configuration	n/a	11/22/1995	Prevention of organ graft rejection.	Research Triangle Pharmaceuticals
	Omega-3 (n-3) polyunsaturated fatty acids	Omacor	5/4/2000	Treatment of IgA nephropathy.	Pronova Biocare, AS
	Omegaven emulsion	Omegaven	2/27/2008	Treatment of parenteral nutrition-associated liver disease	Fresenius Kabi Deutschland GmbH
	Oncolytic adenovirus coding for granulocyte macrophage colony stimulating factor (ONCOS-102)	n/a	12/22/2014	Treatment of malignant mesothelioma	Oncos Therapeutics
	Oprelvekin	Neumega	12/17/1996	Prevention of severe chemotherapy-induced thrombocytopenia.	Genetics Institute, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Oxaliplatin	n/a	10/6/1992	Treatment of ovarian cancer.	Debio Pharm S.A.
	Oxaloacetic acid	n/a	11/18/2015	Treatment of amyotrophic lateral sclerosis	Terra Biological LLC
	Oxalobacter formigenes	n/a	3/29/2006	Treatment of primary hyperoxaluria	OxThera, Inc.
	Oxandrolone	Oxandrin	9/6/1991	Adjunctive therapy for AIDS patients suffering from HIV-wasting syndrome.	Bio-Technology General Corp.
	Oxandrolone	Oxandrin	4/22/1997	Treatment of patients with Duchenne's muscular dystrophy and Becker's muscular dystrophy.	Savient Pharmaceuticals, Inc.
	Oxymorphone	Numorphan H.P.	3/19/1985	For relief of severe intractable pain in narcotic-tolerant patients.	DuPont Merck Pharmaceutical Company
	Oxypurinol	n/a	11/9/1998	Treatment of hyperuricemia in patients intolerant to allopurinol.	Cardiome Pharma Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	P140K MGMT transduced human CD34 cells	n/a	1/9/2013	For bone marrow protection in the treatment of glioblastoma multiforme	Lentigen Corporation
	PEG-b-PLA micelles containing (1) paclitaxel, (2) sirolimus (rapamycin) and (3) tanespimycin (17-AAG)(also known as: triolimus)	n/a	4/2/2015	Treatment of angiosarcoma	Co-D Therapeutics, Inc.
	PEG-glucocerebrosidase	Lysodase	12/9/1992	For use as chronic enzyme replacement therapy in patients with Gaucher's disease who are deficient in glucocerebrosidase.	National Institute of Mental Health, NIH
	PEG-interleukin-2	n/a	2/1/1990	Treatment of primary immunodeficiencies associated with T-cell defects.	Chiron Corporation
	PEGylated recombinant anti-Pseudomonas aeruginosa PcrV Fab' antibody	n/a	10/25/2013	Treatment of cystic fibrosis patients with Pseudomonas aeruginosa lung infections	KaloBios Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	PEGylated recombinant human hyaluronidase PH20	n/a	10/1/2014	Treatment of pancreatic cancer	Halozyme Therapeutics, Inc.
	PG2 (Astragalus polysaccharides extracted and purified from the dry root of Astragalus membranaceus)	n/a	7/5/2012	Treatment of idiopathic thrombocytopenic purpura	PhytoHealth Corporation
	PGC-C12E-terlipressin	n/a	12/27/2012	Treatment of ascites due to all etiologies except for cancer	PharmaIN Corporation
	Paclitaxel	Paxene	4/15/1997	Treatment of AIDS-related Kaposi's sarcoma.	Baker Norton Pharmaceuticals, Inc.
	Paclitaxel	Taxol	3/25/1997	Treatment of AIDS-related Kaposi's sarcoma.	Bristol-Myers Squibb Pharmaceutical Research Institute
	Pafuramidine maleate	n/a	5/14/2007	Treatment of malaria	Immtech Pharmaceuticals, Inc.
	Pafuramidine maleate	n/a	8/31/2007	Treatment of human African trypanosomiasis (sleeping sickness)	Immtech Pharmaceuticals, Inc.
	Papain, trypsin, and chymotrypsin	Wobe-Mugos	12/21/1998	Treatment of multiple myeloma.	Marlyn Nutraceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Parvovirus B19 (recombinant VP1 and VP2; S.frugiperda cells) vaccine	Medi-491	5/7/1999	Prevention of transient aplastic crisis in patients with sickle cell anemia.	MedImmune, Inc.
	Pegademase bovine	Adagen	5/29/1984	For enzyme replacement therapy for ADA deficiency in patients with severe combined immunodeficiency.	Sigma-tau Pharmaceuticals, Inc.
	Pegaspargase	Oncaspar	10/20/1989	Treatment of acute lymphocytic leukemia.	Sigma-tau Pharmaceuticals, Inc.
	Pegfilgrastim	Neulasta	11/20/2013	Treatment of subjects at risk of developing myelosuppression after a radiological or nuclear incident	Amgen, Inc.
	Peginterferon alfa-2a	Pegasys	7/13/1998	Treatment of renal cell carcinoma.	Hoffman-La Roche Inc.
	Peginterferon alfa-2a	Pegasys	9/30/1999	Treatment of chronic myelogenous leukemia.	Hoffman-La Roche Inc.
	Pegvisomant	Somavert	6/24/1997	Treatment of acromegaly.	Sensus Corporation
	Pegylated arginine deiminase	Melanocid	4/12/1999	Treatment of invasive malignant melanoma.	Polaris Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Peldesine	n/a	10/5/1993	Treatment of cutaneous T-cell lymphoma.	BioCryst Pharmaceuticals, Inc.
	Pentamidine isethionate	n/a	10/29/1984	Treatment of Pneumocystis carinii pneumonia.	Aventis Behring L.L.C.
	Pentamidine isethionate	Pentam 300	2/28/1984	Treatment of Pneumocystis carinii pneumonia.	Fujisawa USA, Inc.
	Pentamidine isethionate	Nebupent	1/12/1988	Prevention of Pneumocystis carinii pneumonia in patients at high risk of developing this disease.	Fujisawa USA, Inc.
	Pentamidine isethionate (inhalation)	Pneumopent	10/5/1987	Prevention of Pneumocystis carinii pneumonia in patients at high risk of developing this disease	Fisons Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Pentasaccharide ethyl glycoside consisting of one alpha-D-sialylosyl residue as a sodium salt, two beta-D-galactopyranosyl residues, one 2-acetamido-beta-D-glucopyranosyl unit, and one alpha-L-fucopyranosyl unit	Cylexin	7/18/1997	Treatment of neonates and infants undergoing cardiopulmonary bypass during surgical repair of congenital heart lesions.	Cytel Corporation
	Pentastarch	Pentaspan	8/28/1985	As an adjunct in leukapheresis to improve the harvesting and increase the yield of leukocytes by centrifugal means.	DuPont Pharmaceuticals
	Pentosan polysulfate sodium	Elmiron	8/7/1985	Treatment of interstitial cystitis.	Alza Corporation
	Pentostatin	Nipent	11/24/1999	Treatment of peripheral T-cell lymphomas.	SuperGen, Inc.
	Pentostatin	Nipent	3/27/1998	Treatment of cutaneous T-cell lymphoma.	SuperGen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Pentostatin	Nipent	1/29/1991	Treatment of patients with chronic lymphocytic leukemia.	SuperGen, Inc.
	Pentostatin for injection	Nipent	9/10/1987	Treatment of hairy cell leukemia.	SuperGen, Inc.
	Peptide 144 TGF beta-1 inhibitor	n/a	4/27/2006	Treatment of systemic sclerosis	Digna Biotech, S.L.
	Peptide 144 TGF beta-1-inhibitor	n/a	4/26/2006	Treatment of localized scleroderma	Digna Biotech, S.L.
	Peptide that inhibits mechanosensitive ion channel (MSC) activity	n/a	9/15/2010	Treatment of Duchenne Muscular Dystrophy (DMD.)	Rose Pharmaceuticals
	Peptidomimetic analog of hexarelin	n/a	5/14/2007	Diagnosis of growth hormone deficiency	Aeterna Zentaris GmbH
	Perflubron	Liquivent(Tm)	4/26/2001	Treatment of acute respiratory distress disease (ARDS) in adults	PFC Development/New Alliance Pharmaceutical
	Phenylacetate	n/a	3/6/1998	For use as an adjunct to surgery, radiation therapy and chemotherapy for the treatment of patients with primary or recurrent malignant glioma.	Elan Drug Delivery, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Phenylalanine ammonia-lyase	Phenylase. Ravpal-Peg	3/8/1995	Treatment of hyperphenylal aninemia	BioMarin Pharmaceutical
	Phenylbutyrate	n/a	1/19/2000	Treatment of acute promyelocytic leukemia.	Elan Drug Delivery, Inc.
	Phosphatidylinositol 3-Kinase "PI3K" Inhibitor	n/a	11/2/2010	Treatment of chronic lymphocytic leukemia (CLL)	Semafore Pharmaceuticals
	Picoplatin	n/a	11/2/2005	Treatment of small cell lung cancer	Poniard Pharmaceuticals
	Pilocarpine	Salagen	9/24/1990	Treatment of xerostomia induced by radiation therapy for head and neck cancer.	MGI Pharma, Inc.
	Pilocarpine HCl	Salagen	2/28/1992	Treatment of xerostomia and keratoconjunctivitis sicca in Sjogren's syndrome patients.	MGI Pharma, Inc.
	Plasmid DNA vector expressing cystic fibrosis transmembrane gene	n/a	3/29/2005	Treatment of cystic fibrosis	Copernicus Therapeutics, Inc.
	Plitidepsin	Aplidin	9/30/2004	Treatment of multiple myeloma	PharmaMar USA, Inc.
	Poloxamer 188	Florcor	2/22/1990	Treatment of severe burns requiring hospitalization.	CytRx Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Poloxamer 188	n/a	8/5/1997	Treatment of vasospasm in subarachnoid hemorrhage patients following surgical repair of a ruptured cerebral aneurysm.	CytRx Corporation
	Poloxamer 331	Protox	3/21/1991	Initial therapy of toxoplasmosis in patients with AIDS.	CytRx Corporation
	Poly I: poly C12U	Ampligen	12/9/1993	Treatment of invasive metastatic melanoma (stage IIb, III, IV).	Hemispherx Biopharma, Inc.
	Poly I: poly C12U	Ampligen	12/9/1993	Treatment of chronic fatigue syndrome.	Hemispherx Biopharma, Inc.
	Poly I: poly C12U	Ampligen	5/20/1991	Treatment of renal cell carcinoma.	Hemispherx Biopharma, Inc.
	Poly-ICLC	Hiltonol	3/17/1997	Treatment of primary brain tumors.	Oncovir
	Polyethylene glycol-modified uricase	Zurase	12/21/1998	Treatment of tumor lysis syndrome in cancer patients undergoing chemotherapy.	EnzymeRx, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Polyethylene glycol-modified uricase	n/a	6/6/2005	Treatment of hyperuricemia in patients with gout refractory to conventional therapy or in whom conventional therapy is contraindicated	EnzymeRx, LLC
	Polyethylene glycol-modified uricase	Zurase	9/14/1999	Prophylaxis of hyperuricemia in cancer patients prone to develop tumor lysis syndrome during chemotherapy.	EnzymeRx, LLC
	Polymeric oxygen	n/a	3/25/1992	Treatment of sickle cell anemia.	Capmed USA
	Polyribonucleotide; Polyribonucleosinic/-cyclidylic/-uridylic acid	Ampligen	7/19/1988	Treatment of AIDS.	Hemispherx Biopharma, Inc.
	Polyvalent, shed-antigen melanoma vaccine	n/a	6/9/2006	Treatment of stage IIb to stage IV melanoma	Polynoma LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Porcine Sertoli cells aseptically prepared for intracerebral co-implantation with fetal neural tissue	N-Graft	6/24/1997	Treatment of Hoehn and Yahr stage four and five Parkinson's disease.	Titan Pharmaceuticals, Inc.
	Porcine fetal neural dopaminergic cells and/or precursors aseptically prepared and coated with anti-MHC-1 Ab for intracerebral implantation	Neurocell-Pd	12/17/1996	Treatment of Hoehn and Yahr stage 4 and 5 Parkinson's disease.	Diacrin/Genzyme LLC
	Porcine fetal neural dopaminergic cells and/or precursors aseptically prepared for intracerebral implantation.	Neurocell-Pd	12/17/1996	Treatment of Hoehn and Yahr stage 4 and 5 Parkinson's disease.	Diacrin/Genzyme LLC
	Porcine fetal neural gabaergic cells and/or precursors aseptically prepared and coated with anti-MHC-1 Ab for intracerebral implantation	Neurocell-Hd	12/10/1996	Treatment of Huntington's disease.	Diacrin/Genzyme LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Porcine fetal neural gabaergic cells and/or precursors aseptically prepared for intracerebral implantation for Huntington's disease.	Neurocell-Hd	12/10/1996	Treatment of Huntington's disease.	Diacrin/Genzyme LLC
	Porfimer sodium	Photofrin	6/6/1989	For the photodynamic therapy of patients with primary or recurrent obstructing (either partially or completely) esophageal carcinoma.	QLT Phototherapeutics, Inc.
	Porfimer sodium	Photofrin	11/18/2004	Treatment of cholangiocarcinoma	Concordia Laboratories, Inc.
	Porfimer sodium	Photofrin	11/15/1989	For the photodynamic therapy of patients with transitional cell carcinoma in situ of the urinary bladder.	QLT Phototherapeutics, Inc.
	Porfiromycin	Promycin	9/19/1995	Treatment of head and neck cancer.	Boehringer Ingelheim Pharmaceuticals, Inc.
	Porfiromycin	Promycin	3/13/1997	Treatment of cervical cancer.	Boehringer Ingelheim Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Posaconazole	Posoril	7/16/2004	Treatment of zygomycosis	Schering-Plough Corporation
	Potassium Iodide Oral Solution	Thyroshield	11/17/2004	For use as a thyroid blocking agent in pediatric patients exposed to radioactive iodine	Fleming & Company, Pharmaceuticals
	Potassium citrate	Urocit-K	11/1/1984	Prevention of uric acid nephrolithiasis.	University of Texas Health Science Center at Dallas
	Potassium citrate	Urocit-K	11/1/1984	1. Prevention of calcium renal stones in patients with hypocitraturia 2. For avoidance of the complication of calcium stone formation in patients with uric lithiasis.	University of Texas Health Science Center at Dallas
	Primaquine phosphate	n/a	7/23/1993	For use in combination with clindamycin hydrochloride in the treatment of Pneumocystis carinii pneumonia associated with AIDS.	Sanofi Winthrop, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Procarbazine HCl	Matulane	8/8/2006	Treatment of malignant glioma	Sigma-Tau Pharmaceuticals, Inc.
	Prostaglandin E1 enol ester (AS-013)	Circulase	6/12/1998	Treatment of Fontaine Stage IV chronic critical limb ischemia.	LTT Baio-Pharma Co., Ltd
	Protaxel	n/a	5/21/2003	Treatment of ovarian cancer	Biophysica, Inc.
	Protein C concentrate	Ceprotrin	6/23/1992	For replacement therapy in congenital protein C deficiency for the prevention and treatment of thrombosis, pulmonary emboli, and purpura fulminans.	Baxalta US, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Protein C concentrate	Protein C Concentrate (Human) Vapor Heated, Immuno	6/19/1992	For replacement therapy in patients with congenital or acquired protein C deficiency for the prevention and treatment of warfarin-induced skin necrosis during oral anticoagulation.	Immuno Clinical Research Corp.
	Pulmonary surfactant replacement, porcine	Curosurf	8/2/1993	For the treatment and prevention of respiratory distress syndrome in premature infants.	Chiesi USA, Inc.
	Purified extract of Pseudomonas aeruginosa	Immudyn	9/22/1997	Treatment of immune thrombocytopenia purpura where it is required to increase platelet counts.	Able Laboratories, Inc.
	Purified type II collagen	Colloral	2/9/1995	Treatment of juvenile rheumatoid arthritis.	AutoImmune, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	PyNTTTTGT class of oligodeoxynucleotide with a 24-base single chain composed of nucleotide sequence 5'TCATCATTTTGT CATTTTGT CATT 3'	lmt504	11/24/2014	Treatment of rabies virus infections	Mid-Atlantic BioTherapeutics, Inc.
	Pyruvate	n/a	2/21/2001	Treatment of interstitial lung disease.	Cellular Sciences, Inc
	R-(-)-gossypol	n/a	10/24/2006	Treatment of chronic lymphocytic leukemia.	Ascenta Therapeutics, Inc.
	R-1-[2,3-dihydro-2-oxo-1-pivaloylmethyl-5-(2-pyridyl)-1 H - 1,4-benzodiazepine - 3-yl]-3-(3-methylaminophenyl)urea	n/a	9/10/2009	Treatment of gastric carcinoids	Trio Medicines Ltd.
	R-4-amino-3-(4-chlorophenyl)butanoic acid	n/a	11/28/2008	Treatment of the behavioral abnormalities associated with fragile X syndrome	Seaside Therapeutics, Inc.
	REMUNE HIV 1	n/a	2/14/2014	Treatment of pediatric HIV/AIDS (age through 16 years)	Immune Response BioPharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	RII retinamide	n/a	5/6/1993	Treatment of myelodysplastic syndromes.	Sparta Pharmaceuticals, Inc.
	Rabbit anti-human thymocyte globulin (rATG)	Thymoglobulin	9/26/2006	Induction treatment to prevent rejection and to minimize maintenance immunosuppression in pediatric liver transplant recipients	Children's Hospital of Pittsburgh
	Rapamycin	n/a	3/20/2007	Treatment of tuberous sclerosis complex	OncImmune, Inc.
	Re188 P2045 somatostatin analog	n/a	2/6/2014	Treatment of small cell lung cancer	Andarix Pharmaceuticals
	Re188 P2045 somatostatin peptide analogue	n/a	6/19/2014	Treatment of pancreatic cancer.	Andarix Pharmaceuticals, Inc.
	Recombinant AAV9 expressing human alpha-N-acetylglucosaminidase	n/a	4/30/2014	Mucopolysaccharidosis III-B (Sanfilippo Syndrome Type B)	Abeona Therapeutics LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant Epstein-Barr virus gp350 glycoprotein vaccine	n/a	8/18/2005	Prevention of post-transplantation lymphoproliferative disorders in pediatric recipients of solid-organ transplantation	Henogen S.A.
	Recombinant Fusion Protein	n/a	7/24/2012	Treatment of Myelodysplastic Syndrome	Apogenix GmbH
	Recombinant Human Alpha-Fetoprotein (rhAFP)	n/a	2/22/2001	Treatment of myasthenia gravis	Alpha Cancer Technologies, Inc.
	Recombinant Human Factor VIIa Variant	n/a	11/30/2012	Routine prophylaxis to prevent bleeding episodes in patients with hemophilia A and B patients with inhibitors	Pfizer, Inc.
	Recombinant Human Interleukin-21 (rIL-21)	n/a	10/4/2005	Treatment of stage II (T4), III or IV malignant melanoma.	Zymo Genetics, Inc
	Recombinant Human soluble Fc-gamma Receptor IIb	n/a	3/22/2010	Treatment of idiopathic thrombocytopenic purpura	SuppreMol GmbH

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant P-Selectin glycoprotein ligand	n/a	3/29/2006	Prevention of delayed graft function in renal transplant patients	Y's Therapeutics, Inc.
	Recombinant P-selectin glycoprotein ligand-immunoglobulin Ig	n/a	7/26/2007	Prevention of ischemia reperfusion injury in all solid organ transplants	Y's Therapeutics, Inc.
	Recombinant T-cell receptor ligand	n/a	5/2/2003	Treatment of multiple sclerosis patients who are both HLA-DR2 positive and autoreactive to myelin oligodendrocyte glycoprotein residues 35-55	Artielle ImmunoTherapeutics, Inc.
	Recombinant adeno-associated virus (serotype 2) (rAAV2) gene transfer agent expressing RPE65	n/a	5/11/2009	Treatment of Leber's congenital amaurosis	AmpliPhi Biosciences Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant adeno-associated virus retinal pigment epithelium gene vector AAV2-hRPE65v2	n/a	6/24/2008	Treatment of Leber congenital amaurosis due to RPE65 mutations.	Spark Therapeutics, Inc.
	Recombinant antibody construct against human CD30 and CD16A	n/a	8/20/2009	Treatment of Hodgkin lymphoma	Affimed Therapeutics AG
	Recombinant coagulation factor VIIa	n/a	4/26/2006	Treatment of diffuse alveolar hemorrhage	PharmaOrigin ApS
	Recombinant disintegrin and metalloprotease with thrombospondin type 1 motifs	n/a	7/29/2008	Treatment of thrombotic thrombocytopenic purpura including its congenial, acquired idiopathic, and secondary forms.	Baxter Healthcare Corporation
	Recombinant disintegrin and metalloprotease with thrombospondin type 1 motifs	n/a	7/29/2008	Prevention of thrombotic thrombocytopenic purpura including its congenial, acquired idiopathic and secondary forms.	Baxter Healthcare Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant fully human monoclonal antibody to anthrax protective antigen	Valortim	2/16/2006	Treatment of anthrax infection	PharmAthene, Inc.
	Recombinant fusion protein of Mycobacterium bovis BCG Hsp65 and HPV16 E7	n/a	3/19/2001	Treatment of recurrent respiratory papillomatosis (RRP)	StressGen Biotechnologies, Inc. is now Nventa
	Recombinant fusion protein-extracellular portion of CD95 fused to the Fc part of human IgG1	n/a	10/13/2009	Treatment of glioblastoma multiforme	Apogenix GmbH
	Recombinant human C1 inhibitor	n/a	6/9/2006	Treatment of capillary leakage syndrome	Pharming Technologies B.V.
	Recombinant human C1 inhibitor	n/a	6/9/2006	Prevention and/or treatment of delayed graft function after solid organ transplantation	Pharming Group N.V.
	Recombinant human C1-esterase inhibitor	n/a	2/23/1999	Prophylactic treatment of angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency.	Pharming Group N.V.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant human CD4 immunoglobulin G	n/a	8/30/1990	Treatment of AIDS resulting from infection with HIV-1.	Genentech, Inc.
	Recombinant human Clara Cell 10kDa protein	n/a	7/13/1998	Prevention of neonatal bronchopulmonary dysplasia in premature neonates with respiratory distress syndrome.	Therabron Therapeutics, Inc.
	Recombinant human acid alpha-glucosidase; alglucosidase alfa	1. Myozyme 2. Lumizyme	8/19/1997	Treatment of glycogen storage disease type II.	Genzyme Corporation
	Recombinant human alpha 1-antitrypsin	n/a	4/28/2005	Prevention of bronchopulmonary dysplasia	Arriva Pharmaceuticals, Inc.
	Recombinant human alpha-mannosidase	n/a	2/2/2006	Treatment of alpha-mannosidosis	Zymenex A/S
	Recombinant human antithrombin III	n/a	4/6/2000	Treatment of antithrombin III dependent heparin resistance requiring anticoagulation.	AT III LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant human endostatin protein	n/a	8/13/2001	Treatment of neuroendocrine tumors	EntreMed, Inc.
	Recombinant human fibrinogen	n/a	10/4/2007	Treatment of bleeding in patients deficient in fibrinogen	Pharming Technologies B.V.
	Recombinant human gelsolin	n/a	3/6/1995	Treatment of acute and chronic respiratory symptoms of bronchiectasis.	BioAgeis, Therapeutics, Inc.
	Recombinant human gelsolin	n/a	1/12/1994	Treatment of the respiratory symptoms of cystic fibrosis.	BioAegis Therapeutics, Inc.
	Recombinant human glutamic acid decarboxylase 65KDa isoform	n/a	3/22/2010	Treatment of Type I diabetes with residual beta cell function	Diamyd Therapeutics AB
	Recombinant human granulocyte colony stimulating factor	n/a	7/24/2006	Prevention of implantation failure and unexplained recurrent miscarriage	Nora Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant human highly phosphorylated acid alpha-glucosidase	Tbd	9/20/2000	For enzyme replacement therapy in patients with all subtypes of glycogen storage disease type II (GSDII, Pompe Disease)	Novazyme Pharmaceuticals, Inc.
	Recombinant human highly phosphorylated alpha-L-iduronidase (rhHP-IDUA)	n/a	4/11/2001	Enzyme replacement therapy in patients with all subtypes of Mucopolysaccharidosis I.	Novazyme Pharmaceuticals, Inc.
	Recombinant human insulin-like growth factor-I	Pv802	2/16/2000	Treatment of short-bowel syndrome as a result of resection of the small bowel or as a result of congenital dysfunction of the intestines.	GroPep Pty Ltd.
	Recombinant human insulin-like growth factor-I/insulin-like growth factor binding protein-3	n/a	6/15/1999	Treatment of major burns that require hospitalization.	Insmed, Inc.
	Recombinant human interleukin-12	n/a	10/20/1997	Treatment of renal cell carcinoma.	Genetics Institute, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant human keratinocyte growth factor	n/a	12/20/1999	Reducing the incidence and severity of radiation-induced xerostomia.	Swedish Orphan Biovitrum AB (publ) (SOBI)
	Recombinant human luteinizing hormone	Luveris	10/7/1994	For use in association with recombinant human follicle stimulating hormone for the treatment of women with chronic anovulation due to hypogonadotropic hypogonadism.	EMD Serono, Inc.
	Recombinant human lysosomal acid lipase or cholesteryl ester hydrolase	Cholestrase	7/14/2005	Treatment of lipase deficiencies, including Wolman Disease and cholesteryl ester storage disease	Lysosomal Acid Lipase, LLC
	Recombinant human microplasmin	n/a	1/23/2006	Treatment of peripheral arterial occlusion	ThromboGenics Ltd
	Recombinant human nerve growth factor	n/a	4/16/1999	Treatment of HIV-associated sensory neuropathy.	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant human platelet derived growth factor BB	n/a	2/1/2007	Treatment of osteonecrosis of the jaws	Luitpold Pharmaceuticals, Inc.
	Recombinant human thrombopoietin	n/a	9/29/1997	For use in accelerating platelet recovery in patients undergoing hematopoietic stem cell transplantation.	Genentech, Inc.
	Recombinant humanized MAb 5c8	n/a	3/22/1999	Prevention of rejection of pancreatic islet cell transplants.	Biogen, Inc.
	Recombinant humanized MAb 5c8	n/a	10/14/1998	Prevention and treatment of Factor VIII/Factor IX inhibitors in patients with hemophilia A or B.	Biogen, Inc.
	Recombinant humanized MAb 5c8	n/a	3/22/1999	Prevention of rejection of solid organ transplants.	Biogen, Inc.
	Recombinant humanized anti-LOXL2 monoclonal antibody	n/a	4/18/2011	Treatment of idiopathic pulmonary fibrosis	Gilead Sciences, Inc.
	Recombinant humanized monoclonal antibody 5c8	n/a	2/3/1998	Treatment of immune thrombocytopenic purpura.	Biogen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant humanized monoclonal antibody 5c8	n/a	2/18/1998	Treatment of systemic lupus erythematosus .	Biogen, Inc.
	Recombinant immunotoxin combining the functional components of antibody against mesothelin and the enzymatic activity of a pseudomonas exotoxin	n/a	2/11/2002	Treatment of malignant mesothelioma	National Institutes of Health
	Recombinant immunotoxin combining the functional components of antibody against mesothelin and the enzymatic activity of a pseudomonas exotoxin	n/a	2/11/2002	Treatment of epithelial ovarian cancer	National Institutes of Health
	Recombinant methionyl brain-derived neurotrophic factor	n/a	11/28/1994	Treatment of amyotrophic lateral sclerosis.	Amgen, Inc.
	Recombinant replication deficient adenovirus vector carrying human p53 gene	n/a	4/12/1999	Treatment of primary ovarian cancer.	Schering Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Recombinant retroviral vector - glucocerebrosidase	n/a	11/15/1993	For use as enzyme replacement therapy for patients with types I, II, or III Gaucher disease.	Genetic Therapy, Inc.
	Recombinant secretory leucocyte protease inhibitor	n/a	3/29/1991	Treatment of cystic fibrosis.	Amgen, Inc.
	Recombinant soluble human CD4 (rCD4)	n/a	3/23/1989	Treatment of AIDS in patients infected with HIV virus.	Genentech, Inc.
	Recombinant truncated SPINT2 protease inhibitor	n/a	6/24/2005	Treatment of cystic fibrosis	Aerovance, Inc.
	Recombinant urate oxidase	n/a	10/11/2000	Prophylaxis of chemotherapy-induced hyperuricemia.	Sanofi-Synthelabo Research
	Reduced L-glutathione	Cachexon	2/14/1994	Treatment of AIDS-associated cachexia.	Telluride Pharmaceutical Corporation
	Replication defective recombinant adenovirus serotype 5 vector carrying the p53 gene in its E1 deleted region	Advexin	1/27/2003	Treatment of head and neck cancer	Introgen Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Replication-incompetent GM-CSF-expressing gene-modified allogeneic acute myeloid leukemia cancer cell lines	Gvax Acute Myeloid Leukemia	3/2/2010	Treatment of acute myeloid leukemia	Aduro BioTech, Inc.
	Replication-incompetent GM-CSF-expressing gene-modified allogeneic chronic myeloid leukemia cancer cell lines	Gvax Chronic Myeloid Leukemia	4/30/2010	Treatment of chronic myeloid leukemia	Aduro BioTech, Inc.
	Replication-incompetent GM-CSF-expressing gene-modified allogeneic pancreatic cancer cell lines	Gvax Pancreas	3/1/2010	Treatment of pancreatic cancer.	Aduro BioTech, Inc.
	Respiratory Syncytial Virus Immune Globulin (human)	Hyperimmune Rsv	9/27/1990	Treatment of respiratory syncytial virus lower respiratory tract infections in hospitalized infants and young children.	MedImmune, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Respiratory syncytial virus immune globulin (Human)	Respigam	9/27/1990	Prophylaxis of respiratory syncytial virus lower respiratory tract infections in infants and young children at high risk of RSV disease.	MedImmune & Massachusetts Public Health Biologics Labs.
	Reversal agent linked to chloroquine-like moiety	n/a	3/26/2010	Treatment of malaria	DesignMedix
	Revimmune	n/a	2/18/2011	Treatment of autoimmune hemolytic anemia.	Accentia Biopharmaceuticals
	Reviparin sodium	Clivarine	6/18/2001	Treatment of deep vein thrombosis which may lead to pulmonary embolism in pediatric patients	Abbott
	Reviparin sodium	Clivarine	6/18/2001	Long-term treatment of acute deep vein thrombosis with or without pulmonary embolism in pregnant patients	Abbott

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Rho (D) immune globulin intravenous (human)	Winrho Sd	11/9/1993	Treatment of immune thrombocytopenic purpura.	Rh Pharmaceuticals, Inc.
	Rifabutin	Mycobutin	12/18/1989	Prevention of disseminated Mycobacterium avium complex disease in patients with advanced HIV infection.	Adria Laboratories, Inc.
	Rifabutin	n/a	12/18/1989	Treatment of disseminated Mycobacterium avium complex disease.	Pfizer Inc.
	Rifalazil	n/a	4/13/1999	Treatment of pulmonary tuberculosis.	PathoGenesis Corporation
	Rifampin	Rifadin I.V.	12/9/1985	For antituberculosis treatment where use of the oral form of the drug is not feasible.	Hoechst Marion Roussel
	Rifampin, isoniazid, pyrazinamide	Rifater	9/12/1985	For the short-course treatment of tuberculosis.	Hoechst Marion Roussel
	Rifapentine	Priftin	6/9/1995	Treatment of pulmonary tuberculosis.	Hoechst Marion Roussel

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Rifapentine	Priftin	6/9/1995	Treatment of Mycobacterium avium complex in patients with AIDS.	Hoechst Marion Roussel , Inc.
	Rifapentine	Priftin	3/12/1996	Prophylactic treatment of Mycobacterium avium complex in patients with AIDS and a CD4+ count less than or equal to 75/mm3.	Hoechst Marion Roussel, Inc.
	Rilonacept	Arcalyst	12/20/2004	Treatment of CIAS1-Associated Periodic Syndromes	Regeneron Pharmaceuticals, Inc.
	Riluzole	Rilutek	10/15/1996	Treatment of Huntington's disease.	Rhone-Poulenc Rorer Pharmaceuticals, Inc.
	Riluzole	Rilutek	3/16/1993	Treatment of amyotrophic lateral sclerosis.	Rhone-Poulenc Rorer Pharmaceuticals, Inc.
	Rose Bengal Disodium	n/a	12/21/2006	Treatment of metastatic melanoma	Provectus Pharmaceuticals, Inc.
	Roseburia hominis	Rosburix	8/4/2014	Treatment of ulcerative colitis in pediatric patients age 0 through 16 years	4D Pharma Research Ltd
	S(-)-3-[3-amino-phthalimido]-glutamamide	n/a	3/14/2002	Treatment of multiple myeloma	EntreMed Incorporated

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	S-59 treated FFP (plasma treated with amotosalen hydrochloride & ultraviolet A light	Intercept Blood System For	2/14/2011	Treatment of thrombotic thrombocytopenic purpura	Cerus Corporation
	S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDENISFKEK	n/a	10/20/2009	Treatment of pancreatic cancer	MBiotec GmbH
	S-adenosylmethionine	n/a	4/30/1998	Treatment of AIDS-myelopathy.	Genopia USA, Inc.
	S-nitrosoglutathione	n/a	5/12/2009	Management of cystic fibrosis patients to improve airway clearance and to improve or stabilize pulmonary function	N30 PHARMAceuticals, LLC
	S-nitrosoglutathione	n/a	12/28/2012	Treatment of severe preeclampsia	Salupont Consulting Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutamyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide	n/a	11/16/2015	Treatment of C3 glomerulopathy.	Amyndas Pharmaceuticals
	S3,S13-cyclo(D-tyrosyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutamyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	n/a	10/9/2014	Treatment of paroxysmal nocturnal hemoglobinuria (PNH)	Amyndas Pharmaceuticals
	SC-1 monoclonal antibody	n/a	11/12/2003	Treatment of patients with CD55 (sc-1) positive gastric tumors	Patrys Limited
	SDF-1 (108) Lysine Dimer	n/a	7/7/2005	Treatment of osteogenic sarcoma	Chemokine Therapeutics Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sacrosidase	Sucraid	12/10/1993	Treatment of congenital sucrase-isomaltase deficiency	QOL Medical, LLC
	Sar9, Met(O2)11-Substance P	Homspera	3/16/2011	Treatment of idiopathic pulmonary fibrosis	ImmuneRegen BioSciences, Inc.
	Sargramostim	Leukine	3/6/1995	To reduce neutropenia and leukopenia and decrease the incidence of death due to infection in patients with acute myelogenous leukemia.	Immunex Corporation
	Sargramostim	Leukine	5/3/1990	Treatment of neutropenia associated with bone marrow transplant, for the treatment of graft failure and delay of engraftment, and for the promotion of early engraftment.	Immunex Corporation
	Satumomab pendetide	Oncoscint Cr/Ov	9/25/1989	Detection of ovarian carcinoma.	Cytogen Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Secalciferol	Osteo-D	7/26/1993	Treatment of familial hypophosphatemic rickets.	Teva Pharmaceuticals USA
	Secretory leukocyte protease inhibitor	n/a	6/30/1992	Treatment of bronchopulmonary dysplasia.	Synergen, Inc.
	Selective inhibitor of polymorphonuclear leukocyte (PMN) elastase	n/a	6/4/1996	Therapeutic management of patients with lung disease attributable to cystic fibrosis.	DuPont Pharmaceuticals Company
	Selegiline HCl	Eldepryl	11/7/1984	As an adjuvant to levodopa and carbidopa treatment of idiopathic Parkinson's disease (paralysis agitans), postencephalitic Parkinsonism, and symptomatic Parkinsonism.	Somerset Pharmaceuticals, Inc.
	Selinexor; (Z)-3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-1,2,4-triazol-1-yl)-N-(pyrazin-2-yl)acrylohydrazide	n/a	5/14/2014	Treatment of acute myeloid leukemia	Karyopharm Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Seneca Valley virus	n/a	8/22/2008	Treatment of neuroendocrine tumors	Neotropix, Inc.
	Sermorelin acetate	Geref	9/14/1988	Treatment of idiopathic or organic growth hormone deficiency in children with growth failure.	EMD Serono, Inc.
	Serratia marcescens extract (polyribosomes)	Imuvert	9/7/1988	Treatment of primary brain malignancies.	Cell Technology, Inc.
	Short chain fatty acid enema	Colomed	8/19/1997	Treatment of chronic radiation proctitis.	Richard I. Breuer, M.D.
	Short chain fatty acid solution	Colomed	5/29/1990	Treatment of the active phase of ulcerative colitis with involvement restricted to the left side of the colon.	Richard I. Breuer
	Silibinin-C-2',3-dihydrogensuccinate, disodium salt	Legalon Sil	2/17/2012	Prevention of recurrent hepatitis C in liver transplant patients	Meda AB
	Silver sulfadiazine and cerium nitrate	Flammacerium	11/17/1999	Treatment of patients with severe dermal burns	Sinclair Pharmaceuticals Ltd
	Siponimod	n/a	11/26/2013	Treatment of polymyositis	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sitaxsentan Sodium	n/a	11/2/2004	For the treatment of pulmonary arterial hypertension in the absence of chronic obstructive pulmonary disease or congestive heart failure.	Pfizer Global Research and Development
	Sodium Monomercaptoundecahydro-closododecaborate	Borocell	4/15/1992	For use in boron neutron capture therapy (BNCT) in the treatment of glioblastoma multiforme.	Neutron Technology Corp.& Neutron R&D Partner
	Sodium Thiosulfate	n/a	2/16/2012	Treatment of uremic and non-uremic calciphylaxis	Luitpold Pharmaceuticals, Inc.
	Sodium aluminosilicate	n/a	3/4/2005	Treatment of chronic hepatic encephalopathy	Framework Therapeutics, LLC
	Sodium ascorbate and menadione sodium bisulfite	Apatone	7/31/2007	Treatment of metastatic or locally advanced inoperable transitional cell carcinoma of the urothelium (stage III and IV bladder cancer)	IC-MedTech Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sodium dichloroacetate	n/a	11/10/1994	Treatment of lactic acidosis in patients with severe malaria.	Stacpoole, Peter W. M.D., Ph.D.
	Sodium dichloroacetate	n/a	6/11/1990	Treatment of homozygous familial hypercholesterolemia.	Stacpoole, Peter W. M.D., Ph.D.
	Sodium dichloroacetate	n/a	6/11/1990	Treatment of congenital lactic acidosis	Stacpoole, Peter W. M.D., Ph.D.
	Sodium nitrite	n/a	1/17/2007	Prevention of vasospasm associated with subarachnoid hemorrhage	Hope Pharmaceuticals
	Sodium nitrite	n/a	4/18/2007	Treatment of cyanide poisoning	Hope Pharmaceuticals
	Sodium nitrite	n/a	4/2/2007	Treatment of vaso-occlusive crisis associated with sickle cell disease	Hope Pharmaceuticals
	Sodium nitrite/sodium thiosulfate	Cyanide Antidote Package	3/23/2007	Treatment of cyanide poisoning	Keystone Pharmaceuticals, Inc.
	Sodium phenylacetate/sodium benzoate 10%/10% Injection	Ammonul(R)	6/3/2005	Treatment of grade III and IV hepatic encephalopathy	Ucyclyd Pharma, Inc.
	Sodium phenylbutyrate	n/a	3/20/2007	Treatment of spinal muscular atrophy	Tikvah Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sodium phenylbutyrate	Buphenyl	1/25/2007	Treatment of spinal muscular atrophy	OrphaMed, Inc.
	Sodium phenylbutyrate	n/a	4/24/1998	For use as an adjunct to surgery, radiation therapy and chemotherapy for the treatment of patients with primary or recurrent malignant glioma.	Elan Drug Delivery, Inc.
	Sodium pyruvate	n/a	3/31/2003	Treatment of cystic fibrosis	Cellular Sciences, Inc
	Sodium stibogluconate	n/a	6/16/2006	Treatment of cutaneous leishmaniasis	VioQuest Pharmaceuticals, Inc.
	Sodium tetradecyl sulfate	Sotradecol	6/10/1986	Treatment of bleeding esophageal varices.	Elkins-Sinn, Inc.
	Sodium thiosulfate	n/a	3/17/2004	Prevention of platinum-induced ototoxicity in pediatric patients	Fennec Pharmaceuticals, Inc.
	Sodium thiosulfate and sodium nitrite	n/a	12/18/2006	Treatment of cyanide poisoning	Akorn, Inc.
	Sodium thiosulfate, sodium nitrite, amyl nitrite	Cyanide Antidote Package	6/16/2006	Treatment of cyanide poisoning	Keystone Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Solasonine and solamargine	Coramsine	2/6/2006	Treatment of high risk stage II, stage III and stage IV melanoma	Solbec Pharmaceuticals Limited
	Solasonine and solamargine	Coramsine	11/2/2005	Treatment of renal cell carcinoma	Solbec Pharmaceuticals Limited
	Soluble complement receptor type 1	n/a	3/6/2000	Prevention of post-cardiopulmonary bypass syndrome in children undergoing cardiopulmonary bypass.	Avant Immunotherapeutics, Inc.
	Soluble recombinant human complement receptor type 1	n/a	11/21/1994	Prevention or reduction of adult respiratory distress syndrome.	T Cell Sciences, Inc.
	Somatostatin	n/a	12/22/1994	Treatment of bleeding esophageal varices.	Eumedita Pharmaceuticals A.G. (Schweiz)
	Somatostatin	Zecnil	6/20/1988	Adjunct to the non-operative management of secreting cutaneous fistulas of the stomach, duodenum, small intestine (jejunum and ileum), or pancreas.	Ferring Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Somatrem for injection	Protropin	12/9/1985	1. Long-term treatment of children who have growth failure due to a lack of adequate endogenous growth hormone secretion (prevalence 15,000) 2. Treatment of short stature associated with Turner's syndrome (prevalence 8000).	Genentech, Inc.
	Somatropin	Serostim	3/16/2004	Treatment of patients with HIV-associated adipose redistribution syndrome	EMD Serono, Inc.
	Somatropin	Nutropin	3/6/1987	For use in the long-term treatment of children who have growth failure due to a lack of adequate endogenous growth hormone secretion.	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Somatropin	Humatrope	5/8/1990	Treatment of short stature associated with Turner syndrome.	Eli Lilly and Company
	Somatropin	Genotropin	9/6/1994	Treatment of adults with growth hormone deficiency.	Pharmacia & Upjohn
	Somatropin	Norditropin	8/9/2006	Treatment of short stature in patients with Noonan syndrome	Novo Nordisk Inc.
	Somatropin	Humatrope	12/15/2005	Treatment of short stature in pediatric patients with short stature homeobox-containing gene (SHOX) deficiency	Eli Lilly and Company
	Somatropin (rDNA)	Saizen	5/3/1989	For the enhancement of nitrogen retention in hospitalized patients suffering from severe burns.	EMD Serono, Inc.
	Somatropin (r-DNA)	Zorbtive	3/6/1995	For use alone or in combination with glutamine in the treatment of short bowel syndrome.	EMD Serono, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Somatropin (r-DNA) for injection	Serostim	3/26/1996	Treatment of children with AIDS-associated failure-to-thrive including AIDS-associated wasting.	EMD Serono, Inc.
	Somatropin (rDNA origin)	Saizen	3/6/1987	Treatment of idiopathic or organic growth hormone deficiency in children with growth failure.	EMD Serono, Inc.
	Somatropin (rDNA origin)	Nutropin Depot	10/28/1999	Long-term treatment of children who have growth failure due to a lack of adequate endogenous growth hormone secretion.	Genentech, Inc.
	Somatropin (rDNA origin) injection	Norditropin	7/10/1987	1. Treatment of growth failure in children due to inadequate growth hormone secretion 2. Treatment of short stature associated with Turner's syndrome	Novo Nordisk Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Somatropin [rDNA]	Genotropin	7/6/1999	Treatment of short stature in patients with Prader-Willi syndrome.	Pharmacia & Upjohn
	Somatropin for injection	Serostim	11/15/1991	Treatment of AIDS-associated catabolism/weight loss.	EMD Serono, Inc.
	Somatropin for injection	Nutropin	8/4/1989	Treatment of growth retardation associated with chronic renal failure.	Genentech, Inc.
	Somatropin for injection	Nutropin	11/18/1996	As replacement therapy for growth hormone deficiency in adults after epiphyseal closure.	Genentech, Inc.
	Somatropin for injection	Nutropin	3/23/1989	Treatment of short stature associated with Turner's syndrome.	Genentech, Inc.
	Somatropin for injection	Humatrope	6/12/1986	For the long-term treatment of children who have growth failure due to inadequate secretion of normal endogenous growth hormone.	Eli Lilly and Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sorafenib	Nexavar	10/8/2004	Treatment of renal cell carcinoma.	Bayer Pharmaceutical Corporation
	Sorafenib	Nexavar	4/20/2006	Treatment of hepatocellular carcinoma	Bayer Pharmaceuticals Corporation
	Sotalol HCl	Betapace	9/23/1988	1. Treatment of life-threatening ventricular tachyarrhythmias 2. Prevention of life treating ventricular tachyarrhythmias	Berlex Laboratories, Inc.
	Staphylococcus aureus Immune Globulin (Human)	Altastaph	1/29/2004	Prophylaxis against Staphylococcus aureus infections in low birth weight neonates	Biotest Pharmaceuticals Corporation
	Sterile Bicarbonate Infusate, Hemofiltration, Hemodiafiltration, and Hemodialysis Solution	Accusol	5/29/2009	For use as a replacement solution in adults and children during Continous Renal Replacement Therapy	Baxter Healthcare Corporation
	Sterile talc	Steritalc	12/8/1997	Treatment of pneumothorax	Novatech SA
	Sterile talc	Steritalc	12/8/1997	Treatment of malignant pleural effusion.	Novatech SA

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sterile talc powder	Sclerosol Intrapleural Aerosol	9/18/1995	Treatment of malignant pleural effusion.	Bryan Corporation
	Succimer	Chemet	11/5/1990	Prevention of cystine kidney stone formation in patients with homozygous cystinuria who are prone to stone development.	Sanofi Winthrop, Inc.
	Succimer	Chemet Capsules	5/9/1984	Treatment of lead poisoning in children.	Bock Pharmacal Company
	Succimer	Chemet	3/22/1991	Treatment of mercury intoxication.	Sanofi Winthrop, Inc.
	Sucralfate suspension	n/a	3/4/1991	Treatment of oral ulcerations and dysphagia in patients with epidermolysis bullosa.	Darby Pharmaceuticals, Inc.
	Sucralfate suspension	n/a	3/12/1990	Treatment of oral complications of chemotherapy in bone marrow transplant patients.	Darby Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Sulfadiazine	n/a	3/14/1994	For use in combination with pyrimethamine for the treatment of Toxoplasma gondii encephalitis in patients with and without AIDS.	Eon Labs Manufacturing, Inc.
	Sulfapyridine	n/a	9/10/1990	Treatment of dermatitis herpetiformis.	Jacobus Pharmaceutical Company
	Superoxide dismutase (human)	n/a	3/6/1985	For protection of donor organ tissue from damage or injury mediated by oxygen-derived free radicals that are generated during the necessary periods of ischemia (hypoxia, anoxia), and especially reperfusion, associated with the operative procedure.	Pharmacia-Chiron Partnership

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Suramin	Metaret	5/6/1997	Treatment of hormone-refractory prostate cancer.	Warner-Lambert Company
	Synsorb Pk	n/a	7/17/1995	Treatment of verocytotoxogenic E. coli infections.	Synsorb Biotech Inc.
	Synthetic 47 amino acid-long N-terminally myristoylated, HBV-L-protein derived lipopeptide	n/a	3/31/2015	Treatment of hepatitis D virus infection.	MYR GmbH
	Synthetic derivative of 16-hydroxy-9Z, 12Z, 14E-octadecatrienoic acid	Drepanol	10/24/1991	Prophylactic treatment of sickle cell disease.	Omex International, Inc.
	Synthetic double-stranded siRNA oligonucleotide against caspase 2 mRNA	n/a	9/25/2012	Treatment of ischemic optic neuropathy	Quark Pharmaceuticals, Inc.
	Synthetic human secretin	n/a	9/18/2006	For use in conjunction with diagnostic procedures (excluding ERCP) for pancreatic disorders to increase pancreatic fluid secretion	Innovate Biopharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Synthetic human secretin	n/a	6/16/1999	For use in obtaining desquamated pancreatic cells for cytopathologic examination in pancreatic carcinoma.	ChiRhoClin, Inc.
	Synthetic human secretin	Chirostim	6/16/1999	For use in the diagnosis of gastrinoma associated with Zollinger-Ellison syndrome.	ChiRhoClin, Inc.
	Synthetic human secretin	n/a	6/16/1999	For use in the evaluation of exocrine pancreas function.	ChiRhoClin, Inc.
	Synthetic human secretin	n/a	3/7/2000	For use in conjunction with diagnostic, therapeutic, or combined diagnostic/therapeutic procedures for pancreatic disorders to increase pancreatic fluid secretion.	ChiRhoClin, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Synthetic porcine secretin	Secreflo	6/18/1999	For use in the diagnosis of gastrinoma associated with Zollinger-Ellison syndrome.	ChiRhoClin, Inc.
	Synthetic porcine secretin	Secreflo	3/7/2000	For use in conjunction with diagnostic, therapeutic, or combined diagnostic/therapeutic procedures for pancreatic disorders to increase pancreatic fluid secretion.	ChiRhoClin, Inc.
	Synthetic porcine secretin	Secreflo	6/18/1999	For use in the evaluation of exocrine pancreas function.	ChiRhoClin, Inc.
	Synthetic porcine secretin	n/a	6/18/1999	For use in obtaining desquamated pancreatic cells for cytopathologic examination in pancreatic carcinoma.	ChiRhoClin, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	T cell receptor (TCR) peptide (BV5S2, BV6S5, BV13S1) vaccine	Neurovax	2/6/2014	Treatment of Pediatric Multiple Sclerosis	Immune Response BioPharma, Inc.
	T-cell depleted stem cell enriched cellular product from peripheal blood stem cells	n/a	11/1/2001	Treatment of chronic granulomatous disease	Nexell Therapeutics Inc.
	T4 endonuclease V, liposome encapsulated	n/a	6/27/1989	To prevent cutaneous neoplasms and other skin abnormalities in xeroderma pigmentosum.	AGI Dermatics
	TD-K6a.513a.12	Reveker	6/15/2006	Treatment of pachyonychia congenita	TransDerm, Inc.
	TXA127	n/a	10/25/2013	Treatment of acute radiation syndrome	US Biotest, Inc.
	Tacrolimus	Prograf	4/6/1998	Prophylaxis of graft-versus-host-disease.	Fujisawa USA, Inc.
	Tacrolimus	Prograf	6/6/2005	Prophylaxis of organ rejection in patients receiving heart transplants.	Astellas Pharma US, Inc.
	Taliglucerase alfa	Elelyso For Injection	9/3/2009	Treatment of Gaucher's disease	Pfizer, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Tamibarotene	n/a	10/11/2007	Treatment of acute promyelocytic leukemia (APL).	CytRx Corporation
	Tanespimycin	n/a	9/9/2004	Treatment of multiple myeloma	Bristol-Myers Squibb
	Targeted Gastrin 17 Complexed Peptide	n/a	5/14/2014	Treatment of pancreatic cancer	Tyg Oncology Ltd
	Technetium Tc99m murine monoclonal antibody (IgG2a) to B cell	Lymphoscan	4/7/1992	Diagnostic imaging in the evaluation of the extent of disease in patients with histologically confirmed diagnosis of non-Hodgkin's B-cell lymphoma, acute B-cell lymphoblastic leukemia (in children and adults), and chronic B-cell lymphocytic leukemia.	Immunomedics, Inc.
	Technetium Tc99m rh-Annexin V	Apomate	11/3/2000	Diagnosis or assessment of rejection status in heart, heart-lung, single lung, or bilateral lung transplants.	Theseus Imaging Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Technetium Tc99m sulfur colloid injection, lyophilized	Technetium Tc99m Sulfur Colloi	3/17/2009	For localization of sentinel lymph nodes in patients with melanoma.	Pharmalucence, Inc.
	Tegafur/gimeracil /oteracil	n/a	7/20/2006	Treatment of gastric cancer	Taiho Pharma USA, Inc.
	Temoporfin	Foscan	10/28/1999	Palliative treatment of recurrent, refractory or second primary squamous cell carcinomas of the head and neck in patients considered to be incurable with surgery or radiotherapy.	Biolitec Pharma Ireland Ltd.
	Temozolomide	Temodal	10/18/2004	Treatment of newly diagnosed high grade glioma	Schering-Plough Research Institute
	Temozolomide	Temodal	10/14/1998	Treatment of advanced metastatic melanoma.	Schering-Plough Research Institute
	Temsirolimus	Torisel	12/16/2004	Treatment of renal cell carcinoma	Wyeth Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Teniposide	Vumon For Injection	11/1/1984	Treatment of refractory childhood acute lymphocytic leukemia.	Bristol-Myers Squibb Pharmaceutical Research Institute
	Teriparatide	Parathar	1/9/1987	Diagnostic agent to assist in establishing the diagnosis in patients presenting with clinical and laboratory evidence of hypocalcemia due to either hypoparathyroidism or pseudohypoparathyroidism.	Rhone-Poulenc Rorer Pharmaceuticals, Inc.
	Teriparatide	Parathar	10/28/1999	Treatment of idiopathic osteoporosis.	Biomeasure, Inc.
	Terlipressin	Glypressin	3/6/1986	Treatment of bleeding esophageal varices.	Ferring Laboratories, Inc.
	Testosterone	Theraderm Testosterone Transdermal System	9/22/1997	For use as physiologic testosterone replacement in androgen deficient HIV+ patients with an associated weight loss.	Watson Laboratories

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Testosterone propionate ointment 2%	n/a	7/31/1991	Treatment of vulvar dystrophies.	Star Pharmaceuticals, Inc.
	Tetrabenazine	Xenazine	12/11/1997	Treatment of Huntington's disease	Prestwick Pharmaceuticals, Inc
	Tetrabenazine	n/a	5/12/1998	Treatment of moderate/severe tardive dyskinesia.	Prestwick Pharmaceuticals, Inc.
	Tetraiodothyroacetic acid	n/a	5/1/2000	Suppression of thyroid stimulating hormone in patients with well-differentiated cancer of the thyroid gland.	Danforth, Jr., MD, Elliot
	Tezacitabine	n/a	1/27/2003	Treatment of adenocarcinoma of the esophagus and stomach	Sanofi-Aventis US, Inc.
	Thalidomide	n/a	1/12/1993	Treatment of the clinical manifestations of mycobacterial infection caused by Mycobacterium tuberculosis and non-tuberculous mycobacteria.	Celgene Corporation
	Thalidomide	Synovir	3/11/1996	Treatment of HIV-associated wasting syndrome.	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Thalidomide	n/a	3/5/1990	Prevention of graft versus host disease.	Andrulis Research Corporation
	Thalidomide	n/a	3/5/1990	Treatment of graft versus host disease.	Andrulis Research Corporation
	Thalidomide	n/a	11/15/1988	Treatment and maintenance of reactional lepromatous leprosy.	Pediatric Pharmaceuticals, Inc.
	Thalidomide	Thalomid	7/26/1995	Treatment of erythema nodosum leprosum.	Celgene Corporation
	Thalidomide	Thalomid	4/6/1999	Treatment of Crohn's disease.	Celgene Corporation
	Thalidomide	Thalomid	10/14/1998	Treatment of multiple myeloma	Celgene Corporation
	Thalidomide	n/a	5/15/1995	Treatment and prevention of recurrent aphthous ulcers in severely, terminally immunocompromised patients.	Andrulis Research Corporation
	Thalidomide	n/a	7/29/1998	Treatment of Kaposi's sarcoma.	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Thalidomide	n/a	5/1/1995	Treatment of severe recurrent aphthous stomatitis in severely, terminally immunocompromised patients.	Celgene Corporation
	Thalidomide	n/a	2/27/1998	Treatment of primary brain malignancies.	Celgene Corporation
	Thalidomide	Thalomid	9/27/2004	Treatment of myelodysplastic syndrome	Celgene Corporation
	Thawed donor matched banked umbilical cord blood enriched by cell sorting to produce a subpopulation expressing high levels of intracellular aldehyde dehydrogenase	n/a	10/23/2008	To improve patient outcomes by decreasing time to platelet and neutrophil engraftment in patients with inherited metabolic diseases (IMD) undergoing umbilical cord blood transplantation.	Aldagen, Inc.
	Theranost 68 Ga RGD	n/a	10/1/2014	A Diagnostic for the management of Moyamoya disease (MMD)	Advanced Imaging Projects, LLC (AIP)

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Theranost 68Ga RGD	n/a	1/26/2015	Diagnostic for the clinical management of patients with tuberculosis	Advanced Imaging Projects, LLC
	Thiotepa	Tepadina	4/2/2007	Conditioning treatment prior to hematopoietic stem cell transplantation	Adienne S.A.
	Thymalfasin	Zadaxin	3/13/2006	Treatment of stage IIb through Stage IV malignant melanoma	SciClone Pharmaceuticals, Inc.
	Thymalfasin	Zadaxin	5/3/1991	Treatment of chronic active hepatitis B.	SciClone Pharmaceuticals, Inc.
	Thymalfasin	Zadaxin	3/6/2000	Treatment of hepatocellular carcinoma.	SciClone Pharmaceuticals, Inc.
	Thymalfasin	Zadaxin	1/8/1998	Treatment of DiGeorge anomaly with immune defects.	SciClone Pharmaceuticals, Inc.
	Thymosin beta 4	n/a	5/28/2004	Treatment of epidermolysis bullosa.	RegeneRx Biopharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Thyrotropin alfa	Thyrogen	8/3/2001	Treatment of well-differentiated papillary, follicular or combined papillary/follicular carcinomas of the thyroid	Genzyme Corporation
	Thyrotropin alpha	Thyrogen	2/24/1992	As an adjunct in the diagnosis of thyroid cancer.	Genzyme Corporation
	Tiazofurin (2-Beta-D-ribofuranosyl-4-thiazolecarboxamide)	n/a	12/27/2000	Chronic myelogenous leukemia (CML)	Valeant Pharmaceuticals North America
	Tilarginine acetate	n/a	4/11/2005	Treatment of cardiogenic shock	Arginox Pharmaceuticals, Inc.
	Tinidazole	Tindamax	8/20/2003	Treatment of amebiasis	Presutti Laboratories, Inc.
	Tiopronin	Thiola	1/17/1986	Prevention of cystine nephrolithiasis in patients with homozygous cystinuria.	Pak, Charles Y.C. M.D.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Tiotropium bromide	Spiriva	1/8/2008	To improve pulmonary function in conjunction with standard therapy in the management of patients with cystic fibrosis	Boehringer Ingelheim Pharmaceuticals, Inc.
	Tipifarnib	Zarnestra	7/6/2004	Treatment of acute myeloid leukemia	Johnson & Johnson Pharmaceutical Research & Dev.
	Tiratricol	Triacana	8/13/1991	For use in combination with levo-thyroxine to suppress thyroid stimulating hormone in patients with well-differentiated thyroid cancer who are intolerant to adequate doses of levo-thyroxine alone.	Laphal Laboratoires
	Tissue Plasminogen activator	n/a	4/18/2011	Treatment of acute ischemic stroke (AIS) in children age 16 years and younger.	Catherine Amlie-Lefond, MD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Tissue repair cells obtained from autologous bone marrow expanded ex vivo with a cell production system	n/a	3/13/2006	Treatment of osteonecrosis.	Aastrom Biosciences, Inc.
	Tissue repair cells obtained from autologous bone marrow expanded ex vivo with a cell production system	n/a	1/25/2007	Treatment of dilated cardiomyopathy	Vericel Corporation.
	Tizanidine HCl	Zanaflex	1/31/1994	Treatment of spasticity associated with multiple sclerosis and spinal cord injury.	Athena Neurosciences, Inc.
	Tobramycin	Tobi	6/18/1999	Treatment of bronchiectasis patients infected with Pseudomonas aeruginosa.	Novartis Pharmaceuticals Corp.
	Tobramycin for inhalation	Tobi	10/13/1994	Treatment of bronchopulmonary infections of Pseudomonas aeruginosa in cystic fibrosis patients.	Novartis Pharmaceuticals Corp
	Tolcapone	n/a	12/24/2013	Treatment of transthyretin amyloidosis	SOM Innovation Biotech SL (SOM Biotech)

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Topiramate	Topamax	11/25/1992	Treatment of Lennox-Gastaut syndrome.	Johnson & Johnson Pharmaceutical R & D, LLC
	Toremifene	n/a	8/17/1993	Treatment of desmoid tumors.	Orion Corporation
	Toremifene	Fareston	9/19/1991	Hormonal therapy of metastatic carcinoma of the breast.	Orion Corporation
	Tosedostat	n/a	12/10/2008	Treatment of acute myeloid leukemia	CTI BioPharma Corporation
	Tositumomab and iodine I 131 tositumomab	Bexxar	5/16/1994	Treatment of non-Hodgkin's B-cell lymphoma.	GlaxoSmithKline LLC
	Tramadol hydrochloride	n/a	4/26/2005	Management of postherpetic neuralgia	TheraQuest Biosciences, LLC
	Tramadol hydrochloride	n/a	1/28/2005	Treatment of painful HIV-associated neuropathy	TheraQuest Biosciences, LLC
	Tranilast	Rizaben	12/2/2003	For the treatment of malignant glioma	Angiogen Pharmaceuticals, Pty. Ltd.
	Transforming growth factor-beta 2	n/a	12/18/1992	Treatment of full thickness macular holes.	Celtrix Pharmaceuticals, Inc.
	Transgenic human alpha 1 antitrypsin	n/a	5/19/1999	Treatment of emphysema secondary to alpha 1 antitrypsin deficiency.	PPL Therapeutics (Scotland) Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Treosulfan	Ovastat	5/16/1994	Treatment of ovarian cancer.	Medac GmbH
	Tretinoin	Atra-iv	4/11/2003	Treatment of T-cell non-Hodgkin's lymphoma	Antigenics, Inc.
	Tretinoin	Atra-iv	1/14/1993	Treatment of acute and chronic leukemia.	Antigenics, Inc.
	Tretinoin	n/a	4/15/1985	Treatment of squamous metaplasia of the ocular surface epithelia (conjunctiva and/or cornea) with mucous deficiency and keratinization.	Hannan Ophthalmic Marketing Services, Inc
	Tretinoin	Vesanoid	10/24/1990	Treatment of acute promyelocytic leukemia.	Hoffmann-La Roche, Inc.
	Trientine HCl	Syprine	12/24/1984	Treatment of patients with Wilson's disease who are intolerant, or inadequately responsive to penicillamine.	Merck Sharp & Dohme Research

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Triheptanoin	Triheptanoin-Sasol Special Oil	2/1/2008	Treatment of glycogen storage disorder II (Pompe disease)	Baylor Research Institute
	Triheptanoin	Triheptanoin-Sasol Special Oil	5/26/2006	Treatment of fatty acid disorders	Baylor Research Institute
	Trimetrexate glucuronate	Neutrexin	5/15/1986	Treatment of Pneumocystis carinii pneumonia in AIDS patients.	Medimmune Oncology, Inc.
	Trisaccharides A and B	Biosynject	4/20/1987	For use in ABO-incompatible solid organ transplantation, including kidney, heart, liver and pancreas.	Chembiomed, Ltd.
	Trisaccharides A and B	Biosynject	4/15/1988	Prevention of ABO medical hemolytic reactions arising from ABO-incompatible bone marrow transplantation.	Chembiomed, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Trisaccharides A and B	Biosynject	4/12/1987	Treatment of moderate to severe clinical forms of hemolytic disease of the newborn arising from placental transfer of antibodies against blood group substances A and B.	Chembiomed, Ltd.
	Trisodium citrate concentration	Hemocitrate	6/15/1995	For use in leukapheresis procedures.	Hemotec Medical Products, Inc.
	Trisodium zinc Diethylenetriami nepentaacetate	n/a	2/27/2004	Treatment of patients with known or suspected internal contamination with plutonium, americium, or curium to increase the rate of elimination.	CustomCare Pharmacy
	Trypan blue	Membraneblue	8/2/2006	Selectively staining epiretinal membranes during ophthalmic surgical vitrectomy procedures	Dutch Ophthalmic Research Center Int'l BV

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Tumor necrosis factor-binding protein 1	n/a	1/6/1993	Treatment of symptomatic patients with AIDS including all patients with CD4 counts less than 200 cells per mm <sup>3</sup> .	EMD Serono, Inc.
	Tumor necrosis factor-binding protein II	n/a	1/6/1993	Treatment of symptomatic patients with the AIDS including all patients with CD4 T-cell counts less than 200 cells per mm <sup>3</sup> .	EMD Serono, Inc.
	Tyloxapol	Supervent	3/8/1995	Treatment of cystic fibrosis.	Kennedy & Hoidal, M.D.'s
	Type 1 native bovine skin collagen	n/a	2/1/2008	Treatment of diffuse systemic sclerosis	arGentis Pharmaceuticals, LLC
	Ubiquinol, coenzyme Q10, ubiquinone	Ubi-Q-Nol	4/12/2004	Treatment of pediatric congestive heart failure	Gel-Tec, Division of Tishcon Corporation
	Ubiquinone	Ubi-Q-Gel	12/14/1999	Treatment of mitochondrial cytopathies.	Gel-Tec, Division of Tishcon Corp.
	Unconjugated Chimeric (human-murine) G250 IgG monoclonal antibody	n/a	3/22/2001	Treatment of renal cell carcinoma.	Wilex Biotechnology GmbH
	Urea for intravitreal injection	Neurosolve	12/14/2005	Treatment of retinitis pigmentosa	Vitreo Retinal Technologies, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Urofollitropin	Metrodin	11/25/1987	For induction of ovulation in patients with polycystic ovarian disease who have an elevated LH/FSH ratio and who have failed to respond to adequate clomiphene citrate therapy.	EMD Serono, Inc.
	Urogastrone	n/a	11/1/1984	For acceleration of corneal epithelial regeneration and healing of stromal incisions from corneal transplant surgery.	Chiron Vision
	Ursodiol	Urso 250	6/20/1991	Treatment of patients with primary biliary cirrhosis	Aptalis Pharma US, Inc.
	Vaccinia Immune Globulin (Human) Intravenous	Cnj-016	6/18/2004	Treatment of complications of vaccinia vaccination	Cangene Corporation
	Vaccinia Immune Globulin (Human) Intravenous	n/a	6/18/2004	Treatment of severe complications from the smallpox vaccine	DynPort Vaccine Company LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Valine, isoleucine and leucine	Vil	1/5/1996	Treatment of hyperphenylal aninemia	Leas Research Products
	Valproate	n/a	5/5/2008	Treatment of fragile X syndrome	Neuropharm Ltd,
	Valrubicin	Valstar	5/23/1994	Treatment of carcinoma in situ of the urinary bladder.	Anthra Pharmaceuticals, Inc.
	Vapreotide	Sanvar	4/6/2004	Treatment of symptomatic carcinoid tumors	H3 Pharma, Inc.
	Varbulin	Azixa	11/5/2009	Treatment of glioblastoma multiforme.	Myrexix, Inc.
	Varicella Zoster Immune Globulin (Human)	Varizig	11/7/2006	Passive immunization for the treatment of exposed, susceptible individuals who are at risk of complications from varicella	Cangene bioPharma, Inc.
	Varlitinib	n/a	8/5/2015	Treatment of cholangiocarci noma.	ASLAN Pharmaceuticals
	Vasoactive intestinal peptide	n/a	3/9/2001	Treatment of Acute Respiratory Distress Syndrome.	mondoBIOTECH
	Vasoactive intestinal polypeptide	n/a	6/23/1993	Treatment of acute esophageal food impaction.	Research Triangle Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Vasomera	n/a	11/19/2015	Treatment of cardiomyopathy associated with dystrophinopathies; Duchenne Muscular Dystrophy (DMD), Becker Muscular Dystrophy (BMD), and X-linked dilated cardiomyopathy (XL-dCMP).	PhaseBio Pharmaceuticals, Inc.
	Virulizin	Virulizin	2/1/2001	Treatment of pancreatic cancer.	ZOR Pharmaceuticals, LLC
	Water-miscible vitamin A palmitate	Aquasol A Parenteral	3/26/2010	Prevention of bronchopulmonary dysplasia.	Fox Pharma, Inc.
	Xenogeneic hepatocytes	Hepatassist Liver Assist System	11/27/1998	Treatment of severe liver failure.	Circe Biomedical, Inc.
	Yttrium (90Y) antiferritin polyclonal antibodies	Ferritarg P	9/18/2006	Treatment of Hodgkin's disease.	Alissa Pharma, LLC
	Yttrium(90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10	n/a	12/3/2012	Treatment of soft tissue sarcoma	OncoTherapy Science, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	Yttrium-90 radiolabeled humanized monoclonal anti-carcinoembryonic antigen IgG antibody	Cea-Cide	8/3/1999	Treatment of ovarian carcinoma.	Immunomedics, Inc.
	Zalcitabine	n/a	12/9/1986	Treatment of AIDS.	National Cancer Institute, DCT
	Zalcitabine	Hivid	6/28/1988	Treatment of AIDS.	Hoffmann-La Roche, Inc.
	Zinc acetate	Galzin	11/6/1985	Treatment of Wilson's disease.	Lemmon Company
	Zoledronate	Zometa, Zabel	8/18/2000	Treatment of tumor induced hypercalcemia.	Novartis Pharmaceuticals Corp.
	Zosuquidar trihydrochloride	n/a	12/15/2005	Treatment of acute myeloid leukemia	Kanisa Pharmaceuticals, Inc.
	[131I]-N-(2-(diethylamino)ethyl-4-(4-fluorobenzamido)-5-(iodo)-2-methoxybenzamide	n/a	9/17/2008	Treatment of metastatic melanoma, stages IIB, IIC, III and IV	Molecular Insight Pharmaceuticals, Inc. (Progenics Subsidiary)
	[5,10,15,20-tetrakis(1,3-diethylimidazolium-2-yl)porphyrinato]manganese(III)pentachloride	n/a	11/4/2003	Treatment of Amyotrophic Lateral Sclerosis	Aeolus Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	[5-(5-Chloro-1H-pyrrolo[2,3-b]pyridin-3-ylmethyl)-pyridin-2-yl]-(6-trifluoromethyl-pyridin-3-ylmethyl)-amine hydrochloride salt	n/a	2/14/2014	Treatment of pigmented villonodular synovitis/giant cell tumor of the tendon sheath	Plexxikon, Inc.
	[5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	n/a	10/20/2014	Treatment of pancreatic cancer	Synovo GmbH
	[AC3-γCD2(V)] and Flucytosine (5-FC XR)	n/a	1/12/2011	Treatment of glioblastoma multiforme	Tocagen, Inc.
	[Lu-177]-DOTA-Tyr3-Octreotate	n/a	1/12/2009	Treatment of gastro-entero-pancreatic neuroendocrine tumors	Advanced Accelerator Applications
	[α-N-(2'succinyl-paclitaxel)Thr]-Phe-Phe-Tyr-Gly-Gly-Ser-Arg-Gly-[ε-N-(2'succinyl-paclitaxel)Lys]-Arg-Asn-Asn-Phe-[ε-N-(2'succinyl-paclitaxel)Lys]-Thr-Glu-Glu-Tyr	n/a	5/14/2014	Treatment of glioblastoma multiforme	Angiochem, Inc.
	[met5]-enkephalin	Opioid Growth Factor	1/24/2013	Treatment of pancreatic cancer	TNI BioTech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	[Î±â€šN-(2â€šsuccinyl-paclitaxel)Thr]-Phe-Phe-Tyr-Gly-Gly-Ser-Arg-Gly-[Î¼â€šN-(2â€šsuccinyl-paclitaxel)Lys]-Arg-Asn-Asn-Phe-[Î¼â€šN-(2â€šsuccinyl-paclitaxel)Lys]-Thr-Glu-Glu-Tyr	n/a	4/15/2015	Treatment of breast cancer patients with brain metastases	Angiochem, Inc.
	a live attenuated bioengineered Listeria monocytogenes cancer immunotherapy	n/a	4/29/2014	Treatment of Stage II to IV invasive cervical carcinoma	Advaxis, Inc.
	a-Galactosidase A	Plant-Produced Human A-Galactosidase A	1/21/2003	Treatment of Fabry's disease	iBio, Inc.
	aadeno-associated viral vector, serotype 2, containing the human choroideremia gene encoding human Rab escort protein 1	n/a	9/12/2013	Treatment of choroideremia due to mutations in the human choroideremia gene (CHM)	Sparks Therapeutics, Inc.
	abatacept	Orencia	5/30/2013	Treatment of type 1 diabetes mellitus patients with residual beta cell function	Orban Biotech LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	abeotaxane inhibitor of microtubules	n/a	4/18/2011	Treatment of pediatric neuroblastoma	Cortice Biosciences
	abeotaxane inhibitor of microtubules	n/a	10/7/2011	Treatment of gliomas	Cortice Biosciences
	acadesine	n/a	5/4/2011	Treatment of multiple myeloma	Advancell-Advanced In Vitro Cell Technologies S.A.
	acadesine	n/a	3/3/2011	Treatment of chronic lymphocytic leukemia	Avanced In Vitro Cell Technologies, S.L.
	acamprosate	n/a	3/25/2013	Treatment of fragile X syndrome	Confluence Pharmaceuticals, LLC
	acetyl-D-arginyl-D-arginyl-D-arginyl-D-arginyl-D-arginyl-D-arginyl-D-arginyl-D-arginyl-D-cysteinyl-D-glutaminy-D-cystenyl-D-arginyl-D-arginyl-D-lysyl-D-asparaginyldaide disulfide	n/a	11/24/2015	Treatment of acute myeloid leukemia.	Genus Oncology, LLC
	acetyl-l-carnitine	n/a	7/24/2012	Treatment of Fragile X syndrome	Sigma-Tau Pharmaceuticals, Inc.
	acetyl-l-carnitine (ALC)	n/a	3/18/2011	Treatment of Rett syndrome	Sigma-Tau Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	acetylcysteine	Acetadote	10/19/2001	For the intravenous treatment of moderate to severe acetaminophen overdose	Cumberland Pharmaceuticals, Inc.
	acetylcysteine effervescent tablets for oral solution	n/a	2/24/2015	Preventing hepatic injury from acetaminophin overdose	Arbor Pharmaceuticals, LLC
	acyclovir	n/a	12/13/2010	Treatment of acute herpetic keratitis caused by Herpes Simplex Virus type 1 and 2	Fera Pharmaceuticals
	adalimumab	Humira	5/13/2014	Treatment of non-infectious intermediate, posterior, or pan-uveitis, or chronic non-infectious anterior uveitis	AbbVie, Inc.
	adalimumab	Humira	5/13/2015	Treatment of moderate to severe hidradenitis suppurativa (Hurley stage 2 and Hurley stage 3 disease)	AbbVie, Inc.
	adalimumab	n/a	7/10/2014	Treatment of Behcet's disease	Mucora

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adalimumab	Humira	10/19/2006	Treatment of pediatric Crohn's disease.	AbbVie, Inc.
	adalimumab	Humira	3/21/2005	Treatment of juvenile rheumatoid arthritis	AbbVie Inc.
	adalimumab	Humira	5/11/2011	Treatment of pediatric patients with ulcerative colitis	AbbVie, Inc.
	aden-associated virus vector serotype 9 expressing human a-L-iduronidase	n/a	9/29/2015	Treatment of mucopolysacc haridosis Type I (MSP I).	REGENXBIO, Inc.
	adeno associated viral vector containing human ARSB gene	n/a	3/17/2011	Treatment of mucopolysacc haridosis type VI (MPS VI; Maroteaux-Lamy syndrome)	Fondazione Telethon
	adeno associated viral vector serotype rh.10 carrying the human SGSH and SUMF1 cDNAs	n/a	5/6/2013	Treatment of mucopolysacc haridosis type IIIA (Sanfilippo type A syndrome)	Lysogene
	adeno associated virus with modified transthyretin and sequence encoding factor IX variant gene	n/a	3/14/2014	Treatment of hemophilia B	Baxter Healthcare Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adeno-associated viral (AAV) vector serotype rh. 10 encoding the human N-sulfoglycosamine sulphohydrolase cDNA (SGSH)	n/a	11/18/2015	Treatment of mucopolysaccharidosis type IIIA or Sanfilippo type A syndrome	LYSOGENE
	adeno-associated viral vector composed of a bioengineered AAV capsid and a codon-optimized expression cassette encoding a high-specific activity variant of human coagulation factor IX	n/a	9/21/2015	Treatment of hemophilia B.	Spark Therapeutics, Inc.
	adeno-associated viral vector containing DNA encoding an RNAi targeting rhodopsin in combination with an adeno-associated viral vector containing DNA encoding a rhodopsin gene	n/a	12/13/2012	treatment of retinitis pigmentosa	Genable Technologies Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adeno-associated viral vector containing modified U11 snRNA	n/a	9/15/2010	Treatment of Duchenne muscular dystrophy.	uniQure B.V.
	adeno-associated viral vector containing the human NADH Dehydrogenase 4 Gene	n/a	11/20/2013	Treatment of Leber Hereditary Optic Neuropathy	Gen Sight Biologics
	adeno-associated viral vector expressing human retinoschisin-1 gene	n/a	5/21/2007	Treatment of X-linked juvenile retinoschisis (XLRS).	Applied Genetic Technologies Corporation
	adeno-associated viral vector serotype 2 containing the human Rab escort protein 1 gene	n/a	11/5/2014	Treatment of choroideremia	NightstaRx Ltd.
	adeno-associated viral vector serotype 9 containing human N-acetylglucosaminidase alpha gene	n/a	12/27/2012	Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome)	Laboratorios del Dr. Esteve, S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adeno-associated viral vector serotype 9 containing the human cardiac calsequestrin gene	Calmarythm	10/2/2014	Treatment of catecholamine rgic polymorphic ventricular tachycardia	Cardiogen Sciences, Inc.
	adeno-associated viral vector type 2 expressing human recombinant retinal pigment epithelial 65KDa protein gene	n/a	3/18/2015	Treatment of Retinitis Pigmentosa due to autosomal recessive RPE65 gene mutations	Spark Therapeutics, Inc.
	adeno-associated virus serotype 9 expressing the human Survival Motor Neuron gene	n/a	9/30/2014	Treatment of spinal muscular atrophy	AveXis, Inc.
	adeno-associated virus serotype 9 vector containing human Iduronate-2-sulfatase transgene	n/a	7/16/2015	Treatment of mucopolysacc haridosis type II (Hunter syndrome).	Laboratorios del Dr. Esteve, S.A.
	adeno-associated virus transgene of follistatin	n/a	11/19/2012	Treatment of Duchennes and Becker's muscular dystrophy	Milo Biotechnology

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adeno-associated virus type 8 delivering a vector genome with human retinoschisin promoter (RS/IRBP) and the human retinoschisin cDNA (hRS)	n/a	11/16/2015	Treatment of X-linked retinoschisis	Paul A. Sievig, MD, PhD, National Eye Institute, NIH
	adeno-associated virus vector serotype 9 expressing human sulfamidase	n/a	6/1/2011	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A Syndrome).	Laboratorios del Dr. Esteve, S.A.
	adenoviral vector expressing the E.coli purine nucleoside phosphorylase gene and fludarabine	n/a	6/8/2015	Intratumoral treatment of anatomically accessible oral and pharyngeal cancers (lip, tongue, gum, floor of mouth, salivary gland, and other oral cavity)	PNP Therapeutics, Inc.
	adenovirus containing a human FAS-c gene	n/a	4/2/2012	Treatment of malignant glioma	Vascular Biogenics Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	adenovirus delta 24-RGD, an oncolytic adenovirus genetically modified for treatment of glioma	n/a	10/1/2014	Treatment of glioma	DNAtrix, Inc.
	adenovirus vaccine encoding reduced expression in immortalized cell protein	n/a	7/14/2015	Treatment of mesothelioma	MTG Biotherapeutics
	adult adherent bone marrow-derived multipotent stem cells	Multistem	7/6/2012	Treatment of MPS-1, including Hurler syndrome	Athersys, Inc.
	adult hemogenic endothelial cells	n/a	5/27/2015	Treatment of aplastic anemia	HemoGenyx LLC
	aerosolized beractant	n/a	3/11/2013	Treatment of respiratory distress syndrome	Beena G. Sood, MD, MS
	afamelanotide	n/a	5/14/2014	Treatment of familial benign chronic pemphigus (Hailey-Hailey disease)	Clinuvel, Inc.
	afamelanotide	n/a	12/11/2009	Treatment of solar urticaria	Clinuvel, Inc.
	afamelanotide	n/a	7/17/2008	Treatment of erythropoietic porphyrias	Clinuvel Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	afatinib	Gilotrif	12/3/2012	Treatment of epidermal growth factor receptor (EGFR) mutation-positive non-small cell lung cancer (NSCLC).	Boehringer Ingelheim Pharmaceuticals, Inc.
	afatinib	Gilotrif(R)	6/4/2014	Treatment of malignant brain and central nervous system tumors	Boehringer Ingelheim Pharmaceuticals, Inc.
	afatinib	Gilotrif(R)	8/3/2015	Treatment of non-small cell lung cancer with squamous histology.	Boehringer Ingelheim Pharmaceuticals, INC.
	aldoxorubicin	n/a	9/24/2014	Treatment of small cell lung cancer	CytRx Corporation
	aldoxorubicin	n/a	9/24/2014	Treatment of ovarian cancer	CytRx Corporation
	aldoxorubicin	n/a	9/24/2014	Treatment of glioblastoma multiforme	CytRx Corporation
	alectinib	n/a	1/27/2015	Treatment of ALK-positive non-small cell lung cancer	Genentech, Inc.
	alemtuzumab	Campath	10/20/1997	Treatment of chronic lymphocytic leukemia.	Genzyme Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	alfimeprase	n/a	9/20/2012	Treatment of ST-elevation myocardial infarcted patients who are undergoing primary percutaneous coronary intervention	Niche Therapeutics, LLC
	algenpantucel-L	Hyperacute(R)-Pancreatic Cance	10/21/2010	Treatment of pancreatic cancer.	NewLink Genetics Corporation
	alicaforsen	n/a	6/24/2008	Treatment of pouchitis.	Atlantic Healthcare Limited
	alisertib	n/a	5/14/2012	Treatment of peripheral T-cell lymphoma	Millennium Pharmaceuticals, Inc.
	alisertib	n/a	7/12/2013	Treatment of small cell lung cancer	Millennium Pharmaceuticals, Inc.
	all-cis-docosa-4,7,10,13,16,19-hexaenoic acid	Retriacyl	5/21/2014	Treatment of retinitis pigmentosa	Natac Pharma, S.L.
	allantoin	Alwextin	11/21/2002	Treatment of skin blistering and erosions associated with inherited epidermolysis bullosa	Scioderm, Inc.
	allogeneic cardiosphere-derived cells	n/a	4/21/2015	Treatment of Duchenne Muscular Dystrophy	Capricor, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	allogeneic cord blood cells, ex vivo modulated with 16,16 dimethyl prostaglandin E2 (dmPGE2)	n/a	12/2/2010	Enhancement of stem cell engraftment through ex vivo treatment of human allogeneic hematopoietic stem cells (treatment of neuropenia, thrombocytopenia, lymphopenia, and anemia).	Fate Therapeutics, Inc.
	allogeneic ex-vivo expanded placental adherent stromal cells	n/a	2/18/2013	Treatment of Aplastic Anemia	Pluristem Therapeutics, Inc.
	allogeneic ex-vivo expanded placental adherent stromal cells	n/a	8/22/2011	Treatment of thromboangiitis obliterans (Buerger's disease)	Pluristem Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	allogeneic hematopoietic stem cell	Atir	2/2/2010	Prevention (reduction) of transplant related mortality (TRM, which is caused by graft versus host disease and/or infections) following mismatched (haploidentical ) allogeneic hematopoietic stem cell transplantatio n.	Kiadis Pharma Netherlands B.V.
	allogenic retinal epithelial cells transfected with plasmid vector expressing ciliary neurotrophic growth factor	n/a	7/24/2012	Treatment of macular telangiectasia type 2 (MacTel)	Neurotech USA, Inc.
	allopregnanolone	n/a	4/20/2014	Treatment of status epilepticus	Sage Therapeutics
	allopregnanolone	n/a	7/12/2013	Treatment of Neimann-Pick disease, type C	La Jolla Pharmaceutical Company, Inc.
	allosteric activator of the red blood cell-specific form of pyruvate kinase	n/a	3/24/2015	Treatment of pyruvate kinase deficiency	Agios Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	alpha melanotropin	n/a	9/2/2010	Treatment of chronic beryllium disease	mondoBIOTECH Laboratories AG
	alpha-1 proteinase inhibitor (human)	Glassia	7/28/2011	Treatment of patients with recent onset (	Kamada, Ltd.
	alpha-1 proteinase inhibitor (human)	Glassia	10/23/2014	Treatment of graft versus host disease	Kamada Ltd.
	alpha-tocotrienol quinone	n/a	10/21/2010	Treatment of inherited mitochondrial respiratory chain diseases.	Edison pharmaceuticals, Inc.
	alpha1 proteinase inhibitor (human)	n/a	1/29/2010	Treatment of emphysema secondary to congenital alpha1-antitrypsin deficiency.	Grifols Therapeutics, Inc.
	alpha1-proteinase inhibitor (human)	Prolastin(R)-C	3/3/2015	Treatment of Type 1 diabetes mellitus patients with residual beta-cell function.	Grifols Therapeutics, Inc.
	alpha1-proteinase inhibitor (human)	n/a	4/6/2012	Treatment of cystic fibrosis	Grifols Therapeutics, Inc.
	alpha1-proteinase inhibitor (human)	n/a	2/20/2009	Treatment of cystic fibrosis	CSL Behring LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	alteplase	Activase	1/27/2003	Treatment of intraventricular hemorrhage associated with intracerebral hemorrhage	Daniel F. Hanley, MD
	altiratinib	n/a	8/19/2014	Treatment of glioblastoma multiforme.	Deciphera Pharmaceuticals, LLC
	alvocidib	n/a	4/21/2014	Treatment of acute myeloid leukemia	Tolero Pharmaceuticals, Inc.
	amatuximab	n/a	9/28/2012	Treatment of mesothelioma	Morphotek, Inc.
	ambrisentan	Letairis	7/16/2004	Treatment of pulmonary arterial hypertension	Gilead Colorado
	ambroxol	n/a	6/29/2011	Treatment of Gaucher disease	Belrose Pharma, Inc.
	amifampridine phosphate	n/a	3/3/2015	Treatment of congenital myasthenic syndromes	Catalyst Pharmaceuticals
	amifampridine phosphate	n/a	11/12/2009	Treatment of Lambert-Eaton Myasthenic Syndrome	Catalyst Pharmaceuticals, Inc.
	amikacin sulfate	n/a	1/5/2015	Management of cystic fibrosis patients with Pseudomonas aeruginosa lung infections	PlumeStars s.r.l.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	aminolevulinic acid hydrochloride	Levulan	3/20/2007	Treatment of esophageal dysplasia	DUSA Pharmaceuticals, Inc.
	aminosidine	Paromomycin	3/29/2005	Treatment of visceral leishmaniasis	The Institute for One World Health
	amitriptyline and ketamine	Amiket(TM)	1/19/2010	Treatment of postherpetic neuralgia	Immune Pharmaceuicals, Inc.
	ammonium tetrathiomolybdate	n/a	5/5/2008	Treatment of idiopathic pulmonary fibrosis	Pipex Pharmaceuticals, Inc.
	amphotericin B	n/a	9/15/2010	Treatment of visceral leishmaniasis.	iCo Therapeutics Inc.
	amrubicin	n/a	3/10/2008	Treatment of small cell lung cancer	Celgene Corporation
	amylopectin	n/a	3/24/2015	Treatment of glycogen storage disease types Ia and Ib	Michael K. Davis, MD, MBA
	an adeno-associated viral vector containing a codon-optimised human factor IX gene (AAV5-hFIXco)	n/a	12/22/2011	Treatment of hemophilia B	uniQure, B.V.
	anakinra	Kineret	8/19/2010	Treatment of cryopyrin-associated periodic syndromes	Swedish Orphan Biovitrum AB (publ)

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	andexanet alfa	n/a	2/23/2015	For reversing the anticoagulant effect of direct or indirect factor Xa inhibitors in patients experiencing a serious uncontrolled bleeding event or who require urgent or emergent surgery	Portola Pharmaceuticals
	angiotensin (1-7)	n/a	8/30/2013	Treatment of patients requiring stem cell transplantation to accelerate the mobilization of hematopoietic stem cells (CD34+) from the bone marrow to the peripheral blood when combined with a granulocyte colony-stimulating factor	US Biotest, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	angiotensin (1-7)[A(1-7)]	n/a	7/25/2013	Treatment of Duchenne muscular dystrophy	US Biotest, Inc.
	angiotensin 1-7	n/a	1/29/2010	Treatment of sarcoma.	W. Jeffrey Petty, MD
	angiotensin 1-7	n/a	6/17/2010	To accelerate engraftment of hematopoietic cells (treatment of neutropenia, thrombocytopenia, lymphoma, and anemia)in hematopoietic stem cell transplants.	US Biotest, Inc.
	anisina	n/a	7/14/2015	Treatment of neuroblastoma	Novogen Ltd
	antagonist of the complement 5a receptor	n/a	11/17/2014	Treatment of atypical hemolytic uremic syndrome	ChemoCentryx, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	antagonist of the complement 5a receptor	n/a	6/2/2014	Treatment of anti-neutrophil cytoplasmic autoantibodies associated vasculitides (granulomatosis with polyangiitis or Wegener's granulomatosis), microscopic polyangiitis, and Churg-Strauss syndrome.	ChemoCentryx, Inc.
	antagonist of the endosomal Toll-like receptors (TLRs) 7, 8, and 9	n/a	12/23/2014	Treatment of Waldenstrom's macroglobulinemia	Idera Pharmaceuticals, Inc.
	anthrax immune globulin	n/a	9/3/2009	Treatment of inhalation anthrax disease	Emergent Biosolutions, Inc.
	anthrax immune globulin (human)	n/a	7/29/2008	Treatment of inhalational anthrax	Cangene Corp. - Emergent Biosolutions
	anti human Nogo-A human monoclonal antibody	n/a	10/20/2008	Treatment of acute spinal cord injury	Novartis Pharmaceuticals Corporation
	anti-Beta1 integrin monoclonal antibody	n/a	1/13/2015	Treatment of ovarian cancer	OncoSynergy, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	anti-CD3 mAb (SPV-T3a)-ricin A chain fusion protein and anti-CD7 mAb (WT1)-ricin A chain fusion protein	n/a	9/13/2013	Treatment of graft versus host disease	Xenikos BV
	anti-CD30 Fc engineered humanized monoclonal antibody	n/a	12/2/2008	Treatment of Hodgkin lymphoma	Xencor, Inc.
	anti-Lewis Y humanized monoclonal antibody	n/a	3/9/2012	Treatment of ovarian cancer	Recepta Biopharma S.A.
	anti-T-lymphocyte immune globulin, rabbit	Atg-Fresenius(R)	3/26/2010	Prevention of graft versus host disease (GVHD)	Neovii Biotech NA, Inc.
	anti-T-lymphocyte immune globulin, rabbit	n/a	9/12/2008	Prophylaxis of acute allograft rejection in adult recipients in solid organ transplantation	Neovii Biotech NA, Inc.
	anti-TCR murine monoclonal antibody (MAB, type IgM)	n/a	1/12/2011	Treatment of pediatric multiple sclerosis in patients less than or equal to 16 years of age	Tolera Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	anti-TCR murine monoclonal antibody (MAb, type IgM)	n/a	6/7/2010	Treatment of patients 16 years of age and younger with immune-mediated T1DM and preserved pancreatic beta cell function	Tolera Therapeutics, Inc.
	anti-inhibitor coagulant complex	Feiba	4/12/2013	Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in hemophilia A and B patients with inhibitors	Baxalta US, Inc.
	anti-interferon-gamma Fab from goats	n/a	11/18/2003	For the treatment of immunologic corneal allograft rejection	Advanced Biotherapy, Inc.
	anti-sense oligonucleotide consisting of 2' deoxyribose-phosphate backbone with a phosphorothioate backbone	n/a	9/23/2013	Treatment of cystic fibrosis	ProQR Therapeutics B.V.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	anti-thymocyte globulin [rabbit]	Thymoglobulin	5/25/2010	Prophylaxis of acute organ rejection in patients receiving renal transplants	Genzyme Corporation
	anti-tumor necrosis factor (TNF) polyclonal antibody (bovine)	n/a	11/17/2014	Treatment of pediatric ulcerative colitis (0 through 16 years of age)	Avaxia Biologics, Incorporated
	antiangiogenic components extracted from marine cartilage	Neovastat (Ae-941)	10/16/2002	Treatment of renal cell carcinoma	AEterna Zentaris, Inc.
	antibody drug conjugate consisting of fully human anti-guanlyl cyclase C monoclonal antibody linked to the cytotoxic drug monomethyl auristatin E	n/a	7/21/2014	Treatment of gastric cancer	Millennium Pharmaceuticals, Inc.
	antihemophilic factor (recombinant), Fc fusion protein	Eloctate	11/23/2010	Treatment of hemophilia A	Biogen Idec Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	antihemophilic factor (recombinant), porcine sequence	Obizur	3/16/2004	Treatment and prevention of episodic bleeding in patients with inhibitor antibodies to human coagulation factor VIII	Baxter Healthcare Corporation
	antimesothelin-ADC (antibody drug conjugate)	n/a	7/5/2012	Treatment of mesothelioma	Bayer HealthCare Pharmaceuticals, Inc.
	antineoplaston A10, antineoplaston AS2-1	n/a	11/21/2008	Treatment of gliomas	Burzynski Research Institute, Inc.
	antinuclear antibody conjugated liposomal doxorubicin	n/a	2/3/2015	Treatment of Ewing's sarcoma.	NanoSmart Pharmaceuticals, Inc.
	antisense 20-mer phosphorothioate oligonucleotide [complementary to the coding region of R2 component of the human ribonucleotide reductase mRNA]	Gti-2040	3/12/2003	Treatment for renal cell carcinoma	Lorus Therapeutics, Inc
	antisense oligonucleotide targeted to human transthyretin (TTR) mRNA	n/a	7/24/2012	Treatment of familial amyloid polyneuropathy	Isis Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	antroquinonol	n/a	7/23/2015	Treatment of hepatocellular carcinoma	Golden Biotechnology Corporation
	antroquinonol	Hocena(R)	4/30/2015	Treatment of acute myeloid leukemia	Golden Biotechnology Corporation
	antroquinonol	n/a	1/21/2015	Treatment of pancreatic cancer	Golden Biotechnology Corp.
	apolipoprotein E mimetic peptide	n/a	12/3/2012	Treatment of homozygous familial hypercholesterolemia	LipimetiX Development, LLC
	apremilast	n/a	1/17/2013	Treatment of Behcet's disease	Celgene Corporation
	arimoclomol	n/a	3/29/2005	Treatment of amyotrophic lateral sclerosis	CytRx Corporation
	arimoclomol citrate	n/a	1/13/2015	Treatment of Neimann-Pick disease, type C	Orphazyme ApS
	aripiprazole	Abilify	1/25/2006	Treatment of Tourette's syndrome	Otsuka Pharmaceutical Development
	arsenic	Trisenox	11/2/2001	Treatment of acute myelocytic leukemia subtypes M0, M1, M2, M4, M5, M6 and M7	Teva Branded Pharmaceutical Products R&D, Inc.
	arsenic trioxide	Trisenox	6/13/2003	Treatment of liver cancer	Teva Branded Pharmaceutical Products R&D, Inc.
	arsenic trioxide	Trisenox	7/17/2000	Treatment of myelodysplastic syndrome.	Teva Branded Pharmaceutical Products R&D, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	arsenic trioxide	Trisenox	10/18/2001	Treatment of chronic myeloid leukemia	Teva Branded Pharmaceutical Products R*D, Inc.
	arsenic trioxide capsule (oral)	n/a	11/2/2015	Treatment of acute promyelocytic leukemia.	Orsenix Holdings BV
	artemether/lumefantrine	Coartem	8/31/2007	For the treatment of infections due to Plasmodium falciparum or mixed infections including P. falciparum.	Novartis Pharmaceuticals
	arylsulfatase A (rhASA)	n/a	2/27/2008	Treatment of metachromatic leukodystrophy	Shire Human Genetic Therapies, Inc.
	astuprotimut-R	n/a	5/29/2009	Treatment of MAGE-A3 positive non-small cell lung cancer	GlaxoSmithKline Biologicals, S.A.
	ataluren	n/a	3/10/2008	Treatment of spinal muscular atrophy	PTC Therapeutics, Inc.
	ataluren	Translarna	12/10/2014	Treatment of mucopolysaccharidosis type I	PTC Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ataluren	n/a	9/1/2004	For use in the treatment of cystic fibrosis resulting from a nonsense (premature stopcodon) mutation in the cystic fibrosis transmembrane conductance regulatory gene	PTC Therapeutics, Inc.
	ataluren	n/a	1/10/2005	Treatment of Muscular Dystrophy resulting from premature stop mutations in the dystrohin gene	PTC Therapeutics, Inc.
	ataluren	n/a	9/1/2015	Treatment of aniridia	PTC Therapeutics, Inc.
	auranofin	Ridaura	4/30/2010	Treatment of amebiasis	Ctr for Discovery & Innovation in Parasitic Diseas

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	autologous CD34+ bone marrow derived stem cells transduced with a self-inactivating gammaretroviral vector encoding the human IL2RG (yc)	n/a	1/15/2015	Treatment of severe combined immune deficiency-X1	Boston Children's Hospital
	autologous CD34+ hematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human ABCD1 cDNA	n/a	4/19/2012	Treatment of adrenoleukodystrophy	bluebird bio, Inc.
	autologous CD34+ hematopoietic stem cells transduced with LentiGlobin BB305 lentiviral vector encoding the human BA-T87Q-globin gene	n/a	3/18/2013	Treatment of B-thalassemia major and intermedia	bluebird bio, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	autologous CD34+ hematopoietic stem cells transduced with LentiGlobin BB305 lentiviral vector encoding the human BA-T87Q-globin gene	n/a	2/26/2014	For the treatment of Sickle Cell Disease	bluebird bio Inc.
	autologous CD4+/CD8+ T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	n/a	11/6/2014	Treatment of acute lymphoblastic leukemia (inclusive of B-cell acute lymphoblastic leukemia)	Juno Therapeutics, Inc.
	autologous CD4+CD25hiFoxP3+regulatory T cells	n/a	11/19/2013	Prevention of graft rejection following solid organ transplantation	iREG Medical AB
	autologous Epstein-Barr virus specific T-cells	n/a	3/9/2015	Treatment of Epstein-Barr virus positive non-Hodgkin lymphomas.	Cell Medica, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	n/a	3/14/2014	Treatment of chronic lymphocytic leukemia	Novartis Pharmaceuticals Corporation
	autologous adipose derived mesenchymal stromal cells	n/a	10/22/2014	Treatment of amyotrophic lateral sclerosis	Mayo Clinic
	autologous bone marrow CD34+ cells transduced ex vivo with a self activating HIV-1 - based lentiviral vector, EFS-ADA	n/a	10/21/2014	Treatment of adenosine deaminase deficient severe combined immunodeficiency	Donald B. Kohn, MD - Professor, UC-UCLA
	autologous dendritic cells pulsed with allogeneic tumor cell lysate	n/a	5/6/2014	Treatment of malignant mesothelioma	Amphera BV
	autologous ex vivo expanded CD4+-enriched leukocytes treated with the de-methylating agent 5-aza-2'-deoxycytidine	Alecsat	9/13/2013	Treatment of glioblastoma multiforme	CytoVac A/S

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	autologous genetically modified human dermal fibroblasts	n/a	6/10/2014	Treatment of dystrophic epidermolysis bullosa.	Fibrocell Technologies, Inc.
	autologous lymphocytes depleted ex vivo of immunoreactive T cells using 4,5	Theralux Photodynamic Therapy	4/3/2008	Treatment of chronic graft versus host disease	Kiadis Pharma Canada, Inc.
	autologous neo-urinary conduit	n/a	6/7/2011	Treatment of bladder dysfunction requiring incontinent urinary diversion.	Tengion, Inc.
	autologous olfactory neural progenitors	Rhinocytes	12/31/2008	Treatment of amyotrophic lateral sclerosis	RhinoCyte, Inc.
	autologous peripheral blood T lymphocytes transduced with retroviral vector containing anti CD19 CD28/CD3 zeta chimeric antigen receptor	n/a	3/27/2014	Treatment of diffuse large B-cell lymphoma.	Kite Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	autologous tumor cells fused with polyethylene glycol to human allogeneic FO-1, DNA plasmid transfected tumor cell line	n/a	5/6/2015	Treatment of glioblastoma	Sebastiano Gattoni-Celli, MD
	autologous tumor-derived gp96 heat shock protein-peptide complex	Oncophage	5/10/2002	Treatment of renal cell carcinoma	Agenus, Inc.
	autologous tumor-derived gp96 heat shock protein-peptide complex	Oncophage	7/11/2002	Treatment of metastatic melanoma	Agenus, Inc.
	autologous umbilical cord blood	n/a	7/24/2012	Treatment of pediatric (0-16 yrs old inclusive) cerebral palsy due to acquired brain injury	Duke University
	autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene	n/a	8/26/2009	Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency.	Glaxo Group Limited
	avicin d	n/a	3/24/2015	Treatment of multiple myeloma	Avicin Therapeutics Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	aviptadil	n/a	2/22/2005	Treatment of pulmonary arterial hypertension	Mondobiotech Laboratories AG
	azacitidine	Vidaza	12/3/2001	Treatment of myelodysplastic syndromes	Celgene Corporation
	aztreonam	Cayston	3/12/2002	Inhalation therapy for control of gram-negative bacteria in the respiratory tract of patients with cystic fibrosis	Gilead Sciences (formerly Corus Pharma)
	baclofen	n/a	12/16/1991	Treatment of intractable spasticity due to multiple sclerosis or spinal cord injury.	AngioDynamics, Inc.
	baclofen	Gablofen	10/28/2015	Treatment of complex regional pain syndrome.	Mallinckrodt, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	baclofen	Lioresal Intrathecal	11/10/1987	Treatment of intractable spasticity caused by spinal cord injury, multiple sclerosis, and other spinal diseases (including spinal ischemia, spinal tumor, transverse myelitis, cervical spondylosis, and degenerative myelopathy).	Medtronic, Inc.
	baclofen	n/a	12/2/2003	Treatment of dystonia	Medtronic Neurological
	bacterium Bacteroides thetaiotaomicron	n/a	9/26/2013	Treatment of active Crohn's disease in the pediatric population	4D Pharma Research Ltd
	bardoxolone	n/a	3/30/2015	Treatment of pulmonary arterial hypertension	Reata Pharmaceuticals, Inc.
	basimglurant	n/a	3/9/2012	Treatment of Fragile X Syndrome	Hoffmann-La Roche, Inc.
	beclomethasone	n/a	3/4/2009	Treatment of pediatric patients with ulcerative colitis	Aptalis Pharma US, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	beclomethasone 17,21- dipropionate	Orbec(R)	7/24/2009	Treatment of gastrointestina l symptoms with chronic graft versed host disease in patients undergoing allogenic hematopoietic cell transplantatio n	Soligenix, Inc.
	beclomethasone 17,21- dipropionate	Bec	8/28/2001	Prevention of gastrointestina l graft-versus- host disease	Soligenix, Inc.
	beclomethasone 17,21- dipropionate	n/a	11/19/2012	Prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster	Soligenix, Inc
	beclomethasone 17,21- dipropionate	n/a	12/18/2007	Treatment of pediatric patients with Crohn disease.	Soligenix, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bedaquiline; (1R,2S) 6-bromo- alpha-[2- (dimethylamino) ethyl]-2-methoxy- alpha-(1- naphthyl)-beta- phenyl-3- quinolineethanol	Sirturo	1/10/2005	Treatment of active tuberculosis	Janssen Research & Development, LLC
	belatacept	Nulojix	2/20/2008	Prophylaxis of organ rejection in renal allograft recipients	Bristol-Myers Squibb Company
	beloranib	n/a	1/15/2013	Treatment of Prader-Willi syndrome	Zafgen, Inc.
	bendamustine for 50 ml admixture	n/a	7/2/2014	Treatment of chronic lymphocytic leukemia	Eagle Pharmaceuticals, INC.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bendamustine for 50ml admixture	n/a	7/2/2014	Treatment of Follicular Lymphoma, Small Lymphocytic Lymphoma, Lymphoplasma cytic Lymphoma, Splenic Marginal Zone Lymphoma, Extranodal Marginal Zone B-cell Lymphoma of Mucosa- Associated Lymphoma Tissue (MALT), and Nodal Marginal Zone Lymphoma (Collectively Indolent B-cell Non-Hodgkin's Lymphoma)	Eagle Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bendamustine hydrochloride	Treanda	11/26/2013	Treatment of Follicular Lymphoma, Small Lymphocytic Lymphoma, Lymphoplasmacytic Lymphoma, Splenic Marginal Zone Lymphoma, Extranodal Marginal Zone B-cell Lymphoma of Mucosa-Associated Lymphoma Tissue (MALT), and Nodal Marginal Zone Lymphoma (Collectively Indolent B-cell Non-Hodgkin's Lymphoma)	Cephalon, Inc.
	bendamustine hydrochloride with betadex sulfobutyl ether sodium	n/a	9/10/2013	Treatment of chronic lymphocytic leukemia	Supratek Pharma, Inc.
	benznidazole	n/a	4/14/2014	Treatment of Chagas disease	Chemo Research, S.L.
	benzoate	Naben	7/6/2012	Treatment of pediatric schizophrenia	SyneuRx International (Taiwan) Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	benzoate/phenyl acetate	Ammonul	11/22/1993	Treatment of acute hyperammone mia and associated encephalopathy in patients with deficiencies in enzymes of the urea cycle.	Medicis Pharmaceutical Corp.
	beraprost sodium 314d	n/a	12/22/2011	Treatment of pulmonary arterial hypertension	Lung Rx, Inc.
	betamethasone	n/a	10/7/2015	Treatment of Ataxia Telangiectasia.	Grace Therapeutics, LLC
	bevacizumab	Avastin	11/23/2010	Treatment of fallopian tube carcinoma	Genentech, Inc.
	bevacizumab	n/a	10/21/2010	Treatment of hereditary hemorrhagic telangiectasia	Terence M. Davidson, MD
	bevacizumab	Avastin	11/20/2009	In combination with a platinum and 5-FU or capecitabine for the treatment of stomach cancer.	Genentech, Inc.
	bevacizumab	Avastin	2/9/2006	Therapeutic treatment of patients with ovarian cancer	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bevacizumab	Avastin	11/6/2003	Treatment of renal cell carcinoma	Genentech, Inc.
	bevacizumab	Avastin	11/2/2010	Treatment of primary peritoneal carcinoma.	Genentech, Inc.
	bevacizumab	Avastin	5/26/2006	Treatment of malignant glioma	Genentech, Inc.
	bevacizumab	Avastin	10/20/2004	Treatment of pancreatic cancer	Genentech, Inc.
	bevacizumab	Avastin	10/13/2009	Treatment of melanoma stages IIb through IV as part of a combination chemotherapy regimen	Genentech, Inc.
	bezafibrate	Bezalip	7/24/2013	For therapeutic treatment of Barth syndrome	Barth Syndrome Foundation, Inc.
	bi-shRNA furin and GMCSF Autologous Tumor Cell Vaccine	Fang	10/22/2014	Treatment of Ewing's sarcoma	Gradalis, Inc.
	bi-shRNA furin and granulocyte macrophage colony stimulating factor augmented autologous tumor cell vaccine	Fang	4/18/2011	Treatment of ovarian cancer	Gradalis, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bi-shRNAfurin and granulocyte macrophage colony stimulating factor augmented autologus tumor cell vaccine	Fang	2/17/2012	Treatment of stage IIB to IV melanoma	Gradalis, Inc.
	bifidobacterium infantis 35624	n/a	3/24/2008	Treatment of pediatric ulcerative colitis	Alimentary Health Limited
	bifidobacterium longum infantis 35624	n/a	1/16/2003	Treatment of pediatric Crohn's disease	Alimentary Health Limited
	binimetinib	n/a	7/31/2014	Treatment of ovarian cancer	Array Biopharma, Inc.
	binimetinib	n/a	11/19/2013	Treatment Stage IIB-IV melanoma.	Array BioPharma, Inc.
	biocarbonate infusate	Normocarb Hf	8/9/2005	Use in the management of patients undergoing continuous renal replacement therapy with hemofiltration	Dialysis Solutions, Inc.
	bisantrene HCl	n/a	2/14/2014	Treatment of acute myeloid leukemia	Update Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bispecific antibody (monoclonal antibody)	n/a	8/8/2013	Treatment of HER2-expressing advanced adenocarcinoma of the stomach and gastroesophageal junction	Merrimack Pharmaceuticals, inc.
	bispecific antibody (monoclonal antibody)	n/a	8/8/2013	Treatment of HER2-expressing adenocarcinoma of the esophagus	Merrimack Pharmaceuticals, Inc.
	bispecific antibody targeting interleukin 4 (IL-4) and interleukin 13 (IL-13)	n/a	9/14/2011	Treatment of idiopathic pulmonary fibrosis.	Sanofi-Aventis US, Inc.
	bivalent anti-human myostatin adnectin-IgG1	n/a	2/10/2015	Treatment of duchenne muscular dystrophy	Bristol-Myers Squibb Co.
	bivalirudin	Angiomax	11/2/2005	For use as an anticoagulant in patients with or at risk of heparin-induced thrombocytopenia/heparin-induced thrombocytopenia thrombosis syndrome	The Medicines Company

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	blinatumomab	n/a	2/6/2006	Treatment of indolent B-cell lymphoma, excluding CLL and NHL with CNS involvement	Amgen, Inc.
	blinatumomab	n/a	5/16/2008	Treatment of prolymphocytic leukemia	Amgen, Inc.
	blinatumomab	n/a	5/16/2008	Treatment for hairy cell leukemia.	Amgen, Inc.
	blinatumomab	n/a	5/16/2008	Treatment of acute lymphocytic leukemia	Amgen, Inc.
	bortezomib	Velcade	5/30/2012	Treatment of mantle cell lymphoma.	Millennium Pharmaceuticals, Inc.
	bortezomib	Velcade	1/4/2011	Treatment of follicular non-Hodgkin lymphoma	Millennium Pharmaceuticals, Inc.
	bortezomib	Velcade	2/3/2015	Treatment of acute lymphoblastic leukemia	Millennium Pharmaceuticals, Inc.
	bortezomib	Velcade	1/15/2003	Treatment of multiple myeloma	Millennium Pharmaceuticals, Inc.
	bosutinib	Bosulf	2/24/2009	Treatment of chronic myelogenous leukemia	Wyeth Pharmaceuticals, Inc.
	botulism antitoxin heptavalent (A, B, C, D, E, F, G) (Equine)	n/a	6/29/2011	Treatment of botulism.	Cangene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bovine lactoferrin	n/a	2/19/2015	Prevention of late-onset sepsis in very low birth weight infants	Metrodora Therapeutics, Inc.
	bovine lactoferrin	n/a	2/23/2015	Prevention of necrotizing enterocolitis in very low birth weight infants (birth weight less than or equal to 1500 grams)	Metrodora Therapeutics, Inc.
	brentuximab vedotin	Adcetris	10/23/2008	Treatment of anaplastic large cell lymphoma	Seattle Genetics, Inc.
	brentuximab vedotin	Adcetris	4/15/2013	Treatment of patients with peripheral T-cell lymphoma, not otherwise specified	Seattle Genetics, Inc.
	brentuximab vedotin	Adcetris	1/30/2007	Treatment of Hodgkin's lymphoma	Seattle Genetics, Inc.
	brentuximab vedotin	Adcetris	9/13/2013	Treatment of patients with angioimmunoblastic T-cell lymphoma	Seattle Genetics, Inc.
	brentuximab vedotin	Adcetris(R)	1/31/2014	Treatment of patients with diffuse large B-cell lymphoma.	Seattle Genetics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	brentuximab vedotin	Adcetris	11/19/2012	Treatment of mycosis fungoides	Seattle Genetics, Inc.
	brilliant blue G	Brilliant Peel	12/16/2009	For use in ophthalmic surgery to aid in the removal of the inner limiting membrane of the eye.	Fluron GmbH - Subsidiary of Geuder AG
	bryostatin 1	n/a	3/31/2015	Treatment of Fragile X Syndrome.	Neurotrope BioScience, Inc.
	bryostatin-1	n/a	12/3/2001	For use in combination with paclitaxel in the treatment of esophageal cancer	GPC Biotech, Inc.
	budesonide	Uceris	5/6/2013	Treat of ulcerative colitis in pediatric patients aged 0 through 16 years.	Santarus, Inc.
	budesonide	Nefecon	5/17/2010	To slow the progression of immunoglobulin A nephropathy & delay kidney failure in patients affected by the disease.	Pharmalink AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	bupivacaine	Transdur	6/18/2008	Relief of persistent pain associated with postherpetic neuralgia	Impax Laboratories, Inc.
	buprenorphine in combination with naloxone	Suboxone	10/27/1994	Treatment of opiate addiction in opiate users	Reckitt Benckiser Pharmaceuticals, Inc.
	busulfan	Partaject	11/25/2002	Preparative therapy for pediatric patients undergoing bone marrow transplantation	SuperGen, Inc.
	butylidenephthalide	n/a	8/5/2015	Treatment of Malignant Glioma	Everfront Biotech, Inc.
	cabozantinib	Cometriq	11/29/2010	Treatment of follicular, medullary and anaplastic thyroid carcinoma and metastatic or locally advanced papillary thyroid cancer.	Exelixis, Inc.
	caffeine and sodium benzoate	n/a	8/22/2012	Treatment of seizure prolongation in patients undergoing electroconvulsive therapy	Luitpold Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	calcium benzoate and risperidone	n/a	12/20/2010	Treatment of pediatric patients with schizophrenia	University of California, Los Angeles
	calfactant	Infasurf	6/7/1985	Treatment and prevention of respiratory failure due to pulmonary surfactant deficiency in preterm infants.	ONY, Inc.
	calfactant	Infasurf	9/5/2000	Treatment of acute respiratory distress syndrome (ARDS)	ONY, Inc.
	camostat	n/a	5/18/2011	Treatment of chronic pancreatitis	NIXS Corporation
	canakinumab	Ilaris	12/5/2013	Treatment of hyperimmunoglobulinemia D and periodic fever syndrome	Novartis Pharmaceuticals Corporation
	canakinumab	Ilaris	9/30/2008	Treatment of pediatric (age 16 and under) juvenile rheumatoid arthritis.	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	canakinumab	n/a	9/4/2012	Treatment of TNF-receptor associated periodic syndrome (TRAPS)	Novartis Pharmaceuticals Corporation
	canakinumab	Ilaris	12/18/2007	Treatment of cryopyrin-associated periodic syndromes	Novartis Pharmaceuticals Corporation
	canakinumab	Ilaris	12/5/2013	Treatment of familial mediterranean fever	Novartis Pharmaceuticals Corporation
	cannabidiol	n/a	4/22/2015	Treatment of neonatal hypoxic ischemic encephalopathy	GW Pharma Ltd.
	cannabidiol	n/a	7/1/2014	Treatment of Dravet syndrome	Insys Therapeutics, Inc.
	cannabidiol	n/a	6/23/2014	Treatment of Lennox-Gastaut syndrome	Insys Therapeutics, Inc.
	cannabidiol	n/a	9/24/2014	Treatment of glioma	Insys Therapeutics, Inc.
	cannabidiol	n/a	7/23/2015	Treatment of infantile spasms	Insys Therapeutics, Inc.
	cannabidiol	n/a	7/14/2015	Prevention of Graft versus Host Disease (GVHD).	Talent biotechs Ltd.
	cannabidiol	n/a	8/20/2014	Treatment of glioblastoma multiforme	Insys Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cannabidiol	n/a	11/17/2014	Treatment of pediatric schizophrenia (pediatrics is defined as 0 through 16 years of age)	Insys Therapeutics, Inc.
	cannabidiol	n/a	11/14/2013	Treatment of Dravet syndrome.	GW Pharma Ltd.
	cannabidiol;	n/a	2/27/2014	Treatment of Lennox-Gastaut syndrome	GW Pharma Ltd.
	cantharidin	n/a	7/24/2012	Treatment of perforating diseases, including Kyrle's disease, reactive perforating collangenosis, perforating folliculitis and elastosis perforans serpiginosa	Orenova Group, LLC
	cantrixil	n/a	4/20/2015	Treatment of ovarian cancer	CanTx, Inc.
	caprine hyperimmune serum against HIV lysate	n/a	10/28/2009	Treatment of amyotrophic lateral sclerosis	Daval International Limited
	caprine hyperimmune serum against HIV lysate	n/a	2/14/2014	Treatment of systemic sclerosis	Daval International Limited
	capsaicin	n/a	10/23/2002	Treatment of erythromelalgia	Acorda Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	capsaicin	Qutenza	5/2/2003	Treatment of painful HIV-associated neuropathy	Acorda Therapeutics, Inc.
	capsaicin	Qutenza	5/22/2009	Management of neuropathic pain in patients with postherpetic neuralgia	Acorda Therapeutics, Inc.
	carbenoxolone	n/a	7/2/2014	Treatment of Huntington's Disease	Oxalys Pharmaceuticals, Inc.
	carbetocin	n/a	4/11/2014	Treatment of Prader Willi syndrome	Ferring Pharmaceuticals, Inc.
	carbon monoxide	n/a	9/28/2012	Treatment of sickle cell disease	Hillhurst Biopharmaceuticals, Inc.
	carboxy pyrrolidine hexanoyl pyrrolidine carboxylate	n/a	2/10/2015	Treatment of AL amyloidosis	Glaxo Group Limited, England d/b/a GlaxoSmithKline
	cardiotrophin-1	n/a	10/16/2014	Prevention of ischemia-reperfusion injury in kidney transplant recipients	Digna Biotech, S.L.
	cardiotrophin-1	n/a	6/24/2008	To protect the liver from ischemia-reperfusion injury inherent to the procedure of transplantation.	Digna Biotech, S.L.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cardiotrophin-1	n/a	9/13/2011	Treatment of acute liver failure	Digna Biotech S.L.
	carfilzomib	Kyprolis	1/18/2008	Treatment of multiple myeloma	Onyx Therapeutics, Inc.
	carglumic acid	Carbaglu	1/20/1998	Treatment of N-acetylglutamate synthetase deficiency.	Orphan Europe SARL
	carglumic acid	Carbaglu	6/17/2014	Treatment of organic acidemias	Orphan Europe SARL
	carisbamate	n/a	3/16/2012	Management of patients with infantile spasms	SK Life Science, Inc.
	carlumab	n/a	10/24/2006	Treatment of pancreatic cancer	Centocor, Inc.
	catumaxomab	n/a	7/1/2009	Treatment of gastric cancer	Neoviibiotech North America, Inc.
	cediranib	n/a	12/13/2010	Treatment of glioblastoma	National Institutes of Health
	celiprolol	n/a	1/5/2015	Treatment of Ehlers-Danlos syndrome	Acer Therapeutics, Inc.
	cell based therapeutic composed of allogeneic donor apoptotic cells	Apocell	3/18/2013	Prevention of graft versus host disease	Enlivex Therapeutics Ltd.
	cenersen	Aeza	6/18/2008	Treatment of stage IIB through IV melanoma	Eleos, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cenersen	Aezea	2/27/2008	Treatment of chronic lymphocytic leukemia	Eleos, Inc.
	cenersen	n/a	5/8/2006	Treatment of acute myeloid leukemia	Eleos, Inc.
	ceritinib	Zykadia	9/27/2013	Treatment of patients with non-small cell lung cancer (NSCLC) that is anaplastic lymphoma kinase(ALK)-positive	Novartis Pharmaceuticals Corp.
	cethromycin	Restanza(Tm)	9/9/2009	Prophylactic treatment of tularemia due to Francisella tularensis.	Advanced Life Sciences, Inc.
	cethromycin	Restanza(Tm)	9/9/2009	Prophylactic treatment of plague due to Yersinia pestis	Advanced Life Sciences, Inc.
	cethromycin	n/a	2/28/2007	Prophylactic treatment of patients exposed to inhalation anthrax.	Advanced Life Sciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cetuximab	Erbixux	7/3/2000	Treatment of squamous cell cancer of the head and neck in patients who express epidermal growth factor receptor	ImClone Systems Incorporated
	chelmab-y or chelamusab-Y	Oncorad Ov103	4/24/1990	Treatment of ovarian cancer.	Cytogen Corporation
	chenodeoxycholic acid	n/a	2/12/2007	Treatment of cerebrotendinous xanthomatosis	Sigma-Tau Pharmaceuticals, Inc.
	chenodiol	Chenadal	3/22/2010	Treatment of cerebrotendinous xanthomatosis	Retrophin, Inc.
	chenodiol	Chenix	9/21/1984	For patients with radiolucent stones in well opacifying gallbladders, in whom elective surgery would be undertaken except for the presence of increased surgical risk due to systemic disease or age	Solvay

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	chimeric fusion protein of recombinant human alpha-N-acetylglucosaminidase and human insulin-like growth factor 2	n/a	11/25/2014	Treatment of mucopolysaccharidosis III Type B (MPS IIIB, Sanfilippo Syndrome Type B)	BioMarin Pharmaceutical, Inc.
	chimeric monoclonal antibody against Claudin 6	n/a	3/18/2013	Treatment of ovarian cancer	Ganymed Pharmaceuticals AG
	chimeric monoclonal antibody against claudin-18 splice variant 2	n/a	9/26/2013	Treatment of pancreatic cancer	Ganymed Pharmaceuticals AG
	chimeric monoclonal antibody to claudin 18 splice variant 2	n/a	11/20/2012	Treatment of gastric cancer	GANYMED Pharmaceuticals AG
	chlorobutanol/propylene glycol/hydrochloric acid	Patul-End	2/18/1997	Treatment of patulous eustachian tube	Ear Foundation
	chloropyramine	n/a	10/22/2010	Treatment of pancreatic cancer	CurFAKTor Pharmaceuticals, LLC
	chloroquine	n/a	5/20/2015	Treatment of glioblastoma multiforme.	DualTpharma B.,V.
	chlorotoxin	n/a	12/2/2008	Treatment of stage IIb, IIc, III & IV melanoma	Morphotek, Inc.
	chlorotoxin conjugated to indocyanine green dye	n/a	6/23/2015	Diagnostic for the management of malignant brain tumors	Blaze Bioscience, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cholest-4-en-3-one, oxime	n/a	2/17/2009	Treatment of spinal muscular atrophy.	Genentech, Inc.
	cholic acid	Cholbam	7/18/2003	Treatment of inborn errors of cholesterol and bile acid synthesis and metabolism	Retrophin, Inc.
	choline tetrathiomolybdate	n/a	8/25/2011	Treatment of Wilson's disease.	Wilson Therapeutics AB
	chromium picolinate and chromium histidinate	n/a	12/22/2014	Treatment of pediatric polycystic ovary syndrome (0 through 16 years of age)	JDS Therapeutics, LLC
	cinacalcet	Sensipar	4/30/2010	Treatment of hypercalcemia in patients with primary hyperparathyroidism for whom parathyroidectomy would be indicated on the basis of serum calcium levels, but who are unable to undergo surgery	Amgen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cinacalcet	Sensipar	5/12/2003	Treatment of hypercalcemia in patients with parathyroid carcinoma	Amgen, Inc.
	ciprofloxacin	n/a	6/1/2011	The management of bronchiectasis	Aradigm Corporation
	ciprofloxacin DPI	Ciprofloxacin Pulmosphere(R )	2/2/2010	Management of pulmonary infection due to Pseudomonas aeruginosa in cystic fibrosis patients	Bayer HealthCare Pharmaceuticals, Inc.
	ciprofloxacin dry powder inhaler	n/a	4/17/2014	Treatment of non-cystic fibrosis bronchiectasis	Bayer HealthCare Pharmaceuticals, Inc.
	cis-adamantane-2-spiro-3'-8'-[4'-[2'-(4'-morpholinyl)ethoxy]phenyl]-1',2',4'-trioxaspiro[4.5]decane mesylate	n/a	1/19/2010	Treatment of malaria caused by Plasmodium falciparum, Plasmodium vivax, Plasmodium ovale, or Plasmodium malariae	Medicines for Malaria Venture (MMV)
	civamide	n/a	2/17/2009	Treatment of postherpetic neuralgia.	Winston Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	civamide	Zucapsaicin	12/9/2002	Treatment of postherpetic neuralgia of the trigeminal nerve	Winston Laboratories, Inc.
	cladribine	n/a	3/19/2015	Treatment of neuromyelitis optica and neuromyelitis optica spectrum disorder	Chord Therapeutics S.a.r.l.
	clarithromycin, rifabutin, clofazimine	n/a	4/26/2011	Treatment of pediatric Crohn's disease.	RedHill Biopharma Ltd.
	clenbuterol	Spiropent, Ventipulmin	10/27/2014	Adjunctive therapy with enzyme replacement therapy in the treatment of Pompe disease	Duke University Medical Center
	clobazam	Onfi	12/18/2007	Treatment of Lennox-Gastaut Syndrome	Lundbeck, Inc.
	clofarabine	Clofarex	3/14/2002	Treatment of acute myelogenous leukemia	Genzyme Corp (Ilex Products, Inc.)
	clofarabine	Clolar	2/7/2002	Treatment of acute lymphoblastic leukemia	Genzyme Corporation
	clofazimine	Lamprene	6/25/2014	Treatment of active tuberculosis	Novartis Pharmaceuticals Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	coagulation factor IX (recombinant)	Rixubis	10/31/2012	Prophylactic use to prevent or reduce the frequency of bleeding episodes in patients with hemophilia B (routine prophylaxis in patients where there is no evidence or suspicion of bleeding)	Baxalta US, Inc.
	coagulation factor VIIa (recombinant)	Novoseven Rt	6/18/2004	Treatment of bleeding episodes in Glanzmann's thrombasthenia	Novo Nordisk Inc.
	coagulation factor XIII A-subunit (recombinant)	Tretten	11/6/2003	Prophylaxis of bleeding associated with congenital factor XIII deficiency	Novo Nordisk, Inc.
	cobimetinib	n/a	1/31/2014	Treatment of stage IIb, IIc, III, and IV melanoma with BRAFV600 mutation	Genentech, Inc.
	coccidioidin SD Skin Test Antigen	n/a	12/19/2007	For the diagnosis of Coccidioidomycosis	Allermed Laboratories, Inc.
	coenzyme Q10 and d-alpha-tocopherol	n/a	3/14/2011	Treatment of Friedreich's Ataxia.	NBI Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	colchicine	Colcrys	9/25/2007	Treatment of familial Mediterranean fever	AR Holding Company, Inc.
	collagenase clostridium histolyticum	Xiaflex	5/23/1996	Treatment of advanced (involitional or residual stage) Dupuytren's disease.	Auxilium Pharmaceuticals, Inc.
	collagenase clostridium histolyticum	Xiaflex	3/12/1996	Treatment of Peyronie's disease.	Auxilium Pharmaceuticals, Inc.
	combretastatin A 1 diphosphate	n/a	11/19/2012	Treatment of acute myelogenous leukemia	OXIGENE, Inc.
	conjugate of human transferrin and a mutant diphtheria toxin (CRM 107)	Transmid	12/3/2001	Treatment of malignant tumors of the central nervous system	Xenova Biomedix Limited
	conjugated bile acids	Cobartin	7/18/2003	Treatment of steatorrhea in patients with short bowel syndrome	Jarrow Formulas, Inc.
	copanlisib	n/a	2/5/2015	Treatment of follicular lymphoma	Bayer HealthCare Pharmaceuticals, Inc.
	copper histidine	n/a	5/14/2012	Treatment of Menkes disease	Stephen G. Kaler, M.D.
	corifungin	n/a	7/6/2011	Treatment of visceral leishmaniasis	Sandler Center for Drug Discovery

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	corifungin	n/a	8/22/2011	Treatment of amebic meningoencephalitis.	Ctr for Discovery & Innovation in Parasitic Diseases
	creatine	Creapure	2/12/2002	Treatment of amyotrophic lateral sclerosis	Avicena Group, Inc.
	crenolanib	n/a	10/31/2012	Treatment of acute myelogenous leukemia	AROG Pharmaceuticals, LLC
	crenolanib	n/a	3/18/2011	Treatment of soft tissue sarcoma	AROG Pharmaceuticals, LLC
	crenolanib besylate	n/a	12/20/2010	Treatment of malignant glioma	AROG Pharmaceuticals, LLC
	cridanimod	n/a	1/12/2011	Treatment of progesterone receptor negative endometrial cancer in conjunction with progesterone therapy.	Kevelt Ltd.
	crizotinib	Xalkori	9/28/2012	Treatment of anaplastic large cell lymphoma	Pfizer, Inc.
	crizotinib	Xalkori	9/13/2010	Treatment of ALK-positive, MET-positive, or ROS-positive non-small cell lung cancer	Pfizer, Inc.
	crizotinib	Xalkori	10/31/2012	Treatment of neuroblastoma	Pfizer

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cultured, partially T-Cell depleted, allogenic thymic tissue for transplantation	n/a	8/15/2003	As a therapy for primary immune deficiency resulting from athymia associated with complete DiGeorge Syndrome	Duke University Medical Center
	cyclic pyranopterin monophosphate (cPMP)	n/a	11/5/2009	Treatment of molybdenum cofactor deficiency type A (MoCD)	Alexion Pharmaceuticals, Inc.
	cyclo(-γ-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L-phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt	n/a	6/24/2013	Treatment of acromegaly	Aspireo Pharmaceuticals Limited
	cyclocreatine	n/a	6/18/2012	Treatment of creatine transporter deficiency	Lumos Pharma
	cyclophosphamide	Cyrevia(TM)	6/7/2011	Treatment of systemic sclerosis.	Accentia Biopharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cyclophosphamide	Cyrevia(TM)	6/17/2011	Prevention of graft versus host disease following allogeneic hematopoietic stem cell transplant	Accentia Biopharmaceuticals, Inc.
	cyclosporine	n/a	2/17/2009	Treatment of graft-versus-host disease	Sigmoid Pharma Limited
	cyclosporine	n/a	2/17/2009	Prophylaxis of graft-versus-host disease	Sigmoid Pharma Limited
	cyclosporine 2% ophthalmic ointment	n/a	8/1/1991	For use in corneal melting syndromes of known or presumed immunologic etiopathogenesis, including Mooren's ulcer	Allergan, Inc.
	cyclosporine A	n/a	9/30/2008	Prevention of corneal graft rejection	Santen SAS
	cyclosporine A	Nova22007	4/9/2008	Treatment of herpes simplex virus stromal keratitis	Santen SAS
	cyclosporine; ciclosporin	Neurostat(R)/Ciclosporin(R)	11/23/2010	Treatment of moderate to severe traumatic brain injury.	NeuroVive Pharmaceutical AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	cysteamine	n/a	8/6/2008	Treatment of neuronal ceroid lipofuscinoses (Batten disease)	Raptor Pharmaceuticals, Inc.
	cysteamine	n/a	9/11/2013	Treatment of pancreatic cancer	Raptor Pharmaceuticals, Inc.
	cysteamine	n/a	5/9/2008	Treatment of Huntington's disease	Raptor Therapeutics
	cysteamine enteric coated	Procysbi	10/24/2006	Treatment of cystinosis	Raptor Therapeutics, Inc.
	cytarabine liposome	Depocyt	1/30/2007	Treatment of gliomas	Bruce Frankel, MD
	cytochrome C, flavin mononucleotide and thiamin diphosphate	n/a	6/17/2011	Treatment of mitochondrial disorders	NBI Pharmaceuticals, Inc.
	cytochrome P450 isoform 2B1 gene transfected human embryonic kidney 293 cells encapsulated in polymeric cellulose sulphate	n/a	12/17/2014	Treatment of pancreatic cancer in combination with ifosfamide	Nuvilex, Inc.
	d6-tetrabenazine, deutetabenazine	n/a	11/5/2014	Treatment of Huntington's Disease	Teva Pharmaceuticals, Inc.
	dabrafenib	Tafinlar	1/12/2011	Treatment BRAF V600 mutation positive Stage IIB through IV melanoma	Novartis Pharmaceuticals Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	dabrafenib	Tafinlar	10/20/2014	Treatment of patients with BRAF mutation positive non-small cell lung cancer	Novartis Pharmaceuticals Corp.
	dabrafenib and trametinib	n/a	10/29/2015	Treatment of patients with BRAF mutation positive non-small cell lung cancer.	Novartis Pharmaceuticals Corp.
	dacetuzumab	n/a	8/13/2004	Treatment of multiple myeloma.	Seattle Genetics, Inc.
	dacetuzumab	n/a	10/6/2005	Treatment of chronic lymphocytic leukemia	Seattle Genetics, Inc.
	dacomitinib	n/a	3/3/2015	Treatment of non-small cell lung cancer with EGFR, HER2, HER4, or DDR2 mutations.	Pfizer, Inc.
	dalbavancin	Dalvance	3/30/2015	Treatment of acute osteomyelitis in children (0 through 16 years of age)	Durata Therapeutics International B.V.
	dalfampridine	Ampyra	6/2/1987	Relief of symptoms of multiple sclerosis	Acorda Therapeutics
	dantrolene sodium	n/a	9/25/2012	Treatment of heat stroke	Eagle Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	dantrolene sodium suspension for injection	Ryanodex	8/16/2013	Treatment of malignant hyperthermia syndrome	Eagle Pharmaceuticals, Inc.
	daratumumab	Humax (R) - Cd38	11/9/2015	Treatment of diffuse large B-cell lymphoma.	Janssen Research and Development, LLC
	daratumumab	n/a	8/6/2015	Treatment of follicular lymphoma	Janssen Research & Development, LLC
	daratumumab	n/a	8/20/2015	Treatment of mantle cell lymphoma.	Janssen Research & Development, LLC
	daratumumab	Humax(R)-Cd38	5/6/2013	Treatment of multiple myeloma.	Janssen Research & Development, LLC
	dasiprotimut-T	Biovax Id	6/17/2010	Treatment of mantle cell lymphoma	Biovest International, Inc.
	dasiprotimut-T	Biovaxid	10/28/2009	Treatment of follicular lymphoma	Biovest International, Inc.
	davunetide	n/a	12/7/2009	Treatment of progressive supranuclear palsy.	Allon Therapeutics, Inc.
	debrase	Debridase	8/20/2003	Debridement of acute, deep dermal burns in hospitalized patients	MediWound, Ltd.
	decitabine	Dacogen	3/8/1999	Treatment of myelodysplastic syndromes.	Otsuka Pharmaceutical Development
	defactinib	n/a	2/12/2015	Treatment of ovarian cancer	Verastem, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	deferasirox	Exjade	2/24/2015	Treatment of chronic iron overload in alpha-thalassemia	Novartis Pharmaceuticals Corporation
	deferiprone	n/a	9/1/2011	Treatment of superficial siderosis	ApoPharma, Inc.
	deferiprone	Ferriprox	12/12/2001	Treatment of iron overload in patients with hematologic disorders requiring chronic transfusion therapy	ApoPharma, Inc. A Division of
	deferiprone	n/a	7/31/2008	Treatment of Friedreich's ataxia	ApoPharma, Inc.
	deferoxamine starch conjugate	n/a	12/21/1998	Treatment of chronic iron overload resulting from conventional transfusional treatment of beta-thalassemia major and sickle cell anemia.	Biomedical Frontiers, Inc.
	deferoxamine starch conjugate	n/a	1/28/2005	Treatment of acute iron poisoning	Biomedical Frontiers, Inc.
	defibrotide	n/a	1/8/2007	For the prevention of hepatic veno-occlusive disease.	Gentium SpA

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	defibrotide	n/a	5/21/2003	For the treatment of hepatic veno-occlusive disease	Gentium SpA
	deflazacort	Calcort	9/16/2010	Treatment of Duchenne muscular dystrophy	University of Rochester Medical Center
	deflazacort	n/a	8/16/2013	Treatment of Duchenne muscular dystrophy	Marathon Pharmaceuticals, LLC
	deflazacort	n/a	10/22/2015	Treatment of pediatric (0 through 16 years of age) juvenile idiopathic arthritis (JIA) International League of Associations for Rheumatology (ILAR) categories excluding systemic JIA.	Marathon Pharmaceuticals, LLC
	dehydrated alcohol	n/a	3/16/2012	Treatment of trigeminal neuralgia	Luitpold Pharmaceuticals, Inc.
	dehydrated alcohol	Ablysinol	9/11/2013	Treatment of hypertrophic obstructive cardiomyopathy	Belcher Pharmaceuticals, LLC
	delta-1,4,9(11)-pregnatriene-17-alpha,21-dihydroxy-16-alpha-methyl-3,20-dione	n/a	12/2/2011	Treatment of Duchenne muscular dystrophy	ReveraGen Biopharma

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	demcizumab	n/a	4/30/2014	Treatment of pancreatic cancer	OncoMed Pharmaceuticals, Inc.
	dendritic cells pulsed with synthetic peptides derived from antigens MAGE1, HER-2, AIM-2, TRP-2, gp100 and interleukin-13 receptor alpha	n/a	6/7/2010	Treatment of glioblastoma or brain stem glioma	ImmunoCellular Therapeutics Ltd.
	dendritic hybrid cell vaccine	Neuroblaxin	9/23/2011	Treatment of neuroblastoma	Orbis Health Solutions, LLC
	denileukin diftitox	n/a	7/12/2013	Treatment of cutaneous T-cell lymphoma	Eisai Inc.
	denileukin diftitox	Ontak	4/30/2010	Treatment of peripheral T-cell lymphoma	Eisai, Inc.
	denileukin diftitox	n/a	6/29/2011	Treatment of peripheral T-cell lymphoma (PTCL)	Eisai, Inc.
	denileukin diftitox	Ontak	8/21/1996	Treatment of patients with cutaneous T-cell lymphoma	Eisai, Inc.
	denosumab	Xgeva	9/11/2013	Treatment of hypercalcemia in malignancy	Amgen, Inc.
	denosumab	Xgeva	12/20/2010	Treatment of patients with giant cell tumor of bone	Amgen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	deutetrabenazine	n/a	1/13/2015	Treatment of Tourette syndrome in the pediatric population (defined as 0 through 16 years of age)	Auspex Pharmaceuticals
	dexamethasone intravitreal implant	Ozurdex	9/11/1998	Treatment of non-infectious ocular inflammation of the posterior segment in patients with intermediate, posterior, and panuveitis	Allergan
	dexamethasone phosphate	n/a	12/2/2008	Treatment of corneal graft rejection.	EyeGate Pharmaceuticals, Inc.
	dexamethasone sodium phosphate encapsulated in autologous erythrocytes	n/a	7/24/2012	Treatment of ataxia-telangiectasia	EryDel S.p.A.
	dexanabinol	n/a	8/11/2004	For the attenuation or amelioration of the long-term neurological sequelae associated with moderate and severe traumatic brain injury	Pharmos Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	dexpramipexole	n/a	10/11/2007	Treatment of amyotrophic lateral sclerosis	Knopp Biosciences LLC
	dexrazoxane	Totect(R)	3/25/2004	Treatment of anthracycline extravasation during chemotherapy	Biocodex
	dexrazoxane hydrochloride	n/a	8/19/2014	Prevention of cardiomyopathy for children and adolescents 0 through 16 years of age treated with anthracyclines	Satiscor, LLC
	dextran 1	n/a	3/21/2003	Treatment of cystic fibrosis	BCY LifeSciences Inc.
	diacerein	n/a	10/15/2014	Treatment of epidermolysis bullosa	TWI Biotechnology, Inc.
	diannexin	n/a	10/28/2009	Prevention of ischemia-reperfusion injury in solid organ transplants	Astellas Pharma Global Development, Inc.
	diazepam (intranasal)	n/a	7/31/2012	Management of patients with acute repetitive seizures	Acorda Therapeutics, Inc.
	diazepam (intranasal)	n/a	11/16/2015	Management of acute repetitive seizures.	Neurelis Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	diazepam auto-injector	n/a	5/30/2013	Management of selected, refractory patients with epilepsy on stable regimens of antiepileptic drugs, who require intermittent use of diazepam to control bouts of increased seizure activity	Meridian Medical Technologies-a Pfizer subsidiary
	diazoxide	n/a	12/3/2012	Treatment of Prader Willi Syndrome	Sedogen, LLC
	diazoxide choline	n/a	5/13/2014	Treatment of Prader-Willi Syndrome	Essentialis, Inc.
	dichlorphenamide	n/a	9/2/2010	Treatment of primary periodic paralyses	Taro Pharmaceuticals
	diethylenetriaminepentaacetate (DPTA)	n/a	4/14/2004	For treatment of patients with known or suspected internal contamination with plutonium, americium or curium to increase the rates of elimination.	CIS-US
	diferuloylmethane	n/a	6/13/2003	Treatment of cystic fibrosis	Allertein Therapeutics, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	difluprednate	Durezol	9/30/2008	Treatment of endogenous and traumatic anterior uveitis and panuveitis.	Alcon Pharmaceuticals. Ltd.
	digitoxin	n/a	5/27/2005	Treatment of cystic fibrosis	Silver Pharmaceuticals
	digitoxin	n/a	11/2/2001	Treatment of ovarian cancer	SimRx Advisors LLC
	digitoxin	n/a	10/18/2001	Treatment of soft tissue sarcomas	SimRx Advisors LLC
	digoxin immune fab (ovine)	n/a	2/3/2012	Treatment of severe preeclampsia and eclampsia	Velo Bio, LLC
	dihydroartemisinin and piperazine	Eurartesim	1/8/2007	Treatment of uncomplicated malaria caused by "Plasmodium falciparum," Plasmodium vivax," Plasmodium malariae," or "Plasmodium ovale."	Sigma-Tau Pharmaceuticals, Inc.
	dimebon	n/a	5/12/2009	Treatment of Huntington's Disease.	Medivation, Inc.
	dimethyl fumarate	n/a	9/11/2013	Treatment of Friedreich's Ataxia	Gino Cortopassi

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	dimethyl sulfoxide	n/a	5/9/2008	For use in combination with antimicrobial drugs for the treatment of drug resistant tuberculosis	Abela Pharmaceuticals, Inc.
	dinaciclib	n/a	8/25/2011	Treatment of chronic lymphocytic leukemia.	Merck Sharp & Dohme Ltd.
	dinutuximab	n/a	12/20/2010	Treatment of neuroblastoma	United Therapeutics Corporation
	diphenylcyclopentone	n/a	6/13/2003	Treatment of chronic severe forms of alopecia areata (Alopecia Totalis [AT]/Alopecia Universalis [AU])	Lloyd E. King, Jr.
	docosahexaenoic acid	n/a	6/1/2015	Treatment of short bowel syndrome	Sancilio and Company, Inc.
	docosahexaenoic acid	n/a	4/27/2015	Treatment of sickle cell disease	Sancilio & Company, Inc.
	docosahexaenoic acid, DHA	n/a	12/17/2014	Treatment of primary sclerosing cholangitis	Sancilio and Company, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	domperidone	n/a	9/2/2011	Treatment of hypoprolactinemia in breastfeeding mothers, and in some hypoprolactinemic conditions following the use of cabergoline or bromocriptine in mothers who wish to initiate or return to breastfeeding	Thomas W. Hale, RPh, PhD
	double stranded RNA which targets the mutated KRAS oncogene	n/a	1/26/2015	Treatment of pancreatic cancer	Silenseed Ltd
	double stranded oligomer AD00370 RNA interference-based liver targeted therapeutic	n/a	6/9/2015	Treatment of Alpha-1 Antitrypsin deficiency	Arrowhead Research Corporation
	dovitinib	n/a	9/26/2013	Treatment of adenoid cystic carcinoma	Novartis Pharmaceuticals Corporation
	doxofylline	n/a	2/14/2014	Treatment of bronchiectasis	Alitair Pharmaceuticals, Inc.
	doxorubicin	n/a	8/25/2009	Treatment of hepatocellular carcinoma.	Delcath Systems, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	doxorubicin	n/a	4/6/2015	Treatment of cutaneous T-cell lymphoma	Louis D. Faló, Jr.
	doxorubicin HCL liposome injection	Doxil	12/29/2004	Treatment of multiple myeloma	Johnson & Johnson Pharmaceutical Research & Dev.
	doxorubicin PIHCA nanoparticles	Doxorubicin Transdrug	3/14/2005	Treatment of hepatocellular carcinoma	BioAlliance Pharma
	doxorubicin with pluronics F-127 and L-61	n/a	2/20/2008	Treatment of gastric cancer	Supratek Pharma, Inc.
	droxidopa	Northera	1/17/2007	Treatment of neurogenic symptomatic orthostatic hypotension in patients with primary autonomic failure, dopamine-beta-hydroxylase deficiency, and nondiabetic autonomic neuropathy.	Lundbeck LLC
	dry extract from Betulae Cortex (birch bark)	n/a	8/7/2014	Treatment of epidermolysis bullosa	Birken AG
	duvelisib	n/a	4/15/2013	Treatment of chronic lymphocytic leukemia and small lymphocytic lymphoma	Infinity Pharmaceuticals
	duvoglustat hydrochloride	n/a	6/18/2007	Treatment of Pompe disease	Amicus Therapeutics, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ecallantide	Kalbitor	2/4/2003	Treatment of angioedema	Dyax Corp.
	echinomycin	n/a	5/21/2015	Treatment of acute myeloid leukemia.	OncolImmune, Inc.
	echothiophate iodide	n/a	6/2/2014	Treatment of Stargardt's disease	Makindus, Inc.
	ecopipam hydrochloride	n/a	7/21/2009	Symptomatic treatment of self injurious behaviors in patients with Lesch-Nyhan disease.	Psyadon Pharmaceuticals, Inc.
	ecopipam hydrochloride	n/a	9/29/2010	Treatment of Tourette's syndrome in children 0-16 years old.	Psyadon Pharmaceuticals, Inc.
	eculizumab	Soliris	10/18/2011	Treatment of Shiga-Toxin producing escherichia coli hemolytic uremic syndrome	Alexion Pharmaceuticals, Inc.
	eculizumab	n/a	3/5/2001	Treatment of idiopathic membranous glomerular nephropathy	Alexion Pharmaceuticals, Inc.
	eculizumab	Soliris	1/10/2014	Prevention of delayed graft function after renal transplantation	Alexion Pharmaceuticals, Inc.
	eculizumab	Soliris	6/24/2013	Treatment of neuromyelitis optica	Alexion Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	eculizumab	Soliris	8/20/2003	Treatment of paroxysmal nocturnal hemoglobinuria	Alexion Pharmaceuticals, Inc.
	eculizumab	n/a	6/12/2014	Treatment of Myasthenia Gravis.	Alexion Pharmaceuticals, Inc.
	eculizumab	Soliris	4/29/2009	Treatment of atypical hemolytic uremic syndrome	Alexion Pharmaceuticals, Inc.
	edaravone	n/a	3/12/2015	Treatment of amyotrophic lateral sclerosis	Treeway B.V.
	efaproxiral	n/a	7/28/2004	Adjunct to whole brain radiation therapy for the treatment of brain metastases in patients with breast cancer	Allos Therapeutics, Inc.
	eflornithine	n/a	2/4/2011	Treatment of Familial Adenomatous Polyposis	Cancer Prevention Pharmaceuticals
	eflornithine	n/a	11/23/2010	Treatment of neuroblastoma	Cancer Prevention Pharmaceutical, Inc.
	eflornithine HCL	n/a	4/7/2015	Treatment of gastric cancer	Cancer Prevention Pharmaceuticals, Inc.
	eflornithine plus sulindac	n/a	1/22/2013	Treatment of familial adenomatous polyposis	Cancer Prevention Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	eicosapentaenoic acid	n/a	3/8/2011	Treatment of familial adenomatous polyposis	S.L.A. Pharma Ltd. (UK)
	elacytarabine	n/a	6/18/2008	Treatment of acute myeloid leukemia (AML)	Clavis Pharma ASA
	eliglustat	Cerdelga	9/17/2008	Treatment of Type I Gaucher disease	Genzyme Corporation
	elosulfase alfa	Vimizim	5/15/2009	Use in the treatment of mucopolysaccharidosis (MPS) Type IV A (Morquio A syndrome)	BioMarin Pharmaceutical Inc.
	elotuzumab	n/a	9/1/2011	Treatment of multiple myeloma	Bristol-Myers Squibb Company
	eltrombopag	Promacta	11/8/2013	Treatment of aplastic anemia	Novartis Pharmaceuticals Corp.
	eltrombopag	Promacta	5/5/2008	Treatment of idiopathic thrombocytopenia purpura	Novartis Pharmaceuticals Corp.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	emricasan	n/a	11/20/2013	Treatment of liver transplant recipients with reestablished fibrosis to delay the progression to cirrhosis and end stage liver disease	Conatus Pharmaceuticals Inc.
	enalapril maleate (powder for oral solution)	Epaned	1/30/2013	Treatment of hypertension in pediatric patients	Silvergate Pharmaceuticals, Inc.
	encapsulated spores from fecal microbiota	n/a	8/19/2015	Treatment of recurrent Clostridium difficile infection (CDI)	Seres Health, Inc.
	enochleate amphotericin B	n/a	1/10/2014	Treatment of visceral leishmaniasis	Aquarius Biotechnologies, Inc.
	encorafenib	n/a	11/19/2013	Treatment of Stage IIB-IV melanoma positive for BRAF mutation	Array BioPharmas, Inc.
	encorafenib + binimetinib	n/a	11/19/2013	Treatment in Stage IIB-IV melanoma positive for the BRAF mutation.	Array BioPharma, Inc.
	eniluracil	n/a	12/15/2005	Treatment of hepatocellular carcinoma.	Adherex Technologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ensituximab	n/a	10/21/2010	Treatment of pancreatic cancer.	Neogenix Oncology, Inc.
	entrectinib;N-[5-(3,5-difluorobenzyl)-1H-indazol-3-yl]-4-(4-methylpiperazin-1yl)-2-(tetrahydro-2H-pyran-4-ylamino)benzamide	n/a	12/22/2014	Treatment of neuroblastoma	Ignyta, Inc.
	enzastaurin	n/a	3/4/2009	Treatment of diffuse large B-cell lymphoma	Denovo Biopharma LLC
	epoprostenol	Flolan	3/22/1999	Treatment of secondary pulmonary hypertension due to intrinsic precapillary pulmonary vascular disease.	GlaxoSmithKline
	epratuzumab	Lymphocide	7/13/1998	Treatment of non-Hodgkin's lymphoma	Immunomedics, Inc.
	epratuzumab	n/a	9/30/2008	Treatment of acute lymphoblastic leukemia	Immunomedics, Inc.
	eptifibatid and iloprost	n/a	4/20/2012	Treatment of purpura fulminans	Thrombologic
	erdosteine	n/a	12/20/2013	Treatment of bronchiectasis.	Alitair Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	eribulin mesylate	Halaven(R)	5/14/2012	Treatment of advanced soft tissue sarcoma	Eisai, Inc.
	estradiol Gel	n/a	10/31/2006	Estrogen replacement therapy in females with Turner syndrome	Ascend Therapeutics US, LLC
	etanercept	Enbrel	10/27/1998	Reduction in signs and symptoms of moderately to severely active polyarticular-course juvenile rheumatoid arthritis in patients who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs.	Immunex Corporation
	etarfolatide	n/a	2/16/2000	For the identification of ovarian carcinomas	Endocyte, Inc.
	eteplirsen	n/a	10/23/2007	Treatment of Duchenne Muscular Dystrophy.	Sarepta Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ethanolamine	Ethamolin	1/17/2014	Prophylactic use in pediatric patients (age 0 through 16 years) with esophageal varices that are at risk of bleeding to obliterate varices and to prevent bleeding	QOL Medical, LLC
	ethiodized oil injection	Lipiodol	9/26/2013	Management of patients with known hepatocellular carcinoma (HCC)	Guerbet LLC
	etirinotecan pegol	n/a	4/18/2011	Treatment of ovarian cancer.	Nektar Therapeutics
	everolimus	Afinitor	6/8/2009	Treatment of tuberous sclerosis complex including TSC-associated subependymal giant cell astrocytoma (SEGA), TSC-associated angiomyolipoma and TSC-associated lymphangiomyomatosis (LAM)	Novartis Pharmaceuticals Corporation
	everolimus	Afinitor	2/14/2008	Treatment of neuroendocrine tumors	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	everolimus	n/a	6/2/2014	Treatment of diffuse large B-cell lymphoma	Novartis Pharmaceuticals Corp.
	everolimus	Afinitor	7/23/2012	Treatment of hepatocellular carcinoma	Novartis Pharmaceuticals Corporation
	everolimus ointment	n/a	9/10/2015	Topical treatment of Tuberous Sclerosis Complex-related skin lesions	Aucta Pharmaceuticals, LLC
	evolocumab	Repatha	9/12/2013	Treatment of homozygous familial hypercholesterolemia	Amgen Inc.
	ex vivo cultured human mesenchymal stromal cells	n/a	5/8/2014	Prevention of graft rejection following solid organ transplantation	iCell Science AB
	ex-vivo cultered adult human mesenchymal stem cells	Prochymal(R)	4/30/2010	Treatment of Type 1 diabetes patients with residual beta cell function	Mesoblast, Inc.
	ex-vivo expanded autologous bone marrow-derived mesenchymal stem cells	n/a	12/20/2010	Treatment of Amyotrophic Lateral Sclerosis	TCA Cellular Therapy, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	exendin-(9-39)	n/a	6/1/2011	Treatment of congenital hyperinsulinemic hypoglycemia and other causes of hyperinsulinemic hypoglycemia in adults and children	The Children's Hospital of Philadelphia
	exon 53 specific phosphorothioate oligonucleotide	n/a	1/23/2013	treatment of Duchenne Muscular Dystrophy patients bearing mutations that can be corrected by skipping exon 53	Prosensa Therapeutics B.V.
	expanded allogeneic human dermal fibroblasts in hypothermosol(r)-FRS	n/a	8/20/2009	Treatment of Dystrophic Epidermolysis Bullosa.	Intercytex Ltd.
	expanded human allogeneic neural retinal progenitor cells extracted from neural retina	n/a	8/22/2013	Treatment of retinitis pigmentosa	ReNeuron Ltd
	extract of sorghum bicolor extract	n/a	11/19/2012	Treatment of sickle cell disease	Invenux, LLC
	ezatiostat hydrochloride	Telintra	1/9/2013	Treatment of myelodysplastic syndrome	Telik, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	factor VIII mimetic bispecific antibody	n/a	1/10/2014	Treatment of hemophilia A	Genentech
	factor XIII concentrate (human)	Corifact	1/16/1985	Treatment of congenital factor XIII deficiency	CSL Behring LLC
	farletuzumab	n/a	6/16/2006	Treatment of ovarian cancer	Morphotek, Inc.
	fecal microbiota	n/a	3/10/2014	Treatment of recurrent Clostridium difficile infection (Clostridium difficile gastrointestinal disease)	Rebiotx, Inc.
	fenfluramine HCl	Brabafen	12/20/2013	For the Treatment of Dravet Syndrome	Zogenix, Inc.
	fenretinide	n/a	9/4/2013	Treatment of peripheral T-cell lymphoma	CerRx, Inc.
	fenretinide	n/a	9/4/2013	Treatment of cutaneous T-cell lymphoma	CerRx, Inc.
	fenretinide	n/a	2/1/2007	Treatment of Ewing's sarcoma family of tumors.	Cancer Research UK

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ferric hexacyanoferrate (II) "Prussian Blue"	n/a	6/26/2003	Treatment of patients with known or suspected internal contamination with radioactive or non-radioactive cesium or thallium	Degussa AG
	ferumoxytol	n/a	10/7/2011	For use in magnetic resonance imaging in brain metastases	Oregon Health & Science University
	ferumoxytol	n/a	4/6/2012	For use in magnetic resonance imaging to assess, and monitor treatment of solid tumor malignancies previously diagnosed in pediatric patients (age 16 years and younger)	ArsNova Partners, LLC
	ferumoxytol	Feraheme	4/29/2011	For use in MR imaging for the mangement of brain tumors	Edward A. Neuwelt, MD
	fialuridine	n/a	7/24/1992	Adjunctive treatment of chronic active hepatitis B.	Oclassen Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	fidaxomicin	Dificid	12/13/2010	Treatment of pediatric Clostridium difficile infection	Optimer Pharmaceuticals, Inc.
	filanesib	n/a	5/6/2014	Treatment of multiple myeloma.	Array BioPharma, Inc.
	filgrastim	n/a	1/27/2015	Treatment of amyotrophic lateral sclerosis (ALS)	Neurovision Pharma GmbH
	filgrastim	Neupogen	11/20/2013	Treatment of subjects at risk of developing myelosuppression after a radiological or nuclear incident	Amgen, Inc.
	filociclovir	n/a	11/29/2010	Treatment of active cytomegalovirus infections	Microbiotix, Inc.
	fingolimod	n/a	4/30/2010	Treatment of chronic inflammatory demyelinating polyneuropathy	Novartis Pharmaceutical Corporation
	firtecan pegol	n/a	4/18/2011	Treatment of neuroblastoma	Enzon Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	flubendazole	n/a	1/23/2014	Treatment of lymphatic filariasis caused by nematodes of the family Filarioididea, in children and adults.	Janssen Research and Development, LLC
	flubendazole	n/a	10/25/2013	Treatment of onchocerciasis caused by Onchocerca volvulus	Janssen Research & Development, LLC
	fluciclovine (18F)(anti-1-amino-3-fluorocyclobutane-1-carboxylic acid, labelled with Fluorine-18)	n/a	4/7/2015	For the diagnosis of glioma	Blue Earth Diagnostics Ltd.
	flunarizine hydrochloride	n/a	6/24/2013	Treatment of alternating hemiplegia	Marathon Pharmaceuticals, LLC
	fluticasone propionate	n/a	1/19/2011	Treatment of pediatric and adult eosinophilic esophagitis	Forest Laboratories, Inc.
	fosfomycin/tobramycin	n/a	11/28/2008	Treatment of pulmonary infections associated with cystic fibrosis	CURx Pharmaceuticals, Inc.
	fostamatinib disodium	n/a	8/25/2015	Treatment of immune thrombocytopenic purpura	Rigel Pharmaceuticals, INC.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	fresolimumab	n/a	10/21/2010	Treatment of primary focal segmental glomerulosclerosis	Genzyme, a Sanofi Company
	fully human IgG2 monoclonal antibody that binds insulin receptors	n/a	6/9/2015	Treatment of congenital hyperinsulinism	XOMA (US) LLC
	fusion protein analog with recombinant human growth hormone (rhGH) at once-a-month dosing	n/a	10/16/2013	Treatment of growth hormone deficiency	Versartis, Inc.
	gabapentin	Gralise	11/8/2010	Management of postherpetic neuralgia	Depomed, Inc.
	gabapentin	Neurontin	7/5/1995	Treatment of amyotrophic lateral sclerosis	Warner-Lambert Company
	gabapentin enacarbil	Horizant	6/7/2011	Treatment of postherpetic neuralgia	XenoPort, Inc.
	ganaxolone	n/a	3/24/2015	Treatment of Protocadherin 19 (PCDH19) female epilepsy.	Marinus Pharmaceuticals, Inc.
	ganciclovir	Zirgan	3/22/2007	Treatment of acute herpetic keratitis (dendritic and geographic ulcers)	Sirion Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ganitumab	n/a	11/23/2010	Treatment of pancreatic cancer.	Amgen
	gefitinib	Iressa	8/26/2014	Treatment of epidermal growth factor receptor mutation-positive non-small cell lung cancer	AstraZeneca Pharmaceuticals LP
	gemcabene	n/a	2/6/2014	Treatment of homozygous familial hypercholesterolemia	Gemphire Therapeutics, Inc.
	gemcitabine ready-to-use	n/a	6/24/2015	Treatment of ovarian cancer	Sun Pharmaceutical Industries Ltd.
	gemcitabine ready-to-use	n/a	6/24/2015	Treatment of pancreatic cancer	Sun Pharmaceutical Industries Ltd.
	gene encoding chimeric CD40 ligand	n/a	2/4/2009	Treatment of chronic lymphocytic leukemia	Memgen, LLC
	genetically engineered herpes simplex virus (G207)	n/a	4/29/2002	Treatment of malignant glioma	Aettis, Inc.
	gevokizumab	n/a	2/21/2014	Treatment of pyoderma gangrenosum	XOMA (US) LLC
	gevokizumab	n/a	7/27/2010	Treatment of Behcet's disease	XOMA (US) LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	gevokizumab	n/a	8/20/2012	Treatment of non-infectious intermediate, posterior or pan uveitis, or chronic non-infectious anterior uveitis	XOMA (US) LLC
	givinostat	n/a	4/12/2013	Treatment of Duchenne Muscular Dystrophy and Becker Muscular Dystrophy	Italfarmaco SpA
	glatiramer acetate	Copaxone	11/14/2007	Treatment of amyotrophic lateral sclerosis (ALS).	Teva Neurosciences, Inc.
	glioma derived cell lysates and irradiated cells	n/a	1/12/2011	Treatment of glioma.	Epitopoietic Research Corp.
	glucagon	n/a	12/5/2012	Prevention of hypoglycemia in the congenital hyperinsulinism population	Biodel, Inc.
	glucagon infusion	G-Pump (Tm)	9/25/2014	Prevention of chronic, severe hypoglycemia related to congenital hyperinsulinism	Xeris Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	glucarpidase	Voraxaze	8/19/2003	Treatment of patients at risk of methotrexate toxicity	BTG International Inc.
	glufosfamide	n/a	9/18/2006	For treatment of pancreatic cancer.	Eleison Pharmaceuticals LLC
	glyburide	n/a	9/29/2015	Treatment of acute spinal cord injury	Remedy Pharmaceuticals, Inc.
	glycafilin	n/a	8/22/2008	Prevention of delayed graft function after solid organ transplantation	ProtAffin Biotechnologie AG
	glycerol phenylbutyrate	Ravicti	4/27/2009	Maintenance treatment of patients with deficiencies in enzymes of the urea cycle	Hyperion Therapeutics, Inc.
	glycopyrrolate	Cuvposa	6/9/2006	Treatment of pathologic (chronic moderate to severe) drooling in pediatric patients	Shionogi, Inc.
	glycopyrrolate 2%	n/a	6/17/2010	Treatment of Frey's syndrome	Wellesley Therapeutics, Inc.
	glycosylated recombinant human interleukin-7	n/a	9/27/2012	Treatment of progressive multifocal leukoencephalopathy	Cytheris, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	glycyl-L-2-methylprolyl-L-glutamic Acid	n/a	10/23/2013	Treatment of Fragile X Syndrome	Neuren Pharmaceuticals, Ltd.
	glycyl-L-2-methylpropyl-L-glutamic acid	n/a	2/11/2015	Treatment of Rett syndrome	Neuren Pharmaceuticals, Ltd.
	golimumab	Simponi Aria	4/2/2015	Treatment of polyarticular juvenile idiopathic arthritis in pediatric patients (0 through 16 years of age)	Janssen Research & Development, LLC
	golimumab	Simponi	3/16/2012	Treatment of pediatric ulcerative colitis	Janssen Biotech, Inc.
	golnerminogene pradenovec	Tnferade(Tm) Biologic	10/28/2009	Treatment of pancreatic cancer.	GenVec, Inc.
	granulocyte macrophage colony stimulating factor	n/a	8/27/2008	Treatment of cystic fibrosis	DrugRecure Aps
	granulocyte-macrophage colony stimulating factor-coding oncolytic adenovirus, Ad5/3-D24-GMCSF	n/a	7/24/2013	Treatment of soft tissue sarcoma	Oncos Therapeutics
	guadecitabine	n/a	9/29/2015	Treatment of acute myeloid leukemia.	Astex Pharmaceuticals, Inc.
	guanfacine	Tenex	8/5/1999	Treatment of fragile X syndrome.	Watson Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	gusperimus trihydrochloride	n/a	6/29/2011	Treatment of Wegener's granulomatosis	Nordic Group B.V.
	glyceryl tri (4-pheynlybutyrate)	n/a	9/3/2009	For intermittent or chronic treatment of patients with cirrhosis and any grade hepatic encephalopathy.	Hyperion Therapeutics, Inc.
	halofuginone hydrobromide	n/a	10/13/2011	Treatment of Duchenne Muscular Dystrophy	Halo Therapeutics, LLC
	heat killed Mycobacterium w immunomodulator	Cadi Mw	9/3/2004	Active tuberculosis	Cadila Pharmaceuticals Limited, Inc.
	heat killed mycobacterium w immunomodulator	Cadi Mw	11/21/2002	Adjuvant to multi-drug therapy in the management of multibacillary leprosy	CPL, Inc.
	heat killed mycobacterium w immunomodulator	Cadi-Mw	7/31/2012	Treatment of non-small cell lung cancers that express desmocolin-3	Cadila Pharmaceuticals Limited
	heat killed whole cell mycobacterium obuense	n/a	9/23/2014	Treatment of pancreatic cancer	Immodulon Therapeutics Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	hematopoietic stem and progenitor cells expanded ex-vivo with a low molecular weight aryl hydrocarbon receptor (AHR) antagonist	n/a	8/27/2014	Hematopoietic support in patients with acute lymphoblastic leukemia (ALL)	Novartis Pharmaceuticals Corporation
	heparan sulfate mimetic	n/a	5/29/2014	Treatment of pancreatic cancer	Momenta Pharmaceuticals, Inc.
	heparin activated recombinant human fibroblast growth factor 1 (FGF1)in combination with a surgically implanted biodegradable device	n/a	10/24/2011	Treatment of patients with a confirmed traumatic complete spinal cord injury where no motor or sensory function is preserved below the injury(Scale A)	BioArctic Neuroscience AB
	hepatitis B immune globulin (human)	Hepagam	3/24/2008	Prevention of hepatitis B recurrence following orthotopic liver transplant	Cangene Corporation
	hepatitis B virus neutralizing human monoclonal antibody	Hepabig Gene	5/6/2013	Prevention of hepatitis B recurrence following liver transplantation	Green Cross Corp.
	hepcortespernisi mut-L	n/a	12/17/2014	Treatment of hepatocellular carcinoma	Immunitor, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	herpes simplex type 1 virus containing cellular B-myb gene as tumor-specific promoter	n/a	12/23/2014	Treatment of pancreatic cancer	Karcinolys S.A.S.
	heterologous human adult liver derived progenitor cells (HHALPC)	n/a	1/13/2012	Treatment of urea cycle disorders	Promethera Biosciences
	heterologous human liver derived progenitor cells	n/a	3/9/2012	Treatment of Crigler-Najjar syndrome	Promethera Biosciences
	hexasodium phytate	n/a	12/2/2012	Treatment of calciphylaxis	Laboratoris Sanifit, S.L.
	homoharringtonine	n/a	2/8/2002	Treatment for chronic myelogenous leukemia	American BioScience, Inc.
	human Hepatocarcinoma-Intestine-pancreas/pancreatitis associated protein	n/a	5/11/2011	Treatment of acute liver failure	Alfact Innovation SAS
	human IgG1k monoclonal antibody	n/a	10/16/2013	Treatment of systemic sclerosis	MedImmune
	human MHC non-restricted cytotoxic T-cell line	n/a	7/6/2012	Treatment of ovarian cancer	Galileo Research srl
	human allogeneic bone marrow derived osteoblastic cells	n/a	11/9/2015	Treatment of osteogenesis imperfecta.	Bone Therapeutics SA

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	human allogeneic bone marrow derived osteoblastic cells	Allob	1/10/2014	Treatment of osteonecrosis	Bone Therapeutics SA
	human anti-CD4 monoclonal antibody	Humax-Cd4	8/13/2004	Treatment of mycosis fungoides	Emergent Product Development Seattle, LLC
	human anti-cellular adhesion molecule-1 monoclonal antibody	n/a	7/29/2008	Treatment of multiple myeloma	BioInvent International AB
	human coagulation factor VIII	Octanate	12/3/2012	Immune tolerance induction in hemophilia A patients with inhibitors	OCTAPHARMA USA, Inc.
	human coagulation factor XI	Hemoleven	11/8/2007	Treatment of severe congenital Factor XI deficiency.	Laboratoire francais du Fractionnement et des Biot
	human cystic fibrosis transmembrane conductance regulator (hCFTR) messenger RNA (mRNA) complexed with branched polyethyleneimine (bPEI)	n/a	11/3/2015	Treatment of patients with cystic fibrosis.	Shire
	human fibrinogen concentrate, pasteurized	Riastap	3/13/2008	Treatment of fibrinogen deficient patients.	CSL Behring, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	human fully IgG1 antibody specific for CD33	n/a	6/19/2014	Treatment of acute myeloid leukemia	Boehringer Ingelheim Pharmaceuticals, Inc.
	human gammaglobulin	Oralgam	9/16/2002	Treatment of gastrointestinal disturbances (to include constipation, diarrhea, and abdominal pain) associated with regression-onset autism in pediatric patients.	Latona Life Sciences, Inc.
	human gammaglobulin	Oralgam	5/25/2001	Treatment for juvenile rheumatoid arthritis	Latona Life Sciences, Inc.
	human gammaglobulin	Oralgam	11/14/2003	Treatment of idiopathic inflammatory myopathies	Latona Life Sciences, Inc.
	human glial restricted progenitor cells and their progeny	Q-Cells	9/11/2013	Treatment of amyotrophic lateral sclerosis	Q Therapeutics, Inc.
	human haptoglobin	n/a	11/19/2013	Treatment of sickle cell disease	BioProducts Laboratory Limited
	human heterologous liver cells	n/a	2/14/2011	Treatment of urea cycle disorders	Cytonet GmbH & Co. KG
	human insulin (rDNA)	n/a	4/6/2015	Treatment of short bowel syndrome (SBS).	Nutrinia, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	human insulin beta chain peptide with incomplete Freund's adjuvant vaccine	n/a	2/11/2013	Treatment of Type 1 diabetes patients with residual beta cell function	Orban Biotech, LLC
	human interleukin-3 genetically conjugated to diphtheria toxin protein	n/a	2/18/2011	Treatment of acute myeloid leukemia.	Stemline Therapeutics, Inc.
	human interleukin-3 genetically conjugated to diphtheria toxin protein	n/a	6/6/2013	Treatment of blastic plasmacytoid dendritic cell neoplasm	Stemline Therapeutics, Inc.
	human laminin-111	n/a	9/23/2011	Treatment of merosin (laminin-alpha2) deficient congenital muscular dystrophy type 1A.	Prothelia, Inc.
	human leukocyte antigen-A2 restricted peptides	n/a	1/26/2015	Treatment of glioma.	Stemline Therapeutics, Inc.
	human leukocyte-derived cytokine mixture	n/a	7/7/2005	Neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	IRX Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	human monoclonal anti-PA antibody	n/a	10/21/2010	For post-exposure prophylaxis and treatment of inhalation anthrax.	Emergent Product Development Gaithersburg, Inc.
	human monoclonal antibody against human interleukin 13 (IL-13)	n/a	10/15/2013	Treatment of eosinophilic esophagitis	Novartis Pharmaceuticals Corporation
	human monoclonal antibody directed against active plasma kallikrein	n/a	11/26/2013	Treatment of hereditary angioedema (HAE)	Dyax Corporation
	human monoclonal antibody directed against serotype O1 Pseudomonas aeruginosa	n/a	1/6/2010	Treatment of pneumonia caused by serotype O1 positive Pseudomonas aeruginosa	Kenta Biotech Limited
	human plasminogen	n/a	6/7/2010	Treatment of ligneous conjunctivitis	Kedrion, S.p.A.
	human platelet antigen-1a immunoglobulin (anti-HPA-1a)	Tromplate	6/27/2013	Prevention of fetal and neonatal alloimmune thrombocytopenia	Prophylix Pharma AS

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

<b>Row Num</b>	<b>Generic Name</b>	<b>Trade Name</b>	<b>Designation Date</b>	<b>Designation</b>	<b>Contact Company</b>
	human recombinant DNA-derived, IgG1 kappa monoclonal antibody to connective growth factor	n/a	7/6/2012	Treatment of idiopathic pulmonary fibrosis	FibroGen, Inc.
	human recombinant mesencephalic, astrocyte derived neurotrophic factor	n/a	12/22/2014	Treatment of retinitis pigmentosa	Amarantus BioScience Holdings, Inc.
	human retinal progenitor cells	n/a	7/23/2012	Treatment of retinitis pigmentosa	jCyte, Inc.
	human soluble receptor-Fc fusion protein that targets human activin A	n/a	10/14/2015	Treatment of ovarian cancer.	Atara Biotherapeutics
	human spinal cord derived neural stem cells	n/a	2/4/2011	Treatment of amyotrophic lateral sclerosis	Neuralstem, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	human trivalent, bi-specific monoclonal antibody that binds and co-inhibits two growth factor receptors; insulin-like growth factor receptor 1 (IGF-1R) and v-erb-b2 avian erythroblastic leukemia viral oncogene homolog 3 (ErbB3)	n/a	10/22/2014	Treatment of pancreatic cancer	Merrimack Pharmaceuticals, Inc.
	human tumor necrosis factor coupled to the C terminus of CNGRCG peptide	n/a	10/1/2009	Treatment of liver cancer	Molecular Medicine S.p.A. (Molmed)
	human umbilical cord blood-derived mesenchymal stem cells	Pneumostem	11/26/2013	Prevention of bronchopulmonary dysplasia	MEDIPOST America, Inc.
	humanized Fc engineered monoclonal antibody against CD19	n/a	12/1/2014	Treatment of diffuse large B-cell lymphoma.	MorphoSys AG
	humanized Fc engineered monoclonal antibody against CD19	n/a	4/29/2014	Treatment of patients with chronic lymphocytic leukemia/small lymphocytic lymphoma	MorphoSys AG

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	humanized IgG1 anti-serum amyloid A monoclonal antibody	n/a	2/17/2012	Treatment of AA amyloidosis and AL amyloidosis	Prothena Therapeutics Limited
	humanized IgG1 monoclonal anti-CD20 antibody	n/a	5/26/2011	Treatment of follicular lymphoma	MENTRIK Biotech, LLC
	humanized IgG2 antibody	n/a	7/22/2008	Treatment of vaso-occlusive crisis in patients with sickle cell disease.	Selexys Pharmaceuticals Corp.
	humanized IgG4 monoclonal antibody	n/a	12/12/2011	Prevention of ischemia/reperfusion injury associated with solid organ transplantation	Opsona Therapeutics
	humanized anti-IL-6R receptor neutralizing IgG2 monoclonal antibody	n/a	6/30/2014	Treatment of neuromyelitis optica and neuromyelitis optica spectrum disorder	Chugai Pharma USA, LLC
	humanized immunoglobulin monoclonal antibody against CD38	n/a	5/22/2014	Treatment of multiple myeloma.	Sanofi U.S. Services, Inc.
	humanized monoclonal antibodies hu1B7 and hu11E6	n/a	9/11/2014	Treatment of Bordetella pertussis	Synthetic Biologics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	humanized monoclonal antibody against human integrin alphaVbeta6	n/a	8/5/2010	Treatment of idiopathic pulmonary fibrosis	Biogen Idec, Inc.
	humanized monoclonal antibody of the IgG4 kappa isotype targeting CD47	n/a	8/20/2015	Treatment of acute myeloid (myelogenous) leukemia.	Stanford University
	humanized monoclonal antibody that targets the alpha-subunit of the human IL-3 receptor also known as the CD123 antigen; (JNJ-56022473)	n/a	5/5/2015	Treatment of acute myeloid leukemia	Janssen Research & Development, LLC
	humanized monoclonal antibody to tissue factor	n/a	4/26/2011	Treatment of pancreatic cancer.	Morphotek, Inc.
	humanized monoclonal antibody against human integrin alphaVbeta6	n/a	6/18/2008	Treatment of fibrosis-associated chronic allograft nephropathy in kidney transplant patients.	Stromedix, Inc.
	humanized single chain monoclonal antibody(scFV: IgG1 hinge: IgG1 CH2 and CH3 domains)	n/a	11/17/2011	Treatment of chronic lymphocytic leukemia	Emergent Product Development Seattle, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	humanized, afucosylated IgG1 kappa monoclonal antibody	n/a	12/3/2009	Treatment of scleroderma	MedImmune
	hyaluronic acid	n/a	3/19/2002	Treatment of emphysema in patients due to alpha-1 antitrypsin deficiency	CoTherix
	hydralazine - magnesium valproate	Transkrip(R)	3/17/2011	Treatment of cutaneous T-cell lymphoma.	Neolpharma S.A.DE C.V.
	hydralazine - magnesium valproate	Transkrip(R)	3/17/2011	Treatment of myelodysplastic syndrome	Neolpharma S.A.DE C.V.
	hydrocinamate-[Orn-Pro-dCha-Trp-Arg](CH3COO)	n/a	3/9/2015	Treatment of amyotrophic lateral sclerosis.	Alsonex Pty Ltd
	hydrocortisone modified release capsules	Chronocort(R)	9/3/2015	Treatment of adrenal insufficiency.	Diurnal Ltd.
	hydrocortisone modified release capsules	Chronocort	3/18/2015	Treatment of congenital adrenal hyperpasia	Diurnal Limited
	hydrocortisone modified release tabs	Duocort	6/18/2008	Treatment of adrenal insufficiency	Shire ViroPharma, Inc.
	hydrocortisone oral granules	Infacort(R)	5/13/2015	Treatment of pediatric adrenal insufficiency (0 through 16 years of age).	Diurnal Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	hydroxycarbamide (hydroxyurea)	Siklos	7/24/2013	Treatment of sickle cell disease in patients under 18 years of age	addmedica Laboratories
	hydroxyprogesterone caproate	Makena	1/25/2007	Prevention of preterm birth in singleton pregnancies	KV Pharmaceutical Company
	hydroxyurea	Pedroxia	3/16/2015	Treatment of symptomatic sickle cell disease in pediatric patients, less than 17 years of age.	Ebelle D'Ebelle Pharmaceuticals LLC
	hydroxyurea	n/a	4/15/2005	Treatment of pediatric patients with sickle cell anemia.	UPM Pharmaceuticals, Inc.
	ibalizumab	n/a	10/20/2014	Treatment of HIV-1 infection in treatment experienced adult patients with documented multi-antiretroviral class resistance and evidence of HIV-1 replication despite ongoing antiretroviral therapy	TaiMed Biologics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ibritumomab tiuxetan	Zevalin	9/6/1994	Treatment of B- cell non- Hodgkin's lymphoma.	Spectrum Pharmaceuticals, Inc.
	ibrutinib	Imbruvica	10/15/2013	Treatment of Waldenstrom's macroglobulin emia	Pharmacyclics, LLC
	ibrutinib	Imbruvica	12/3/2012	Treatment of mantle cell lymphoma	Pharmacyclics, LLC
	ibrutinib	Imbruvica	2/5/2015	Treatment of splenic marginal zone lymphoma	Pharmacyclics, LLC
	ibrutinib	n/a	9/8/2014	Treatment of follicular lymphoma	Pharmacyclics, LLC
	ibrutinib	n/a	5/16/2013	Treatment of multiple myeloma	Pharmacyclics, LLC
	ibrutinib	Imbruvica	2/5/2015	Treatment of nodal marginal zone lymphoma	Pharmacyclics, LLC
	ibrutinib	n/a	10/23/2013	Treatment of diffuse large B- cell lymphoma	Pharmacyclics, LLC
	ibrutinib	n/a	5/30/2013	Treatment of small lymphocytic lymphoma	Pharmacyclics, LLC
	ibrutinib	Imbruvica	4/6/2012	Treatment of chronic lymphocytic leukemia (CLL)	Pharmacyclics, LLC
	ibudilast	n/a	6/1/2015	Treatment of Krabbe disease.	MediciNova, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	icatibant	Firazyr	11/25/2003	Treatment of angioedema	Shire Orphan Therapies
	idarucizumab	n/a	5/28/2015	To reverse the anticoagulant effect of dabigatran due to uncontrolled life-threatening bleeding requiring urgent intervention or a need to undergo an emergency surgery/urgent invasive procedure	Boehringer Ingelheim Pharmaceuticals, Inc.
	idebenone	n/a	3/25/2004	Treatment of Friedreich's ataxia	Santhera Pharmaceuticals LLC
	idebenone	n/a	5/22/2009	Treatment of mitochondrial myopathy, encephalopathy, lactic acidosis with stroke-like episodes syndrome (MELAS)	Santhera Pharmaceuticals Limited
	idebenone	n/a	10/31/2006	Treatment of Leber's hereditary optic neuropathy.	Santhera Pharmaceuticals Limited
	idebenone	n/a	2/16/2007	Treatment of Duchenne muscular dystrophy	Santhera Pharmaceuticals Limited

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	idelalisib	n/a	10/15/2013	Treatment of extranodal marginal zone lymphoma	Gilead Sciences, Inc.
	idelalisib	n/a	9/26/2013	Treatment of lymphoplasmacytic lymphoma with or without Walenstrom's macroglobulin emia	Gilead Sciences, Inc.
	idelalisib	Zydelig	9/26/2013	Treatment of follicular lymphoma	Gilead Sciences, Inc.
	idelalisib	Zydelig	10/15/2013	Treatment of chronic lymphocytic leukemia and small lymphocytic lymphoma	Gilead Sciences, Inc.
	idelalisib	n/a	8/25/2011	Treatment of chronic lymphocytic leukemia	Gilead Sciences, Inc.
	idelalisib	n/a	10/15/2013	Treatment of nodal marginal zone lymphoma.	Gilead Sciences, Inc.
	idelalisib	n/a	10/15/2013	Treatment of splenic marginal zone lymphoma	Gilead Sciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	idursulfase	Elaprase	11/28/2001	Long term enzyme replacement therapy for patients with mucopolysaccharidosis II (Hunter Syndrome)	Shire Human Genetic Therapies, Inc.
	idursulfase IT	n/a	9/3/2009	For treatment of neurocognitive symptoms associated with Hunter Syndrome	Shire Human Genetic Therapies
	idursulfase beta	n/a	2/11/2013	Treatment of Hunter Syndrome (mucopolysaccharidoses)	Green Cross Corp.
	iduvec	n/a	1/21/2011	Treatment of Mucopolysaccharidosis Type I	Zebraic Corporation
	iferanserin (S-MPEC)	n/a	12/13/2010	For use in pulmonary arterial hypertension.	Sam Amer & Company, Inc.
	ifosfamide	n/a	8/7/1985	Treatment of soft tissue and bone sarcomas	Bristol-Myers Squibb Company
	iloprost	n/a	4/6/2012	Treatment of pulmonary arterial hypertension	Algorithm Sciences, LLC
	imatinib	Gleevec	10/11/2005	Treatment of Philadelphia-positive acute lymphoblastic leukemia	Novartis Pharmaceuticals Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	imatinib mesylate	n/a	5/6/2014	Treatment of progressive multifocal leukencephalopathy	Inhibikase Therapeutics, Inc.
	imatinib mesylate	Gleevec	12/19/2005	Treatment of dermatofibrosarcoma protuberans	Novartis Pharmaceuticals Corporation
	imatinib mesylate	Gleevec	11/1/2001	Treatment of gastrointestinal stromal tumors	Novartis Pharmaceuticals Corp.
	imetelstat	n/a	6/11/2015	Treatment of myelofibrosis	Janssen Research & Development, LLC
	imexon	Amplimexon	8/12/2005	Treatment of ovarian cancer.	AmpliMed Corporation
	immune globulin (human)	n/a	3/2/2010	Treatment of Guillain-Barre syndrome	Octapharma USA, Inc.
	immune globulin infusion (human)	Gammagard Liquid	7/20/2006	Treatment of multifocal motor neuropathy	Baxter Healthcare Corporation
	immune globulin intravenous (human)	Gammaplex	4/29/2011	Treatment of idiopathic thrombocytopenic purpura (also known as primary immune thrombocytopenia)	Bio Products Laboratory

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	immunoglobulin G degrading enzyme of Streptococcus pyogenes	n/a	9/10/2015	Prevention of antibody mediated organ rejection in solid organ transplant patients.	Hansa Medical AB
	immunologically active synthetic peptides	n/a	6/1/2015	Treatment of Hemophilia A	Apitope International NV
	inBreath airway transplant system	n/a	9/4/2014	To restore the structure and/or function of the trachea subsequent to tracheal damage due to cancer, injury or infection	Harvard Apparatus Regenerative Technology, Inc.
	inecalcitol	n/a	8/3/2015	Treatment of acute myeloid leukemia.	Hybrigenics, S.A.
	infliximab	Remicade	11/14/1995	Treatment of Crohn's disease	Centocor, Inc.
	infliximab	Remicade	11/12/2003	Treatment of pediatric (0 to 16 years of age) ulcerative colitis	Janssen Biotech Inc.
	infliximab	Remicade	10/23/2002	Treatment of juvenile rheumatoid arthritis	Centocor, Inc.
	inhibitor of microRNA-451	n/a	2/4/2011	Treatment of polycythemia vera	miRagen Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	inolimomab	Leukotac	10/23/2002	Treatment of graft versus host disease	Jazz Pharmaceuticals
	inotuzumab ozogamicin	n/a	3/25/2013	Treatment of B-cell acute lymphoblastic leukemia	Pfizer, Inc.
	insecticidal toxin derived from Bacillus thuringiensis	n/a	9/5/2008	Treatment of soil transmitted helminth infection strongyloidiasis	University of California, San Diego
	interferon gamma	n/a	11/4/2011	Treatment of Friedreich's ataxia	Roberto Testi, MD
	interferon gamma-1b	Actimmune	10/1/2014	Treatment of Friedreich's Ataxia	Horizon Pharma Ireland Limited
	interferon-alpha secreting autologous micro-organ tissue converting into a biopump	Infradure Biopump	6/14/2012	Treatment of chronic hepatitis D	Medgenics, Inc.
	interleukin-1 receptor antagonist anakinra	Kineret	9/15/2015	Treatment of Still's disease including systemic juvenile idiopathic arthritis and adult-onset Still's disease.	Swedish Orphan Biovitrum AB
	intravenous carbamazepine	n/a	6/27/2013	Treatment of epilepsy patients who cannot take anything by mouth (NPO)	Lundbeck LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	intraventricular nimodipine	n/a	5/28/2015	Treatment of subarachnoid hemorrhage	Edge Therapeutics, Inc.
	iobenguane I 131	n/a	7/5/2007	Treatment of neuroendocrine tumors	Jubilant DraxImage, Inc.
	iobenguane sulfate I-123	Omaclear	10/21/2005	For the detection, localization, and staging of pheochromocytomas.	Brogan Pharmaceuticals, Inc.
	ipilimumab	Yervoy	6/3/2004	Treatment of high risk Stage II, Stage III, and Stage IV melanoma	Bristol-Myers Squibb Pharmaceutical Research Insti
	iron(III)-hexacyanoferrate (II)	Radiogardase	5/1/2003	Treatment of patients with known or suspected internal contamination with radioactive or non-radioactive cesium or thallium	Heyl Chemisch-Pharmzeutische Fabrik GMBH & Co, KG
	isavuconazonium sulfate	Cresemba	10/25/2013	Treatment of zygomycosis	Astellas Pharma Global Development Inc.
	isavuconazonium sulfate	n/a	10/20/2014	Treatment of invasive candidiasis/candidemia	Astellas Pharma Global Development, Inc.
	isavuconazonium sulfate	Cresemba	5/6/2013	Treatment of invasive aspergillosis	Astellas Pharma Global Development Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	isocitrate dehydrogenase 1 (IDH1)-mutant inhibitor	n/a	6/9/2015	Treatment of acute myeloid leukemia (AML)	Agios Pharmaceuticals, Inc.
	isocitrate dehydrogenase 2-mutant inhibitor	n/a	6/12/2014	Treatment of acute myelogenous leukemia	Celgene Corporation
	isofagomine tartrate	n/a	1/10/2006	Treatment of Gaucher disease	Amicus Therapeutics, Inc.
	isotretinoin	n/a	4/10/2014	Treatment of congenital ichthyosis	Patagonia Pharmaceuticals, LLC
	ivacaftor	Kalydeco	12/20/2006	Treatment of patients with cystic fibrosis	Vertex Pharmaceuticals, Inc.
	ixazomib citrate	n/a	2/18/2011	Treatment of multiple myeloma	Millennium Pharmaceuticals
	ketotifen	n/a	2/25/2015	Treatment of mastocytosis	Melbourne Laboratories LLC
	lactic acid bacteria (Lactobacilli, Bifidobacteria, and Streptococci)	n/a	1/15/2002	Treatment of active chronic pouchitis	VSL Pharmaceuticals, Inc.
	lactic acid bacteria (Lactobacilli, Bifidobacteria, and Streptococcus species)	n/a	1/15/2002	Prevention of disease relapse in patients with chronic pouchitis	VSL Pharmaceuticals, Inc.
	lactobacillus brevis CD2	n/a	10/4/2011	Treatment Behcet's disease	VSL Pharmaceuticals, Inc.
	laminin-111 (human)	n/a	7/21/2011	Treatment of Duchenne Muscular Dystrophy	Prothelia, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lanreotide acetate	n/a	9/8/2011	Treatment of symptoms associated with carcinoid syndrome	Ipsen Biopharmaceuticals, Inc.
	lanreotide acetate	Somatuline Depot	8/25/2011	Treatment of neuroendocrine tumors	Ipsen Biopharmaceuticals, Inc.
	lapatinib	Tykerb	5/29/2009	Treatment of ErbB2 positive gastric cancer	Novartis Pharmaceuticals Corp.
	lapatinib ditosylate hydrochloride	Tykerb	5/29/2009	Treatment of ErbB2 positive esophageal cancer	Novartis Pharmaceuticals Corp.
	laromustine	Onrigin	10/21/2004	Treatment of acute myelogenous leukemia	Vion Pharmaceuticals, Inc.
	laronidase	Aldurazyme	9/24/1997	Treatment of patients with mucopolysaccharidosis-I.	BioMarin Pharmaceutical, Inc.
	late stage human motor neuron progenitors	Motorgraft(TM)	11/25/2009	Treatment of spinal muscular atrophy	California Stem Cell, Inc.
	lebrikizumab	n/a	3/9/2015	Treatment of idiopathic pulmonary fibrosis	Genentech, Inc.
	lenalidomide	Revlimid	1/17/2007	Treatment of chronic lymphocytic leukemia	Celgene Corporation
	lenalidomide	Revlimid	1/29/2004	Treatment of myelodysplastic syndromes	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lenalidomide	Revlimid	4/27/2009	Treatment of mantle cell lymphoma	Celgene Corporation
	lenalidomide	Revlimid	4/29/2015	Treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue	Celgene Corporation
	lenalidomide	Revlimid	9/17/2013	Treatment of follicular lymphoma	Celgene Corporation
	lenalidomide	Revlimid	3/28/2011	Treatment of diffuse large B-cell lymphoma	Celgene Corporation
	lenalidomide	Revlimid	9/20/2001	Treatment of multiple myeloma	Celgene Corporation
	lenvatinib	n/a	3/27/2014	Treatment of hepatocellular carcinoma	Eisai, Inc.
	lenvatinib	n/a	3/26/2014	Treatment of stage IIB to Stage IV melanoma	Eisai, Inc.
	lenvatinib	Lenvima	12/27/2012	Treatment of follicular, medullary, anaplastic, and metastatic or locally advanced papillary thyroid cancer	Eisai, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lestaurtinib	n/a	9/3/2009	Treatment of Philadelphia-negative classic myeloproliferative disorders	Teva Branded Pharmaceutical Products R&D, Inc.
	letermovir	n/a	12/12/2011	Prevention of human cytomegalovirus viremia and disease in at risk populations	Merck Sharpe & Dhome Corporation
	leukocyte interleukin	Multikine	5/4/2007	Neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	CEL-SCI Corporation
	leukotriene A4 hydrolase inhibitor	n/a	1/26/2015	Treatment of cystic fibrosis	Celtaxsys, Inc.
	levetiracetam	Keppra	4/30/2010	Treatment of neonatal seizures	University of California
	levocarnitine	Carnitor	11/15/1989	1. Prevention of secondary carnitine deficiency in valproic acid toxicity 2. Treatment of secondary carnitine deficiency in valproic acid toxicity	Sigma-Tau Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	levoleucovorin	Fusilev	12/18/1990	For use in combination chemotherapy with the approved agent 5-fluorouracil in the palliative treatment of metastatic adenocarcinoma of the colon and rectum	Spectrum Pharmaceuticals, Inc.
	levomefolate calcium	n/a	2/10/2015	Treatment of megaloblastic anemia caused by folate deficiency	Cox Biosciences LLC
	levothyroxine sodium	n/a	4/26/2011	The preservation of organ function in brain-dead organ donors.	Fera Pharmaceuticals, LLC
	liposomal P-ethoxy growth factor receptor-bound protein 2 antisense oligonucleotide	n/a	4/27/2015	Treatment of acute myeloid leukemia	Bio-Path Holdings, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	liposomal amikacin	Arikace	8/25/2009	Treatment of bronchiectasis in patients with Pseudomonas aeruginosa or other susceptible microbial pathogens	Insmmed, Inc.
	liposomal amikacin	Arikace	3/25/2013	Treatment infections caused by non-tuberculous mycobacteria	Insmmed, Inc.
	liposomal amikacin	Arikace	3/9/2006	Treatment of bronchopulmonary Pseudomonas aeruginosa infections in cystic fibrosis patients	Insmmed, Inc.
	liposomal busulfan	Busulipo	6/24/2013	For use as a conditioning regimen for patients with malignancies undergoing autologous or allogenic hematopoietic stem cell transplantation	Pharmalink AB
	liposomal ciprofloxacin plus ciprofloxacin	n/a	6/1/2012	For the management of cystic fibrosis	Aradigm Corporation
	liposomal cyclosporine	n/a	5/11/2009	Treatment of bronchiolitis obliterans	PARI Pharma GmbH

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	liposomal cyclosporine for inhalation	n/a	6/24/2008	Prevention of bronchiolitis obliterans.	PARI Pharma GmbH
	liposomal encapsulated paclitaxel	n/a	1/21/2015	Treatment of ovarian cancer	Insys Therapeutics, Inc.
	liposomal gadodiamide	n/a	11/12/2008	Treatment of glioma.	MedGenesis Therapeutix, Inc.
	liposomal irinotecan	n/a	7/21/2011	Treatment of pancreatic cancer	Merrimack Pharmaceuticals, Inc.
	liposomal p-ethoxy growth receptor bound protein-2 antisense product	n/a	12/5/2003	Treatment of chronic myelogenous leukemia	Bio-Path, Inc.
	liposomal topotecan hydrochloride	n/a	10/30/2008	Treatment of gliomas	MedGenesis Therapeutix, Inc.
	liposomal $\alpha$ -galactosylceramide	Lip. Alpha Galactosylceramide	9/28/2012	Prevention of graft-versus-host disease	REGiMMUNE Corporation
	liposome encapsulated paclitaxel	n/a	12/3/2014	Treatment of gastric cancer	Insys Therapeutics, Inc.
	lisinopril oral solution	n/a	10/14/2015	Treatment of hypertension in pediatric patients 0 through 16 years of age.	Silvergate Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lisinopril oral solution	n/a	1/27/2015	Treatment of primary hypertension with complications and secondary hypertension in pediatric patients (ages 0 through 16 years of age)	BioRamo, LLC
	listeria monocytogenes	n/a	9/5/2013	Treatment of pancreatic cancer	Aduro BioTech, Inc.
	lisuride	n/a	1/17/2013	Treatment of pulmonary arterial hypertension	Sinoxa Pharma GmbH
	lithium citrate tetrahydrate (in reverse micelle formulation)	n/a	12/13/2010	Treatment of Huntington's disease.	Medesis Pharma
	live attenuated E. Coli expressing Beta catenin shRNA	n/a	12/20/2010	Treatment of Familial Adenomatous Polyposis	Marina Biotech
	live attenuated bioengineered Listeria monocytogenes immunotherapy	n/a	5/21/2014	Treatment of osteosarcoma.	Advaxis, Inc.
	live attenuated bioengineered Listeria monocytogenes immunotherapy	n/a	11/4/2013	Treatment of human papilloma virus-associated head and neck cancer	Advaxis, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lomitapide	Juxtapid	10/23/2007	Treatment of homozygous familial hypercholesterolemia	Aegerion Pharmaceuticals, Inc.
	lomitapide	n/a	3/3/2011	Treatment of Familial Chylomicronemia	Aegerion Pharmaceuticals, Inc.
	lonafarnib	n/a	4/18/2011	Treatment of Hutchinson-Gilford progeria syndrome	The Progeria Research Foundation, Inc.
	long acting recombinant Factor VIIa-CTP3	n/a	2/27/2014	Treatment and prophylaxis of bleeding episodes in patients with hemophilia A or B with inhibitors to factor VIII or factor IX	PROLOR Biotech, Ltd
	losartan	n/a	12/12/2011	Treatment of Marfan Syndrome	National Marfan Foundation
	low molecular weight dextran sulfate	Ibsolvmir(R)	7/6/2011	Treatment to mobilize progenitor cells prior to stem cell transplantation	TikoMed AB
	low molecular weight dextran sulfate	Ibsolvmir	10/20/2009	Prevention of graft rejection during pancreatic islet transplantation	TikoMed AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	lucerastat	n/a	10/29/2015	Treatment of Fabry Disease.	Actelion Pharmaceuticals Ltd
	lumacaftor/ivacaftor	Orkambi	6/30/2014	Treatment of cystic fibrosis	Vertex Pharmaceuticals Inc.
	lurbinectedin	n/a	8/20/2012	Treatment of ovarian cancer	PharmaMar USA, Inc.
	lutetium (177Lu)-edotreotide	n/a	5/21/2015	Treatment of gastro-entero-pancreatic neuroendocrine tumors	ITG Isotope Technologies Garching GmbH
	lyso-thermosensitive liposomal doxorubicin	Thermodox (R)	3/17/2009	Treatment of hepatocellular carcinoma	Celsion Corporation
	lysosomal enzyme N-acetylgalactosamine-6-sulfate sulfatase	n/a	9/10/2008	Treatment of mucopolysaccharidosis Type IVA (Morquio Syndrome)	Vivendy Therapeutics LTD
	mPEG-r-crisantaspase	Asparec(Tm)	2/1/2010	Treatment of acute lymphoblastic leukemia.	Jazz Pharmaceuticals, Inc.
	mTOR kinase inhibitor (CC-223)	n/a	10/16/2013	Treatment of hepatocellular carcinoma	Celgene Corporation
	macitentan	Opsumit	9/3/2009	Treatment of pulmonary arterial hypertension	Actelion Pharmaceuticals Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	manganese (II) chloride tetrahydrate (with L-alanine and vitamin D3 as promoters of absorption)	n/a	9/26/2013	Use as a targeted contrast agent for diagnostic MRI for the detection and localization of focal liver lesions in patients where use of gadolinium based contrast agents may be medically inadvisable or where gadolinium based contrast agents cannot be administered	CMC Contrast AB
	mannopentaose phosphate sulfate	n/a	4/27/2004	Treatment of high-risk Stage II, Stage III, and Stage IV melanoma	Medigen Biotechnology Corp.
	maribavir	n/a	6/7/2011	Treatment of clinically significant cytomegalovirus viremia and disease in at-risk patients.	Shire ViroPharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	maribavir	n/a	2/1/2007	Prevention of cytomegalovirus viremia and disease in the populations at risk.	Shire ViroPharma, Inc.
	marizomib	n/a	9/21/2015	Treatment of malignant glioma.	Triphase Research & Development I Corp.
	masitinib	n/a	4/20/2005	Treatment of malignant gastrointestinal stromal tumors	AB Science
	masitinib	n/a	9/14/2005	Treatment of mastocytosis	AB Science
	masitinib	n/a	7/21/2009	Treatment of patients with pancreatic cancer	AB Science
	masitinib mesylate	n/a	9/14/2015	Treatment of gastric cancer including cancer of the gastroesophageal junction	AB Science
	masitinib mesylate	n/a	3/18/2015	Treatment of amyotrophic lateral sclerosis.	AB Science
	maytansinoid conjugated humanized monoclonal antibody against FOLRI	n/a	7/14/2014	Treatment of ovarian cancer	ImmunoGen, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mebendazole	Vermox 500 Mg Chewable Tablets	9/3/2014	Treatment of single or mixed gastrointestinal infestations by Trichuris trichiura (whipworm), Ascaris lumbricoides (large roundworm), and Ancylostoma duodenale and Nectar americanus (hookworm).	Janssen Pharmaceutical Research & Development, LLC
	mecasermin	Iplex	12/3/2007	Treatment of myotonic dystrophy	Insmmed, Inc.
	mecasermin rinfabate	n/a	9/20/2012	Prevention of retinopathy of prematurity in premature infants born at risk for the disease	Premacure AB
	mecasermin rinfabate	Iplex	7/23/2012	Treatment of amyotrophic lateral sclerosis	PCUT BioPartners, Inc.
	mecasermin rinfabate	Iplex	5/17/2002	Treatment of growth hormone insensitivity syndrome (GHIS)	Insmmed, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mecasermin, recombinant human insulin- like growth factor- 1	Increlex	9/21/2015	Treatment of Rett Syndrome.	Keck Graduate Institute of Applied Life Sciences
	meclorethamine	Valchlor	8/17/2004	Treatment of mycosis fungoides	Actelion Pharmaceuticals Ltd.
	mefloquine HCL	Lariam	4/13/1988	For use in the treatment of acute malaria due to Plasmodium falciparum and Plasmodium vivax, and for the prophylaxis of Plasmodium falciparum malaria which is resistant to other available drugs	Hoffmann-La Roche, Inc.
	melarsoprol- hydroxypropylbet adex	n/a	9/13/2013	Treatment of human African trypanosomias is (sleeping sickness)	Peter Kennedy, CBE, MD, PhD, DSc, FRCP FMedSci,
	melatonin	n/a	4/12/2013	Treatment of neonatal hypoxic ischemic encephalopath y	Scharper S.p.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	meloxicam	Mobic	11/22/2002	Treatment of juvenile rheumatoid arthritis	Boehringer Ingelheim Pharmaceuticals, Inc.
	melphalan	n/a	11/19/2012	Treatment of retinoblastoma	Icon Bioscience, Inc.
	melphalan	n/a	7/14/2015	Treatment of cholangiocarcinoma.	Delcath Systems, Inc.
	melphalan	n/a	11/19/2012	Treatment of Stage IIB through IV melanoma	OncoTx, LLC
	melphalan	n/a	11/24/2008	High dose conditioning treatment prior to hematopoietic progenitor (stem) cell transplantation	Spectrum Pharmaceuticals, Inc.
	melphalan flufenamide hydrochloride	n/a	3/16/2015	Treatment of plasma cell myeloma, also referred to as multiple myeloma	Oncopeptides AB
	melphalan hydrochloride	n/a	11/12/2008	Treatment of patients with ocular (uveal) melanoma.	Delcath Systems, Inc.
	melphalan hydrochloride	n/a	5/22/2009	Treatment of neuroendocrine tumors	Delcath Systems, Inc.
	melphalan hydrochloride	n/a	9/26/2013	Treatment of patients with hepatocellular carcinoma	Delcath Systems, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	melphalan hydrochloride	n/a	11/12/2008	Treatment of patients with cutaneous melanoma.	Delcath Systems, Inc.
	menadione sodium bisulfite	n/a	5/14/2014	Treatment of autosomal dominant polycystic liver disease	IC-MedTech Corporation
	menaquinone	Menaquinoneg old	9/4/2012	Treatment of calciphylaxis	Nu Science Trading, LLC
	menatetrenone	Mk4(R)	11/2/2010	Treatment of hepatocellular carcinoma (HCC).	NBI Pharmaceuticals, Inc.
	menatetrenone	Mk4(R)	11/2/2010	Treatment of acute promyelocytic leukemia.	NBI Pharmaceuticals, Inc.
	menatetrenone	Mk4(R)	2/4/2011	Treatment of myelodysplastic syndrome.	NBI Pharmaceuticals, Inc.
	menatetrenone	Mk4	3/17/2011	Treatment of acute myeloid leukemia	NBI Pharmaceuticals, Inc.
	menatetrenone and Vitamin D3	n/a	8/22/2011	Treatment of myelodysplastic syndrome	NBI Pharmaceuticals, Inc.
	mepivacaine	n/a	10/18/2006	Treatment of painful HIV-associated neuropathy	Relmada Therapeutics, Inc.
	mepivacaine	n/a	1/8/2007	Treatment of postherpetic neuralgia	Relmada Therapeutics, Inc.
	mepolizumab	n/a	7/14/2011	Treatment of Churg-Strauss Syndrome.	GlaxoSmithKline LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mepolizumab	n/a	5/28/2004	For first-line treatment in patients with hypereosinophilic syndrome	GlaxoSmithKline LLC
	mercaptopurine oral solution	Purixan	8/20/2012	Treatment of acute lymphoblastic leukemia in pediatric patients	Nova Laboratories Limited
	mesalamine	Canasa	1/19/2010	Treatment of pediatric ulcerative colitis	Aptalis Pharma US, Inc.
	mesalamine and N-acetylcysteine	n/a	9/10/2009	Treatment of pediatric patients with ulcerative colitis (inclusive through age 16 years)	Altheus Therapeutics, Inc.
	mesalamine; 5-aminosalicylic acid	Lialda	2/27/2008	Treatment of ulcerative colitis in pediatric patients (revised indication 10/21/2010).	Shire
	mesencephalic, astrocyte-derived neurotrophic factor	n/a	9/10/2015	Treatment of retinal artery occlusion.	Amarantus BioScience Holdings, Inc.
	mesenchymal stromal cells secreting neurotrophic factors	Nurown	2/4/2011	Treatment of Amyotrophic Lateral Sclerosis	BrainStorm Cell Therapeutics Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	met-enkephalin; Met-N	n/a	2/18/2011	Treatment of pancreatic cancer.	NBI Pharmaceuticals, Inc.
	metadoxine	n/a	12/16/2013	Treatment of Fragile X Syndrome	Alcobra, Inc.
	metformin	n/a	5/29/2014	Treatment of pediatric polycystic ovary syndrome	EffRx Pharmaceuticals SA
	methotrexate	n/a	8/20/2009	Treatment of acute lymphoblastic leukemia	Orbona Pharma Ltd
	methotrexate oral liquid formulation	n/a	3/18/2015	Treatment of acute lymphoblastic leukemia in pediatric patients aged 0 through 16 years.	Chesapeake Therapeutics
	methotrexate oral solution	n/a	5/28/2015	Treatment of acute lymphoblastic leukemia in pediatric patients (0 through 16 years of age)	Silvergate Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	methotrexate oral solution	n/a	8/27/2015	Treatment of oligoarticular juvenile idiopathic arthritis (persistent oligoarthritis, psoriatic juvenile idiopathic arthritis, enthesitis-related arthritis, or undifferentiated arthritis) and polyarticular juvenile idiopathic arthritis in children 0 through 16 years of age	Silvergate Pharmaceuticals, Inc.
	methoxsalen	Uvadex	5/12/1994	For the prevention of acute rejection of cardiac allografts	Therakos, Inc.
	methylene blue	n/a	7/24/2012	Treatment of methemoglobinemia	Fera Pharmaceuticals, LLC
	methylene blue injection	n/a	8/11/2012	Treatment of congenital and acquired methemoglobinemia	Luitpold Pharmaceuticals, Inc.
	methylene blue 0.5%	n/a	12/18/2012	Treatment of hereditary and acquired methemoglobinemia	Provepharm SAS

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	methylparaben suberohydroxamic acid phenyl ester	n/a	4/15/2013	Treatment of cutaneous T- cell lymphoma	TetraLogic Pharmaceuticals
	metreleptin	n/a	8/22/2001	Treatment of leptin deficiency secondary to generalized lipodystrophy and partial familial lipodystrophy	Aegerion Pharmaceuticals, Inc.
	metreleptin	Myalept	8/22/2001	Treatment of metabolic disorders secondary to lipodystrophy	Aegerion Pharmaceuticals, Inc.
	metronidazole	n/a	9/17/2008	Treatment of pouchitis	Avivia Project BV
	metronidazole	n/a	4/26/2011	Treatment of pouchitis.	S.L.A. Pharma Limited (UK)
	metyrapone	n/a	9/25/2012	Treatment of Cushing's syndrome	Laboratoire HRA Pharma
	metyrosine	Demser	7/25/2008	Treatment of velocardiofacia l syndrome associated psychosis.	Cerberus Princeton, LLC
	mexiletine	n/a	9/2/2010	Treatment of nondystrophic myotonia	University of Rochester Medical Center
	mibefradil	n/a	8/25/2009	Treatment of glioblastoma multiforme.	Cavion, LLC
	mibefradil	n/a	10/16/2008	Treatment of pancreatic cancer.	Cavion, LLC
	mibefradil	n/a	6/15/2007	Treatment of ovarian cancer	Cavion, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	miconazole	n/a	5/22/2014	Treatment of fungal otitis externa (otomycosis)	Hill Dermaceuticals, Inc.
	midazolam	n/a	10/20/2009	Rescue treatment of seizures in patients who require control of intermittent bouts of increased seizure activity (e.g. acute repetitive seizures, seizure clusters)	Upsher-Smith Laboratories, Inc.
	midazolam	n/a	7/24/2012	Treatment of nerve agent-induced seizures	Meridian Medical Technologies, Inc.
	midazolam	n/a	5/8/2006	Treatment of bouts of increased seizure activity in selected refractory patients with epilepsy who are on stable regimens of anti-epileptic drugs and who require intermittent use of midazolam	UCB, Inc

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mifamuritide	Junovan	6/5/2001	Treatment of osteosarcoma	Millennium Pharmaceuticals, Inc.
	mifepristone	Korlym	7/5/2007	Treatment of the clinical manifestations of endogenous Cushing's syndrome	Corcept Therapeutics, Inc.
	migalastat hydrochloride	n/a	2/25/2004	Treatment of Fabry Disease	Amicus Therapeutics, Inc.
	miglustat	Zavesca (R)	11/12/2008	Treatment of the neurological manifestations of Niemann-Pick disease, type C.	Actelion Pharmaceuticals Ltd
	miglustat	Zavesca	5/29/1998	Treatment of Gaucher disease.	Actelion Pharmaceuticals Ltd
	milatuzumab	n/a	6/24/2008	Treatment of chronic lymphocytic leukemia.	Immunomedics, Inc.
	milatuzumab	n/a	3/10/2008	Treatment of multiple myeloma	Immunomedics, Inc.
	milciclib maleate	n/a	9/20/2012	Treatment of thymic epithelial tumors.	Tiziana Life Sciences PLC
	miltefosine	Impavido	10/10/2006	Treatment of leishmaniasis.	Knight Therapeutics (USA)

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	miltefosine	Miltex/Impavid o	3/18/2009	Topical treatment of cutaneous lymphoma encompassing cutaneous manifestations of T-cell lymphoma and B-cell lymphoma	ExperGen Drug Development GmbH
	mipomersen	Kynamro	5/23/2006	Treatment of homozygous familial hypercholesterolemia	Genzyme Corporation
	misoprostol	Gymiso	1/10/2005	Treatment of intrauterine fetal death not accompanied by complete expulsion of the products of conception in the second and third trimesters of pregnancy.	Gynuity Health Projects, LLC
	mitomycin	n/a	6/1/2011	Prevention of corneal sub-epithelial haze formation following surface ablation laser keratectomy	Mobius Therapeutics, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mitomycin	n/a	1/13/2011	Prevention of recurrence of pterygium after its surgical excision.	Mobius Therapeutics, LLC
	mitomycin	Mitogel	9/8/2014	Treatment of upper tract urothelial cell cancers (transitional cell carcinoma of the renal pelvis and ureter)	TheraCoat, Ltd.
	mitomycin-C	Mitosol	1/8/2008	Treatment of refractory glaucoma as an adjunct to ab externo glaucoma surgery.	Mobius Therapeutics, LLC
	mocetinostat	n/a	8/7/2014	Treatment of diffuse large B-cell lymphoma	Mirati Therapeutics, Inc.
	modified a-cobratoxin	n/a	9/1/2015	Treatment of pediatric multiple sclerosis (0 through 16 years of age).	Nutra Pharma Corporation
	modified human papillomavirus capsid protein conjugated to a near infrared dye	n/a	4/8/2015	Treatment of uveal melanoma	Aura Biosciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	modified recombinant human C-type natriuretic peptide (CNP)	n/a	1/17/2013	Treatment of achondroplasia	BioMarin Pharmaceutical, Inc.
	modified recombinant human Factor VIIa (rFVIIa) molecule	n/a	5/30/2013	Treatment of bleeding episodes in hemophilia A or B subjects with inhibitors	Bayer HealthCare Pharmaceuticals, Inc.
	modified recombinant mitochondrial transcription factor A (TFAM) containing the mitochondrial transduction domain	n/a	8/20/2012	Treatment of inherited mitochondrial respiratory chain disease	Gencia Corporation
	mogamulizumab	n/a	7/14/2011	Treatment of adult T-cell leukemia/lymphoma (ATLL).	Kyowa Hakko Kirin Pharma, Inc.
	mogamulizumab	n/a	11/2/2010	Treatment of peripheral T-cell lymphoma.	Kyowa Hakko Kirin Pharma, Inc.
	mogamulizumab	n/a	11/2/2010	Treatment of patients with cutaneous T-cell lymphoma.	Kyowa Hakko Kirin Pharma, Inc.
	monarsen	n/a	11/14/2003	Treatment of myasthenia gravis	Bioline Rx, Ltd.
	mongersen	n/a	7/20/2015	Treatment of pediatric Crohn's disease	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	monoclonal antibody	n/a	1/21/2011	Prevention of congenital cytomegalovirus (CMV)infection following primary CMV infection in pregnant women.	Theraclone Sciences
	monoclonal antibody 11-1F4	n/a	12/7/2009	For use as a radioimaging agent in amyloidosis	Alan Solomon, M.D.
	monoclonal antibody 11-1F4	n/a	12/11/2009	For use as a therapeutic agent for patients AL amyloidosis.	Alan Solomon, M.D.
	monoclonal antibody 3F8	n/a	10/16/2008	Treatment of neuroblastoma	United Therapeutics Corporation
	monoclonal antibody Hu3F8	n/a	1/10/2014	Treatment of osteosarcoma	Memorial Sloan-Kettering Cancer Center
	monoclonal antibody consisting of three mouse/human chimeric IgG1 monoclonal antibodies (c2G4, c4G7, and c13C6) that target Ebola virus	n/a	8/25/2014	Treatment of Ebola virus infection	LeafBio, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	monoclonal antibody directed at hepatitis C virus E2 glycoprotein	n/a	11/4/2013	Prevention of Hepatitis C recurrence in patients receiving liver transplantatio n	MassBiologics-University of MA Medical School
	monoclonal antibody targeting eotaxin-2	n/a	1/6/2015	Treatment of systemic sclerosis	ChemomAb, Ltd.
	monoclonal antibody targeting eotaxin-2	n/a	10/29/2015	Treatment of idiopathic pulmonary fibrosis.	ChemomAb, Ltd.
	monomethyl auristatin E conjugated to a monoclonal antibody which targets guanylyl cyclase C	n/a	7/29/2014	Treatment of pancreatic cancer.	Millennium Pharmaceuticals, Inc.
	motexafin gadolinium	Xcytrin	1/27/2003	For use in conjunction with whole brain radiation for the treatment of brain metastases arising from solid tumors	Pharmacyclics, Inc.
	mouse-human chimeric antibody of the IgG1 isotype, directed against human CD37	n/a	3/24/2015	Treatment of chronic lymphocytic leukemia (CLL).	Boehringer Ingelheim Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mouse-human chimeric monoclonal anti-GD2 IgG1 antibody	n/a	9/20/2012	Treatment of neuroblastoma	APEIRON Biologics AG
	moxetumomab pasudotox	n/a	11/15/2007	Treatment of CD22-positive chronic lymphocytic leukemia	MedImmune, LLC
	moxetumomab pasudotox	n/a	11/15/2007	Treatment of hairy cell leukemia	MedImmune, LLC
	moxetumomab pasudotox	n/a	6/28/2013	Treatment of acute lymphoblastic leukemia	MedImmune, LLC
	moxidectin	n/a	9/29/2010	Treatment of onchocerciasis volvulus in children and adults.	Medicines Development Limited
	multi-vitamin infusion without vitamin K	M.V.I.-12	3/8/2004	Prevention of vitamin deficiency and thromboembolic complications in people receiving home parenteral nutrition and warfarin-type anticoagulant therapy	Mayne Pharma (USA) Inc.
	murine monoclonal antibody against CD26	Begedina	2/18/2011	Treatment of graft versus host disease	Adienne S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	mycobacterium vaccae	n/a	8/20/2010	Treatment of tuberculosis	Immodulon Therapeutics Ltd
	myo-inositol	n/a	4/7/2005	Prevention of retinopathy of prematurity in preterm infants at risk for developing retinopathy of prematurity	Abbott Nutrition
	nabumetone	n/a	5/5/2008	Treatment of pediatric juvenile rheumatoid arthritis	Cook Pharma
	naloxone	n/a	11/23/2010	Topical treatment of pruritus associated with mycosis fungoides	Elorac, Inc.
	naltrexone	n/a	1/19/2010	Treatment of Crohn's disease in pediatric patients	TNI BioTech, Inc.
	naltrexone	n/a	2/23/2015	Treatment of postherpetic neuralgia	Allodynic Therapeutics LLC
	naltrexone	n/a	1/13/2015	Treatment of autoimmune hepatitis	TaiwanJ Pharmaceuticals Co., Ltd.
	naltrexone/clonidine combination	n/a	4/14/2014	Treatment of postherpetic neuralgia	Allodynic Therapeutics LLC
	nano-diamino-tetraiodothyroacetic acid	n/a	7/9/2015	Treatment of glioblastoma multiforme (GBM)	NanoPharmaceuticals, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	nano-diamino-tetraiodothyroacetic acid	n/a	10/28/2015	Treatment of pancreatic cancer	NanoPharmaceuticals, LLC
	nanoparticulate paclitaxel	Nanotax(R)	10/6/2015	Treatment of ovarian cancer.	NanOlogy, LLC
	naproxcinod	n/a	3/16/2015	Treatment of Duchenne muscular dystrophy.	Nicox SA
	natural human lymphoblastoid interferon-alpha	n/a	11/18/2002	Treatment of polycythemia vera	Amarillo Biosciences, Inc.
	necitumumab	n/a	11/20/2015	Treatment of squamous non-small cell lung cancer	Eli Lilly and Company
	nelarabine	Arranon	8/10/2004	Treatment of acute lymphoblastic leukemia and lymphoblastic lymphoma	Novartis Pharmaceuticals Corp.
	nelarabine	n/a	9/2/1999	Treatment of chronic lymphocytic leukemia.	Novartis Pharmaceuticals Corp.
	neostigmine	n/a	3/18/2013	Treatment of acute colonic pseudo-obstruction	Luitpold Pharmaceuticals, Inc.
	neostigmine methylsulfate	n/a	2/17/2012	Treatment of Myasthenia Gravis	Luitpold Pharmaceuticals, Inc.
	neridronate	n/a	3/25/2013	Treatment of complex regional pain syndrome (CRPS-1, CRPS-II, CRPS-NOS)	Grunenthal USA, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	nevirapine	n/a	11/25/2009	Prevention of HIV infection in pediatric patients under the age of 16 years	Auritec Pharmaceuticals
	nifurtimox	n/a	12/31/2013	Treatment of Chagas disease	MetronomX Therapeutics, LLC
	nifurtimox	Lampit	8/5/2010	Treatment of Chagas disease (American Trypanosomiasis) caused by T. cruzi	Bayer HealthCare Pharmaceuticals, Inc.
	nilotinib	Tasigna	4/27/2006	Treatment of chronic myelogenous leukemia	Novartis Pharmaceutical Corporation
	nimodipine	Nymalize	9/16/2011	Treatment of subarachnoid hemorrhage.	Arbor Pharmaceuticals, Inc.
	nimotuzumab	n/a	9/8/2015	Treatment of pancreatic cancer.	InnoCIMab Pte Ltd
	nintedanib	Ofev	6/29/2011	Treatment of patients with idiopathic pulmonary fibrosis.	Boehringer Ingelheim Pharmaceuticals, Inc.
	niprisan	Hemoxin	8/15/2003	Treatment of sickle cell disease	Xechem International, Inc.
	niraparib	n/a	4/30/2010	Treatment of ovarian cancer	TESARO, Inc.
	nitazoxanide	Cryptaz	10/23/2001	Treatment for intestinal amebiasis	Romark Laboratories, L.C.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	nitazoxanide	Alinia	2/14/2002	Treatment of intestinal giardiasis	Romark Laboratories, L.C.
	nitric oxide	Inomax (R)	9/5/2008	Use in combination with a drug delivery device for acute treatment of sickle cell vaso-occlusive crisis (pain crises)	INO Therapeutics
	nitric oxide	n/a	9/23/2014	Treatment of cystic fibrosis	Advanced Inhalation Therapies Ltd.
	nitric oxide	Inomax	12/28/2011	Treatment of pulmonary arterial hypertension	Bellerophon Therapeutics
	nitric oxide	n/a	6/18/2012	Treatment of persistent pulmonary hypertension in newborns	GeNO, LLC
	nivolumab	n/a	1/23/2013	Treatment of Stage IIb to IV melanoma	Bristol-Myers Squibb Co.
	nivolumab	Opdivo(R)	9/2/2015	Treatment of hepatocellular carcinoma.	Bristol-Myers Squibb Company
	nivolumab	n/a	9/15/2015	Treatment of small cell lung cancer.	Bristol-Myers Squibb Company
	nivolumab	n/a	8/27/2015	Treatment of glioblastoma	Bristol-Myers Squibb Co
	nivolumab	n/a	8/7/2014	Treatment of Hodgkin lymphoma	Bristol-Myers Squibb Co.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	non-peptide small molecule orally available agonist of the parathyroid hormone receptor type I	n/a	12/15/2014	Treatment of hypoparathyroidism	Chugai Pharma USA, LLC
	non-replicating adeno-associated viral vector, serotype 8, expressing human myotubularin gene	At001	12/3/2014	Treatment of x-linked myotubular myopathy	Audentes Therapeutics, Inc.
	non-replicating recombinant adeno-associated virus vector containing a fragment of the gene encoding channelrhodopsin-2 protein	n/a	10/20/2014	Treatment of retinitis pigmentosa	RetroSense Therapeutics, LLC
	obeticholic acid	n/a	4/9/2008	Treatment of primary biliary cirrhosis	Intercept Pharmaceuticals, Inc.
	obinutuzumab	Gazyva(R)	4/15/2015	Treatment of follicular lymphoma	Genentech, Inc.
	obinutuzumab	Gazyva	2/17/2012	Treatment of chronic lymphocytic leukemia	Genentech, Inc.
	obinutuzumab	n/a	2/17/2012	Treatment of diffuse large B cell lymphoma	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	obinutuzumab	Gazyva	6/11/2015	Treatment of splenic marginal zone lymphoma	Genentech, Inc.
	oblimersen	Genasense	7/31/2000	Treatment of advanced malignant melanoma (Stages II,III, IV).	Genta, Inc.
	oblimersen	Genasense	8/28/2001	Treatment of acute myelocytic leukemia	Genta, Inc.
	oblimersen	Genasense	8/28/2001	Treatment of chronic lymphocytic leukemia	Genta, Inc.
	oblimersen	Genasense	8/28/2001	Treatment of multiple myeloma	Genta, Inc.
	octreotide	Sandostatin Lar	8/5/2010	Treatment of neuroendocrine tumors	Novartis Pharmaceuticals Corporation
	octreotide (oral)	Octreolin(Tm)	6/17/2010	For the oral treatment of acromegaly	Chiasma, Inc.
	octreotide acetate subcutaneous implant	n/a	12/7/2009	Treatment of acromegaly	Endo Pharmaceuticals Solutions, Inc.
	ofatumumab	Arzerra	3/10/2009	Treatment of chronic lymphocytic leukemia	Novartis Pharmaceuticals Corp.
	ogluflanide disodium	n/a	9/24/2001	Treatment of ovarian cancer	Implicit Bioscience Pty Ltd
	olaparib	Lynparza	10/16/2013	Treatment of ovarian cancer	AstraZeneca Pharmaceuticals LP

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	olaptosed pegol	n/a	8/19/2014	Treatment of glioblastoma in conjunction with radiotherapy.	Noxxon Pharma AG
	olaratumab	n/a	10/9/2014	Treatment of soft tissue sarcoma	Eli Lilly and Company
	oleylphosphocholine	n/a	10/25/2013	Treatment of leishmaniasis	Dafra Pharma International nv
	oligopeptide containing 6 amino acids (H-Phe-Ser-Arg-Tyr-Ala-Arg-OH)	n/a	2/27/2014	Treatment of amyotrophic lateral sclerosis	Genervon Biopharmaceuticals, LLC
	olipudase alfa	n/a	8/3/2000	Treatment of acid sphingomyelinase deficiency (Niemann-Pick disease)	Genzyme Corporation
	omacetaine mepesuccinate	n/a	1/12/2009	Treatment of myelodysplastic syndromes	IVAX International GmbH
	omacetaxine mepesuccinate	Synribo	3/10/2006	Treatment of chronic myelogenous leukemia	IVAX International GmbH
	ombitasvir/paritaprevir/ritonavir and dasabuvir	Viekira Pak	7/16/2015	Treatment of pediatric patients with hepatitis C virus infection (0 through 16 years of age)	Abbvie, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ombrabulin; N-{2-methoxy-5-[(Z)-2-(3,4,5-trimethoxyphenyl)vinyl]phenyl}-L-serinamide hydrochloride	n/a	3/3/2011	Treatment of soft tissue sarcoma	Sanofi-Aventis U.S., Inc.
	omeprazole-lansoprazole with buffer	n/a	2/10/2015	Treatment of esophageal ulcers	Effexus Pharmaceuticals, LLC
	omigapil	n/a	6/24/2008	Treatment of congenital muscular dystrophy.	Santhera Pharmaceuticals Limited
	oncophage	n/a	4/14/2009	Treatment of glioma	Agenus, Inc.
	ondansetron inhalation powder	n/a	10/14/2015	Prevention of chemotherapy-induced nausea and vomiting due to highly emetogenic chemotherapy in pediatric patients (0 through 16 years of age) .	Luxena Pharmaceuticals, Inc.
	opioid growth factor	n/a	4/16/2013	Treatment of liver and intrahepatic bile duct cancer	Primocure Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	opium tincture	n/a	12/2/2011	Treatment of chronic diarrhea in short bowel syndrome patients with an inadequate response to anti-diarrheal treatment	Valeant Pharmaceuticals International, Inc.
	oprozomib	n/a	10/28/2014	Treatment of multiple myeloma	Onyx Therapeutics, Inc.
	oprozomib	n/a	8/25/2014	Treatment of Waldenstrom's macroglobulin emia	Onyx Therapeutics, Inc.
	optically pure phenylalanine derivative	n/a	8/20/2012	Treatment of narcolepsy	Jazz Pharmaceuticals International III Limited
	oral unfractionated heparin	n/a	1/29/2004	Treatment of sickle cell disease	TRF Technologies, Inc.
	osilodrostat	n/a	9/13/2013	Treatment of Cushing's disease	Novartis Pharmaceuticals corporation
	otelixizumab	n/a	2/6/2006	Treatment of new-onset type I diabetes mellitus	GlaxoSmithKline
	oxaloacetate	n/a	7/24/2012	Treatment of gliomas	Terra Biological LLC
	oxfendazole	n/a	7/14/2014	Treatment of cysticercosis (including neurocysticercosis)	Robert H. Gilman, MD, DTMH and
	oxybate	Xyrem	11/7/1994	Treatment of narcolepsy.	Jazz Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	oxytocin	n/a	11/24/2014	Treatment of Prader-Willi syndrome	Pr Maithe Tauber
	p1-(uridine 5')-p4-(2'-deoxycytidine 5') tetraphosphate, tetrasodium salt	n/a	3/7/2001	For the treatment of cystic fibrosis	Inspire Pharmaceuticals, Inc.
	p38 mitogen-activated kinase inhibitor	n/a	5/27/2014	Treatment of dilated cardiomyopathy, including dilated cardiomyopathy secondary to lamin A/C gene mutations	Array BioPharma, Inc.
	paclitaxel	n/a	5/1/2009	Treatment of pancreatic cancer	MediGene AG
	paclitaxel aqueous gel	Oncogel (Tm)	12/23/2008	Treatment of brain cancer.	BTG International Inc.
	paclitaxel aqueous gel	Oncogel(Tm)	2/1/2008	Treatment of esophageal cancer	BTG International, Inc.
	paclitaxel nanoparticles	n/a	1/2/2013	Treatment of pancreatic cancer	CIRJ Co., Ltd.
	paclitaxel poliglumex	Opaxio	9/20/2012	Treatment of glioblastoma multiforme	CTI BioPharma Corporation
	paclitaxel protein-bound particles	Abraxane	9/3/2009	Treatment of pancreatic cancer.	Abraxis BioScience, LLC
	paclitaxel, micellar	Paclical	4/3/2009	Treatment of ovarian cancer	Oasmia Pharmaceutical AB

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	paclitaxel-coated butress	n/a	7/15/2014	For use as local administration at the time of curative surgery in the treatment of stage 0 to III non-small cell lung cancer.	AcuityBio, Inc.
	pafuramidine maleate	n/a	11/17/2006	Treatment of pneumocystis jiroveci pneumonia	Immtech Pharmaceuticals, Inc.
	palovarotene	n/a	7/21/2014	Treatment of fibrodysplasia ossificans progressiva	Clementia Pharmaceuticals, Inc.
	panobinostat	Farydak	8/20/2012	Treatment of multiple myeloma	Novartis Pharmaceuticals Corporation
	pantothenate phosphate	n/a	5/4/2015	Treatment of pantothenate kinase associated neurodegener ation (PKAN).	Retrophin, Inc.
	paquinimod	n/a	1/17/2014	Treatment of systemic sclerosis	Active Biotech AB
	parathyroid hormone	Natpara	8/31/2007	Treatment of hypoparathyro idism	NPS Pharmaceuticals, Inc.
	paricalcitol	Zemplar	10/27/2015	Treatment of pediatric hyperparathyr oidism	Abbvie, inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	paromomycin	n/a	2/11/2015	Treatment of cutaneous leishmaniasis (Old World and New World)	The Surgeon General, Dept. of the Army
	paromomycin sulfate/gentamicin sulfate	n/a	1/18/2008	Treatment of all uncomplicated cutaneous leishmaniasis (both New World and Old World)	Office of the Surgeon General
	pasireotide	n/a	8/25/2009	Treatment of acromegaly	Novartis Pharmaceuticals Corporation
	pasireotide	Signifor	7/24/2009	Treatment of Cushing's disease	Novartis Pharmaceuticals Corporation
	pazopanib	n/a	5/6/2013	Treatment of ovarian cancer.	Novartis Pharmaceuticals Corp.
	pazopanib	Votrient	10/20/2009	Treatment of soft tissue sarcomas	Novartis Pharmaceuticals Corp.
	pegargiminsase	n/a	7/21/2014	Treatment of mesothelioma.	Polaris Group
	peginterferon alfa-2b	Sylatron	4/9/2008	Treatment of malignant melanoma stages IIb through IV.	Schering-Plough Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pegloticase	Krystexxa	2/21/2001	To control the clinical consequences of hyperuricemia in patients with severe gout in whom conventional therapy is contraindicated or has been ineffective.	Crealta Pharmaceuticals LLC
	pegsitacase	Uricase-Peg 20	12/3/2009	Treatment of Lesch-Nyhan syndrome.	EnzymeRx, LLC
	pegylated arginine deiminase	Hepacid	3/26/1999	Treatment of hepatocellular carcinoma	Polaris Pharmaceuticals, Inc.
	pegylated carboxyhemoglobin	n/a	10/21/2010	Treatment of acute painful sickling crises in patients with sickle cell disease	Sangart, Inc.
	pegylated carboxyhemoglobin bovine	Sanguinate	1/15/2015	Treatment of the comorbidities associated with Sickle Cell Disease	Prolong Pharmaceuticals, LLC
	pegylated granulocyte colony stimulating factor	n/a	4/30/2014	Treatment of patients at risk of developing myelosuppression following a radiological or nuclear incident	PharmaEssentia

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pegylated human recombinant arginase I	n/a	3/16/2015	Treatment of arginase I deficiency (hyperargininemia).	AERase, Inc.
	pegylated proline interferon alpha-2b	n/a	4/10/2014	Treatment of myelofibrosis	PharmaEssentia, Corporation
	pegylated proline interferon alpha-2b	n/a	4/11/2014	Treatment of essential thrombocythemia	PharmaEssentia, Corporation
	pegylated proline-interferon alpha-2b; PEG-P-IFNa-2b	n/a	4/2/2012	Treatment of polycythemia vera	PharmaEssentia Corporation
	pelareorep	Reolysin(R)	2/10/2015	Treatment of ovarian cancer	Oncolytics Biotech, Inc.
	pelareorep	Reolysin	4/15/2015	Treatment of malignant glioma	Oncolytics Biotech, Inc.
	pelareorep	Reolysin(R)	2/24/2015	Treatment of primary peritoneal cancer	Oncolytics Biotech, Inc.
	pelareorep	Reolysin	2/24/2015	Treatment of fallopian tube cancer	Oncolytics Biotech, Inc.
	pelareorep	Reolysin	5/4/2015	Treatment of gastric cancer	Oncolytics Biotech, Inc.
	pelareorep	Reolysin(R)	2/11/2015	Treatment of pancreatic cancer	Oncolytics Biotech, Inc.
	pembrolizumab	Keytruda	6/16/2015	Treatment of gastric cancer, including gastroesophageal junction adenocarcinoma	Merck, Sharp & Dohme

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pembrolizumab	Keytruda	11/19/2012	Treatment of Stage IIB through IV malignant melanoma	Merck, Sharp & Dohme Corp.
	pemetrexed disodium	Alimta	8/28/2001	Treatment of malignant pleural mesothelioma	Eli Lilly and Company
	pentagastrin	n/a	5/29/2009	To aid in the diagnosis of medullary thyroid carcinoma	Medical Defense Technologies, LLC
	pentapeptide with sequence Ac-VSRRR-NH2	n/a	1/13/2015	Treatment of intracerebral hemorrhage	CereNOva, LLC
	pentetate trisodium	Diethylenetriaminepentaacetate	4/12/2004	Treatment of patients with known or suspected internal contamination with plutonium, americium, or curium.	Heyl Chemisch-Pharmazeutische Fabrik GMBH & Co. KG
	pentetrazol	n/a	9/9/2015	Treatment of idiopathic hypersomnia.	Balance Therapeutics, Inc.
	pentosan polysulfate sodium	n/a	11/21/2008	Treatment of sickle cell disease.	TRF Pharma, Inc.
	pentosan polysulfate sodium	Lysosan (Tm)	1/5/2015	Treatment of mucopolysaccharidosis (MPS) type VI	Plexcera Therapeutics, LLC
	pentosan polysulfate sodium	n/a	9/16/2011	Treatment of sickle cell disease.	Vanguard Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pentoxifylline	n/a	6/14/2012	Treatment of Behcet's disease	Keck Graduate Institute of Applied Life Sciences
	peptide YY	n/a	4/18/2011	Treatment of hepatocellular carcinoma.	Mondobiotech Laboratories AG
	peptide-based vaccine targeting telomerase reverse transcriptase	n/a	2/20/2009	Treatment of telomerase reverse transcriptase (TERT) positive non-small cell lung cancer in HLA-A*0201 positive patients	VAXON-Biotech
	peptide-conjugated phosphorodiamidate morpholino oligomer (PPMO)	n/a	9/3/2009	Treatment of Duchenne muscular dystrophy in patients with a deletion, duplication or frame shift mutation correctable by skipping of exon 50 of the dystrophin gene to allow restoration of the reading frame (including mutations within exons 51, 51-53, or 51-55).	Sarepta Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	peptides mimicking antigen receptors on autoimmune B cells and autoimmune T cells associated with myasthenia gravis	n/a	2/4/2011	Treatment of myasthenia gravis	CuraVac Europe SPRL
	perampanel	Fycompa	12/7/2012	Treatment of Lennox-Gastaut Syndrome	Eisai, Inc.
	peretinoin	n/a	9/23/2011	Treatment of hepatocellular carcinoma.	Kowa Pharmaceutical Europe Co. Ltd.
	perhexiline maleate	n/a	6/14/2012	Treatment of moderate to severe symptomatic (NYHA class III or IV) hypertrophic cardiomyopathy	Heart Metabolics, Limited.
	perifosine	n/a	7/9/2010	Treatment of neuroblastoma	Aeterna Zentaris GmbH
	perillyl alcohol	n/a	4/18/2011	Treatment of glioma	NeOnc Technologies, Inc.
	pertuzumab	n/a	7/12/2013	Treatment of gastric cancer	Genentech, Inc.
	pertuzumab	n/a	6/8/2009	Treatment of ovarian cancer	Genentech, Inc.
	pexastimogene devacirepvec	n/a	5/6/2013	Treatment of hepatocellular carcinoma	SillaJen Biotherapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	phenobarbital sodium injection	n/a	10/23/2013	Treatment of hypoxic-ischemic encephalopathy to prevent seizures in neonates	Fera Pharmaceuticals, LLC
	phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl) monohydrochloride	n/a	3/16/2015	Treatment of Huntington's disease.	Ipsen Biopharmaceuticals, Inc.
	phenylephrine	n/a	2/14/2002	Treatment of ileal pouch anal anastomosis related fecal incontinence	S.L.A. Pharma
	phenylephrine	n/a	1/31/2012	Treatment of Tetralogy of Fallot	Luitpold Pharmaceuticals, Inc.
	phosphorothioate antisense oligonucleotide against EWS-Fli-1	n/a	9/22/2008	Treatment of Ewing's sarcoma	The Cure Our Children Foundation
	phoxilium	n/a	2/14/2014	For use as a replacement solution in patients undergoing continuous renal replacement therapy	Gambro Renal Products, Inc.
	picibanil	n/a	5/15/2009	Treatment of patients with lymphatic malformations	Royds Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	picibanil	n/a	3/17/2011	Treatment of lymphatic malformations	Richard Smith, M.D.
	pirfenidone	Esbriet	3/5/2004	Treatment of idiopathic pulmonary fibrosis	Genentech
	pirfenidone	n/a	7/31/2014	Treatment of idiopathic pulmonary fibrosis	Genoa Pharmaceuticals, Inc.
	pirfenidone	n/a	11/19/2013	Treatment of systemic sclerosis (including the associated interstitial lung disease)	Genentech, Inc.
	plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a nonviral cationic lipid based gene transfer agent	n/a	6/30/2014	Treatment of cystic fibrosis	Imperial Innovations Limited
	plasmin (human)	n/a	3/30/2009	Treatment of acute peripheral arterial occlusion	Grifols Therapeutics, Inc.
	plasminogen (human)	n/a	3/5/2013	Treatment of hypoplasminogenemia, or type I plasminogen deficiency	ProMetic Biotherapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pleconaril	n/a	12/22/2014	Treatment of symptomatic entoviral infection in the neonate	AntiVirus Therapeutics, Inc.
	plerixafor	Mozobil (R)	7/10/2003	For use to improve the yield of progenitor cells in the apheresis product for subsequent stem cell transplantation following myelosuppressive or myeloablative chemotherapy	Genzyme Corporation
	polidocanol	n/a	2/19/2015	Treatment of congenital venous malformations	Provensis Ltd
	polifeprosan 20 with carmustine	Gliadel	12/13/1989	Treatment of malignant glioma.	Guilford Pharmaceuticals, Inc.
	poloxamer 188	n/a	11/8/2013	Treatment of Acute Limb Ischemia	Mast Therapeutics Inc.
	poloxamer 188 (purified)	n/a	6/27/1989	Treatment of sickle cell disease (this includes the treatment and prevention of complications of sickle cell disease)	Mast Therapeutics Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	poloxamer-188 NF	n/a	1/19/2010	Treatment of Duchenne muscular dystrophy	Phrixus Pharmaceuticals, Inc.
	poly(lactide-co- glycolide) carboxylated microparticle	n/a	10/25/2013	Treatment of acute encephalitis syndrome	Cour Pharmaceutical Development Company, Inc.
	poly-CD-PEG- camptothecin	n/a	5/21/2015	Treatment of ovarian cancer.	Cerulean Pharma, Inc.
	polyethylene glycol modified recombinant C- terminal truncate of human cystathionine beta-synthase	n/a	3/17/2015	Treatment of homocystinuri a	Orphan Technologies Limited
	polyinosinic- polycytidilic acid	Poly-Iclc	8/2/2002	As an adjuvant to smallpox vaccination	Oncovir
	polyinosinic- polycytidilic acid	Poly-Iclc	3/3/2003	Treatment of flavivirus infections including those due to West Nile, Japanese encephalitis, dengue, St. Louis encephalitis, yellow fever, Murray valley, and Banzai viruses	Oncovir
	polyinosinic- polycytidilic acid (Poly-ICLC)	Hiltonol	11/19/2002	Treatment for orthopox virus infections	Oncovir

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	polyphenon E	n/a	7/17/2008	Treatment of chronic lymphocytic leukemia	Mitsui Norin Co., Ltd
	pomalidomide	Pomalyst	1/15/2003	Treatment of multiple myeloma	Celgene Corporation
	pomalidomide	n/a	9/21/2010	Treatment of persons with myeloproliferative neoplasm-associated myelofibrosis and anemia who are red blood cell transfusion dependent.	Celgene Corporation
	ponatinib	Iclusig(R)	3/12/2015	Treatment of gastrointestinal stromal tumors (GIST).	ARIAD Pharmaceuticals, Inc.
	ponatinib	Iclusig	11/20/2009	Treatment of Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ALL)	ARIAD Pharmaceuticals Inc.
	ponatinib	Iclusig	11/20/2009	Treatment of chronic myeloid leukemia	ARIAD Pharmaceuticals Inc.
	porcine GM1 ganglioside	n/a	12/3/2012	Treatment of acute spinal cord injury	TRB Chemedica International S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	porfimer	Photofrin	10/19/2001	For the ablation of High-Grade Dysplasia in Barrett's Esophagus in patients who are not considered to be candidates for esophagectomy	Axcan Scandipharm Inc.
	potassium sodium aluminosilicate	n/a	1/8/2007	For treatment of poisoning by or exposure to cesium.	Framework Therapeutics, LLC
	pracinostat	n/a	2/27/2014	Treatment of acute myeloid leukemia	MEI Pharma Inc.
	pralatrexate	Folotyn	7/20/2006	Treatment of T-cell lymphoma	Allos Therapeutics, Inc.
	pralatrexate	n/a	5/3/2010	Treatment of advanced or metastatic transitional cell carcinoma of the urinary bladder	Allos Therapeutics, Inc.
	pralatrexate	n/a	10/20/2008	Treatment of follicular lymphoma	Allos Therapeutics, Inc.
	pralatrexate	n/a	10/20/2008	Treatment of diffuse large B-cell lymphoma	Allos Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pralmorelin hydrochloride	n/a	10/18/2012	As a diagnostic agent for the detection of growth hormone deficiency	Sella Pharmaceuticals, Inc.
	pramipexole	Mirapex	1/31/2008	Treatment of Tourette's syndrome in pediatric patients	Boehringer-Ingelheim Pharmaceuticals, Inc.
	prasugrel hydrochloride	Effient	5/26/2015	Treatment of sickle cell disease	Eli Lilly
	pritumumab	n/a	10/28/2014	Treatment of glioma	Nascent Biotech, Inc.
	procaspase-activating compound 1	n/a	7/23/2015	Treatment of malignant glioma	Vanquish Oncology, Inc.
	profimer sodium	Photofrin(R)	12/2/2011	Treatment of malignant mesothelioma	Concordia Laboratories, Inc.
	progesterone	n/a	12/22/1994	Establishment and maintenance of pregnancy in women undergoing in vitro fertilization or embryo transfer procedures.	Watson Laboratories, Inc.
	prolactin receptor antagonist	Prolanta	4/15/2013	Treatment of ovarian cancer	Oncolix, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	propranolol	Hemangeol	9/5/2008	Treatment of proliferating infantile hemangiomas requiring systemic therapy	Pierre Fabre Dermatologie
	propranolol and etodolac	n/a	7/8/2015	Treatment of malignant glioma.	Vicus Therapeutics, LLC
	propranolol and etodolac	n/a	4/27/2015	Treatment of pancreatic cancer.	Vicus Therapeutics, LLC
	propranolol hydrochloride and etodolac	n/a	2/24/2015	Treatment of hepatocellular carcinoma	Vicus Therapeutics, LLC
	prothrombin complex concentrate (human)	Kcentra	12/27/2012	Treatment of patients needing urgent reversal of Vitamin K antagonist therapy for treatment of major bleeding and/or surgical procedures	CSL Behring
	purified autologous type 1 regulatory T lymphocytes specific for human type II collagen	n/a	9/1/2015	Treatment of chronic non-infectious uveitis	TxCell SA
	purified bovine type collagen	n/a	4/27/2009	Treatment of idiopathic pulmonary fibrosis	ImmuneWorks, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	pyridoxine; vitamin B6	n/a	3/3/2011	Treatment of pyridoxine dependent seizures.	NBI Pharmaceuticals, Inc.
	pyrimethamine	n/a	8/16/2011	Treatment of GM-2 gangliosidoses (Tay-Sachs disease and Sandhoff disease).	ExSAR Corporation
	pyrvinium	n/a	1/5/2015	Treatment of familial adenomatous polyposis	StemSynergy Therapeutics, Inc.
	quinacrine	n/a	9/28/2012	Treatment of hepatocellular carcinoma	Cleveland BioLabs, Inc & Incuron, LLC Joint Ventur
	quinine Sulfate	n/a	6/3/2004	Treatment of malaria	AR Holding Company, Inc.
	quinine sulfate	n/a	12/2/2008	Treatment of malaria excluding Plasmodium faliparum	Zydus Pharmaceuticals, Inc.
	rAAV2-CB- hRPE65	n/a	2/11/2005	Treatment of type II Leber's Congenial Amaurosis	Applied Genetic Technologies Corp.
	rSP-C lung surfactant	Venticute	4/3/2000	Treatment of adult respiratory distress syndrome.	Byk Gulden Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	rSP-C surfactant	Venticute	9/18/2006	For use in patients with pneumonia or aspiration of gastric contents leading to intubation, mechanical ventilation, and severe oxygen impairment	Altana Pharma
	rVIIa-FP	n/a	12/22/2011	Treatment and prophylaxis of bleeding episodes in patients with congenital hemophilia and inhibitors to coagulation factor VIII or IX	CSL Behring
	radiolabeled somastatin analog	Galiomedix(TM)	12/31/2013	Diagnostic for the management of neuroendocrine tumors	RadioMedix, Inc.
	raloxifene	Evista	7/14/2005	Reduction of the risk of breast cancer in postmenopausal women	Eli Lilly and Company
	raloxifene hydrochloride	Evista	8/20/2010	Treatment of hereditary hemorrhagic telangiectasia	Consejo Superior de Investigaciones Cientificas

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ramucirumab	n/a	11/4/2011	Treatment of hepatocellular carcinoma	Eli Lilly and Company
	ramucirumab	Cyramza	2/16/2012	Treatment of gastric cancer	Eli Lilly and Company
	ranagengliotucel-L	Glionix(Tm)	5/29/2009	Treatment of astrocytic tumors	NovaRx Corporation
	ranprinase	Onconase	1/25/2007	Treatment of malignant mesothelioma	Alfacell Corporation
	rasburicase	Elitek	10/11/2000	Treatment of malignancy-associated or chemotherapy-induced hyperuricemia.	Sanofi-Synthelabo Research
	raxibacumab	Abthraxtm	11/12/2003	Treatment of anthrax	Human Genome Sciences, Inc.
	recombinant AAV9 expressing human sulfoglucosamine sulfohydrolase	n/a	4/29/2014	Treatment of mucopolysaccharidosis type III-A (Sanfilippo Syndrome Type A)	Abeona Therapeutics
	recombinant DNA plasmid	n/a	1/31/2014	Treatment of stage IIb, IIc, III and IV melanoma.	Scancell Ltd.
	recombinant IgA protease of bacterium Heamophilus influenzae	n/a	4/18/2011	Treatment of immunoglobulin A nephropathy (IgAN, Berger's disease).	Shire Human Genetics Therapies
	recombinant activated Factor X variant	n/a	3/3/2015	Treatment of intracerebral hemorrhage	Pfizer, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant adeno-associated virus vector AAV2/rh8 expressing human B-hexosaminidase A and B subunits	n/a	3/25/2013	For the treatment of Sandhoff disease	Nat'l Tay-Sachs & Allied Diseases Association
	recombinant adeno-associated virus alpha 1-antitrypsin vector	Raav-Aat	1/27/2003	Treatment of alpha1-antitrypsin deficiency	Applied Genetic Technologies Corp.
	recombinant adeno-associated virus encoded gene for X-linked mammalian inhibitor of apoptosis protein (XIAP)	n/a	8/25/2009	Treatment of Huntington's disease.	Neurologix, Inc.
	recombinant adeno-associated virus serotype 9 vector containing the transgene UBE3A encoding for ubiquitin protein ligase E3A/E6-AP	n/a	10/29/2015	Treatment of Angelman Syndrome.	Agilis Biotherapeutics, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant adeno-associated virus serotype rhesus 74 (rh74) expressing the human CT GalNac transferase (GALGT2) gene, also called the Sda or Cad GAlNac transferase or b4Galnt2, under control of the muscle creatine kinase (MCK) promoter	n/a	6/16/2015	Treatment of Duchenne muscular dystrophy	Nationwide Children's Hospital
	recombinant adeno-associated virus vector expressing the cyclic nucleotide gated channel alpha subunit (CNGA3)	n/a	11/16/2015	Treatment of achromatopsia caused by mutations in the CNGA3 gene	Applied Genetic Technologies Corp.
	recombinant adeno-associated virus vector expressing the cyclic nucleotide gated channel beta subunit (rAAV-CNGB3)	n/a	2/4/2011	Treatment of achromatopsia caused by mutations in the CNGB3 gene.	Applied Genetic Technologies Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant adenovirus vector AAV2/rh8 expressing human B-hexosaminidase A & B subunits	n/a	3/25/2013	Treatment of Tay-Sachs disease	Na't Tay-Sachs & Allied Diseases Association
	recombinant chimeric monoclonal antibody to anthrax	Anthim	6/9/2006	Treatment of exposure to B. anthracis spores	Elusys Therapeutics, Inc.
	recombinant derivative of C3 transferase	Cethrin	11/18/2005	Treatment of acute spinal cord injury	Vertex Pharmaceuticals, Inc.
	recombinant elafin	n/a	3/18/2013	Prevention of inflammatory complications of transthoracic esophagectomy	Proteo Biotech AG
	recombinant fusion protein comprising exenatide and XTEN	n/a	10/7/2015	Treatment of short bowel syndrome.	NAIA Pharmaceuticals, Inc.
	recombinant fusion protein consisting of a modified form of extracellular domain of human Activin receptor IIB	n/a	3/18/2013	Treatment of myelodysplastic syndrome	Celgene Corporation

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant fusion protein consisting of a modified form of the extracellular domain of human activin receptor IIB (ActRIIB) linked to a human IgG1 Fc domain	n/a	3/11/2013	Treatment of B-thalassemia	Celgene Corporation
	recombinant fusion protein linking coagulation factor IX with albumin (rIX-FP)	n/a	4/27/2012	Treatment of patients with congenital factor IX deficiency (hemophilia B).	CSL Behring, LLC
	recombinant fusion protein linking coagulation factor VIIa with albumin (rVIIa-FP)	n/a	5/6/2013	Treatment of congenital factor VII deficiency which includes treatment and prophylaxis of bleeding episodes in patients with congenital factor VII deficiency	CSL Behring
	recombinant fusion protein with a truncated form of the cytotoxic protein Pseudomonas exotoxin	Proxinium	1/28/2005	Treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck	Viventia Biotech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant glycosylated independent lysosomal targeting (GILT) tagged human acid alpha glucosidase	n/a	8/20/2010	Treatment of Pompe disease.	BioMarin Pharmaceutical, Inc.
	recombinant human CD24FcIg (humanized fusion protein consisting of the extracellular domain of CD24 linked to IgG1 Fc domain	n/a	6/9/2015	Prevention of graft-versus-host disease	Oncolmmune, Inc.
	recombinant human GM-CSF, molgramostim	n/a	10/31/2012	Treatment of pulmonary alveolar proteinosis	Serendex ApS
	recombinant human IgG1 monoclonal antibody	n/a	12/14/2009	Treatment of X-linked hypophosphatemia (formerly known as vitamin D-resistant rickets)	Ultragenyx Pharmaceutical, Inc.
	recombinant human Naglu-insulin-like growth factor II	n/a	3/5/2013	Treatment of mucopolysaccharidosis type IIIB (Sanfilippo syndrome type B)	Shire Human Genetic Therapies, Inc.
	recombinant human Pentraxin-2	n/a	8/26/2014	Treatment of myelofibrosis	Promedior, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human Pentraxin-2; recombinant human Serum Amyloid P	n/a	2/17/2012	Treatment of idiopathic pulmonary fibrosis.	Promedior, Inc.
	recombinant human acid ceramidase	Plexcerase(Tm)	12/24/2013	Treatment of Faber disease	Plexcera Therapeutics, LLC
	recombinant human alkaline phosphatase	n/a	5/13/2015	Treatment of hypophosphat asia.	AM-Pharma BV
	recombinant human alpha 1-antitrypsin (rAAT)	n/a	11/20/2001	Treatment of cystic fibrosis	AiroMedica LLC
	recombinant human alpha-1 antitrypsin	n/a	3/6/1998	Treatment of cystic fibrosis.	PPL Therapeutics (Scotland) Limited
	recombinant human alpha-1 antitrypsin (rAAT)	n/a	8/28/2001	To delay progression of chronic obstructive pulmonary disease resulting from AAT deficiency-mediated emphysema and bronchiectasis	AiroMedica LLC
	recombinant human alpha-N-acetylglucosaminidase	n/a	4/15/2013	Treatment of mucopolysaccharidosis IIIB (Sanfilippo B syndrome)	Alexion Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human alpha-glucosidase conjugated with synthetic bis-mannose-6-phosphate-Man6 glycan	n/a	11/19/2013	Treatment of Pompe Disease	Genzyme, a Sanofi Company
	recombinant human anti-GDF-8 monoclonal antibody	n/a	7/24/2012	Treatment of Duchenne Muscular Dystrophy.	Pfizer, Inc.
	recombinant human antithrombin	Atryn	12/7/2007	Treatment of congenital antithrombin deficiency to prevent the occurrence of serious, potentially life-threatening venous thromboembolisms which may develop as a result of surgical or obstetrical procedures	GTC Biotherapeutics, Inc.
	recombinant human beta-glucuronidase	n/a	2/16/2012	Treatment of mucopolysaccharidosis VII (MPS VII, Sly Syndrome)	Ultragenyx Pharmaceutical, Inc.
	recombinant human collagen alpha-1 (VII) chain homotrimer (rC7)	n/a	3/17/2014	Treatment of dystrophic epidermolysis bullosa	Shire Human Genetic Therapies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human deoxyribonuclease I	n/a	8/18/2014	Treatment of graft-vs-host disease	SciVac Ltd.
	recombinant human deoxyribonuclease I (DNase I)	n/a	8/18/2014	Prevention of graft-vs-host disease	SciVac Ltd.
	recombinant human endostatin protein	n/a	2/21/2002	Treatment of metastatic melanoma	EntreMed, Inc.
	recombinant human erythropoietin (rHuEPO)	n/a	5/26/2011	Treatment of multiple myeloma	XTL Biopharmaceuticals, Ltd
	recombinant human fucosyltransferase VI [ FTVI ] + GDP-fucose	n/a	1/27/2015	To increase the activity of regulatory T-cells (Tregs) used in the prevention of graft versus host disease in patients undergoing hematopoietic stem cell transplantation	TargaZyme, Inc.
	recombinant human galactocerebrosidase (rhGALC);	Galaczym	12/12/2011	Treatment of globoid cell leukodystrophy (Krabbe Disease)	ACE BioSciences A/S
	recombinant human growth hormone in an ocular delivery system	n/a	12/3/2012	Treatment of persistent corneal epithelial defects	Jade Therapeutics LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human histidyl tRNA synthetase	n/a	4/22/2015	Treatment of facioscapulohumeral muscular dystrophy	aTyr Pharma, Inc.
	recombinant human histone H1.3; recombinant human N-bis-methyl histone H1.3	Oncohist	10/20/2008	Treatment of acute myeloid leukemia	Xenetic Biosciences Plc
	recombinant human lecithin:cholesterol acyltransferase (rhLCAT)	n/a	9/2/2010	Treatment of LCAT deficiency syndromes	AlphaCore Pharma, LLC
	recombinant human minibody against complement component	Mubodina(R)	6/7/2011	Treatment of atypical hemolytic uremic syndrome associated with an inherited abnormality of the complement system.	Adienne S.A
	recombinant human minibody against complement component C5	Mubodina	2/4/2009	Treatment of primary membranoproliferative glomerulonephritis	Adienne S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human minibody against complement component C5 fused with RGD-motif	n/a	2/4/2009	Prevention of ischemia/reperfusion injury associated with solid organ transplantation.	Adienne S.A.
	recombinant human monoclonal IgG1 antibody against programmed death ligand-1 (anti-PD-L1)	n/a	9/21/2015	Treatment of merkel cell carcinoma.	EMD Serono, Inc.
	recombinant human monoclonal IgM antibody targeting glucose regulated protein 78	n/a	10/25/2013	Treatment of multiple myeloma	Patrys Ltd.
	recombinant human monoclonal antibody against activin receptors type II	n/a	6/18/2012	Treatment of inclusion body myositis	Novartis Pharmaceuticals Corp.
	recombinant human monoclonal antibody of the IgG1 kappa class against human macrophage colony-stimulating factor	n/a	8/19/2014	Treatment of pigmented villonodular synovitis, including giant cell tumor of the tendon sheath	Novartis Pharmaceuticals Corp

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human monoclonal antibody to hsp90	Mycograb	9/16/2002	Treatment of invasive candidiasis	Novartis Pharmaceuticals Corp.
	recombinant human nerve growth factor	n/a	8/8/2013	Treatment of retinitis pigmentosa	Dompe s.p.a.
	recombinant human nerve growth factor	n/a	6/23/2014	Treatment of neurotrophic keratitis	Dompe s.p.a.
	recombinant human neutrophil inhibitor (hNE)	n/a	12/9/2003	Treatment of cystic fibrosis	Dyax Corporation
	recombinant human platelet derived growth factor-BB	n/a	8/6/2010	Treatment of osteochondritis dissecans	Biomimetic Therapeutics, Inc.
	recombinant human porphobilinogen deaminase	Porphozyme	9/9/2002	Treatment of acute intermittent porphyria attacks	Zymenex A/S
	recombinant human porphobilinogen deaminase, erythropoetic form	n/a	7/11/2002	Treatment of acute intermittent porphyria preventing attacks	Zymenex A/S
	recombinant human proinsulin (Including rhPI-Methionine)	n/a	12/10/2008	Treatment of retinitis pigmentosa	ProRetina Therapeutics, S.L.
	recombinant human surfactant protein D	n/a	6/23/2014	Prevention of bronchopulmonary dysplasia	Airway Therapeutics LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant human tripeptidyl-peptidase 1 (rhTPP1)	n/a	4/1/2013	Treatment of neuronal ceroid lipofuscinosis type 2	BioMarin Pharmaceutical, Inc.
	recombinant human type I pancreatic elastase	n/a	4/3/2009	Prevention of arteriovenous fistula and arteriovenous graft failure in patients with chronic kidney disease (CKD) who are receiving hemodialysis or preparing for hemodialysis	Proteon Therapeutics, Inc.
	recombinant human type VII collagen	n/a	6/18/2008	Treatment of hereditary dystrophic epidermolysis bullosa (DEB)	David T. Woodley, MD and Mei Chen, MD
	recombinant human vascular endothelial growth factor	n/a	6/7/2010	Treatment of amyotrophic lateral sclerosis	NeuroNova AB
	recombinant humanized IgG1k monoclonal antibody to human invariant T cell receptor (iTCR)	n/a	4/12/2013	Treatment of sickle cell disease	NKT Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant humanized anti-interleukin 13 (IL-13) monoclonal antibody	n/a	2/11/2015	Treatment of eosinophilic esophagitis	Receptos, Inc.
	recombinant humanized anti-matrix metalloprotein 9 (MMP9) monoclonal antibody IgG4	n/a	7/16/2015	Treatment of gastric cancer	Gilead Sciences, Inc.
	recombinant humanized anti-tau antibody	n/a	1/22/2015	Treatment of progressive supranuclear palsy	C2N Diagnostics, LLC
	recombinant humanized monoclonal antibody of the immunoglobulin G1 subclass directed against colony stimulating factor-1 receptor expressed on macrophages	n/a	5/14/2014	Treatment of pigmented villonodular synovitis and tenosynovial giant cell tumor	Genentech, Inc.
	recombinant kallikrein inhibitor	n/a	11/23/2010	Treatment of Netherton Syndrome.	Dermadis SA
	recombinant lens epithelium derived growth factor 1-326	n/a	5/19/2014	Treatment of retinitis pigmentosa	Ocugen, Inc.
	recombinant monoclonal antibody to human serum amyloid P component	n/a	2/10/2015	Treatment of AL amyloidosis	Glaxo Group Limited, England d/b/a GlaxoSmithKline

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	recombinant multimerized human IgG1 Fc	n/a	7/14/2015	Treatment of chronic inflammatory demyelinating polyneuropathy (CIDP).	Pfizer, Inc.
	recombinant nematode anticoagulant protein c2 (rNAPc2)	n/a	12/8/2014	Treatment of viral hemorrhagic fever post-exposure to Ebola virus (treatment of Ebola)	ARCA Biopharma, Inc.
	recombinant neuroglobin (rNgb) containing mutations H64Q/C46G/C55S/C120S	n/a	11/17/2014	Treatment of patients with carbon monoxide poisoning	University of Pittsburgh
	recombinant ovine interferon tau	Tauferon	1/25/2005	Treatment of pediatric multiple sclerosis	PEPGEN Corporation
	recombinant thymidine phosphorylase encapsulated with autologous erythrocytes	n/a	12/13/2010	Treatment of mitochondrial neurogastrointestinal encephalomyopathy due to thymidine phosphorylase deficiency.	St. George's University of London
	recombinant von Willebrand factor (rhVWF)	n/a	11/23/2010	Treatment of von Willebrand disease.	Baxalta, Inc.
	reduced oxidized N-acetyl heparin	n/a	3/19/2015	Treatment of multiple myeloma	Sigma-Tau Research Switserland S.A.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	refanalin	n/a	5/25/2010	To improve renal function and prevent delayed graft function following renal transplantation	Angion Biomedica Corporation
	regorafenib	Stivarga	1/12/2011	Treatment of gastrointestinal stromal tumors	Bayer HealthCare Pharmaceuticals, Inc.
	regorafenib	Stivarga	6/4/2015	Treatment of hepatocellular carcinoma.	Bayer HealthCare Pharmaceuticals, Inc.
	reparixin	n/a	9/25/2012	Prevention of graft loss in pancreatic islet transplantation	Dompe S.p.A.
	reparixin	n/a	1/27/2003	Prevention of delayed graft function in solid organ transplant	Dompe S.p.A.
	replication-deficient recombinant serotype 2 adeno-associated viral vector containing hAQP1 cDNA	n/a	5/3/2013	Treatment of symptoms of Grade 2 and Grade 3 late xerostomia from parotid gland hypofunction caused by radiotherapy for cancer of the oral cavity.	John A. Chiorini, PhD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	repository corticotropin injection	H.P. Acthar Gel	6/28/2013	Treatment of amyotrophic lateral sclerosis	Questor Pharmaceuticals, Inc.
	repository corticotropin or adrenocorticotropic hormone	H.P. Acthar Gel	5/21/2003	Treatment of infantile spasms	Questcor Pharmaceuticals, Inc.
	resiniferatoxin	n/a	5/13/2003	Treatment of intractable pain at end-stage disease	Sorrento Therapeutics, Inc.
	reslizumab	Cinquil	1/12/2011	Treatment of hypereosinophilic syndrome	Teva Pharmaceuticals, Inc.
	reslizumab	n/a	12/19/2007	Treatment of children with eosinophilic esophagitis	TEVA
	resminostat	n/a	9/16/2011	Treatment of Hodgkin's lymphoma.	4SC AG
	resminostat	n/a	6/29/2011	Treatment of hepatocellular carcinoma	4SC AG
	retroviral gamma-c cDNA containing vector	n/a	4/29/2002	Treatment of X linked severe combined immune deficiency disease	AVAX technologies, Inc.
	revusiran	n/a	5/18/2015	Treatment of transthyretin amyloidosis	Alnylam Pharmaceuticals, Inc.
	rh-microplasmin, ocriplasmin	Jetrea	3/16/2004	Adjunct to surgery in cases of pediatric vitrectomy	ThromboGenics Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	rhIGF-I/rhIGFBP-3	Somatokine	12/9/2003	Treatment of extreme insulin resistance syndromes (type A, Rabson-Mendenhall syndrome, Leprechaunism, Type B syndrome)	Insmmed, Inc.
	ribavirin	Virazole	4/12/1991	Treatment of hemorrhagic fever with renal syndrome	Valeant Pharmaceuticals International
	ribavirin	Rebetol	4/4/2003	Treatment of chronic hepatitis C in pediatric patients	Schering Corporation
	ribavirin elaidate	n/a	9/2/2011	Treatment of follicular, medullary, and anaplastic thyroid carcinoma and metastatic or locally advanced papillary thyroid cancer	Translational Therapeutics, Inc.
	riboflavin ophthalmic solution & ultraviolet A	n/a	9/2/2011	Treatment of keratoconus	Avedro, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	riboflavin ophthalmic solution ultraviolet-A (UVA) irradiation	n/a	12/2/2011	Treatment of corneal ectasia following refractive surgery	Avedro, Inc.
	ricin vaccine	Rivax(R)	1/7/2011	Prevention of ricin intoxication.	Soligenix, Inc.
	rifaximin	Normix	2/10/1998	Treatment of hepatic encephalopathy	Salix Pharmaceuticals, Inc.
	rigosertib	n/a	3/16/2012	Treatment of ovarian cancer	Onconova Therapeutics, Inc.
	rigosertib	n/a	3/18/2011	Treatment of pancreatic cancer.	Onconova Therapeutics, Inc
	rigosertib	n/a	9/3/2009	Treatment of myelodysplastic syndromes	Onconova Therapeutics, Inc.
	rilonacept	Arcalyst	1/9/2013	Treatment of familial Mediterranean fever	Philip J Hashkes, MD, MSc.
	rilotumumab	n/a	6/18/2012	Treatment of gastric cancer including gastroesophageal junction adenocarcinoma	Amgen, Inc.
	rindopepimut	n/a	11/19/2007	Treatment of EGFRvIII-expressing glioblastoma multiforme	Celldex Therapeutics, Inc.
	riociguat	Adempas	9/19/2013	Treatment of pulmonary arterial hypertension.	Bayer HealthCare Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	riociguat	Adempas	7/24/2014	Treatment of systemic sclerosis	Bayer HealthCare Pharmaceuticals
	riociguat	Adempas	9/19/2013	Treatment of chronic thromboembolic pulmonary hypertension	Bayer HealthCare Pharmaceuticals, Inc.
	rituximab	Rituxan	6/13/1994	Treatment of non-Hodgkin's B-cell lymphoma	Genentech, Inc.
	rituximab	Rituxan	1/29/2004	Treatment of chronic lymphocytic leukemia	Genentech, Inc.
	rituximab	Rituxan	3/12/2002	Treatment of immune thrombocytopenic purpura	Genentech, Inc.
	rituximab	Rituxan(R); Mabthera(R)	2/23/2015	Treatment of pemphigus vulgaris.	Genentech, Inc.
	rituximab	Rituxan	2/14/2006	Treatment of patients with anti-neutrophil cytoplasmic antibody-associated vasculitis (Wegener's Granulomatosis, Microscopic Polyangiitis, and Churg-Strauss Syndrome)	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	rociletinib	n/a	5/14/2013	Treatment of non-small cell lung cancer and mutations in the epidermal growth factor receptor	Clovis Oncology, Inc.
	rofecoxib	Vioxx	3/16/2004	Treatment of juvenile rheumatoid arthritis	MERCK & Co., Inc.
	romidepsin	Istodax	9/30/2004	Treatment of non-Hodgkin T-cell lymphomas	Celgene Corporation
	romiplostim	Nplate	3/27/2003	Treatment of immune thrombocytopenic purpura	Amgen, Inc.
	romiplostim	n/a	10/31/2007	Treatment of thrombocytopenia associated with myelodysplasia syndrome	Amgen Inc.
	rosuvastatin	Crestor	2/14/2014	For the treatment of pediatric homozygous familial hypercholesterolemia	AstraZeneca Pharmaceuticals LP
	rozrolimupab	n/a	9/13/2010	Treatment of primary immune thrombocytopenia.	Symphogen A/S
	rsATP7A cDNA	n/a	1/10/2014	Treatment of Menkes disease	Stephen G. Kaler, MD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	rt-PA	Activase	10/20/2014	Treatment of plastic bronchitis	Kathleen A Stringer, PharmD, FCCP - Professor
	rubitecan	n/a	7/17/2002	Treatment of pediatric patients infected with human immunodeficiency virus and acquired immunodeficiency syndrome	SuperGen, Inc.
	rucaparib	n/a	7/31/2012	Treatment of ovarian cancer	Clovis Oncology, Inc.
	rufinamide	Banzel	10/8/2004	Treatment of Lennox-Gastaut Syndrome.	Eisai, Inc.
	ruxolitinib	Jakafi	8/16/2013	Treatment of pancreatic cancer	Incyte Corporation
	ruxolitinib	Jakafi	3/26/2010	Treatment of polycythemia vera	Incyte Corporation
	ruxolitinib phosphate	Jakafi	9/5/2008	Treatment of myelofibrosis	Incyte Corporation
	ruxolitinib phosphate	Jakafi	3/22/2010	Treatment of essential thrombocythemia	Incyte Corporation
	sacituzumab govitecan	n/a	5/29/2014	Treatment of pancreatic cancer.	Immunomedics, Inc.
	sacituzumab govitecan	n/a	11/27/2013	Treatment of small cell lung cancer	Immunomedics, Inc.
	salicylic acid 6%	n/a	2/17/2012	Treatment of rare congenital ichthyoses.	Orenova Group, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	salirasib	n/a	12/18/2006	Treatment of pancreatic cancer.	Kadmon Corporation, LLC
	salmeterol xinafoate/fluticasone propionate	n/a	10/29/2009	Treatment of symptomatic exophthalmos associated with thyroid related eye disease	Lithera, Inc.
	saposin C	n/a	2/3/2015	Treatment of glioblastoma multiforme	Bexion Pharmaceuticals, LLC
	sapropterin	Kuvan	1/29/2004	Treatment of hyperphenylalaninemia	BioMarin Pharmaceutical, Inc.
	sarcosine	n/a	10/12/2011	Treatment of obsessive compulsive disorder in pediatric patients (0 to 16 years of age)	Guochuan Emil Tsai, MD, PhD
	sarizotan	n/a	7/7/2015	Treatment of Rett syndrome.	Newron Pharmaceuticals US, Inc.
	sdTD-K6a.513a.12; small interfering RNA composed of 2 strands of hybridized RNAs	n/a	4/15/2013	Treatment of pachyonychia congenita	TransDerm, Inc.
	sebelipase alfa	n/a	7/1/2010	Treatment of lysosomal acid lipase deficiency	Synageva BioPharma Corp.
	selective antagonist of the chemokine receptor type 4	n/a	9/5/2013	Treatment of acute myeloid leukemia	BioLineRx, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	selective deacylglycerol acyltransferase 1 inhibitor	n/a	3/28/2011	Treatment of hypertriglyceridemia in the setting of Type I hyperlipoproteinemia, also known as Familial Chylomicronemia Syndrome	Novartis Pharmaceuticals Corporation
	selective inhibitor of fungal lanosterol demethylase	n/a	8/19/2014	Treatment of cryptococcal meningitis	Viamet Pharmaceuticals, Inc.
	selexipag	n/a	4/30/2010	Treatment of pulmonary arterial hypertension	Actelion Ltd
	self-complementary adeno-associated virus vector, serotype 9, packaging the full length GAN gene in the viral capsid	n/a	9/27/2013	Treatment of Giant Axonal Neuropathy	Hannah's Hope Fund
	selinexor	n/a	1/5/2015	Treatment of multiple myeloma	Karyopharm Therapeutics, Inc.
	selisistat	n/a	12/7/2009	Treatment of Huntington's disease	Siena Biotech SpA
	selumetinib	n/a	4/15/2015	Treatment of uveal melanoma.	AstraZeneca Pharmaceuticals LP
	setmelanotide	n/a	9/21/2015	Treatment of Prader-Willi Syndrome.	Rhythm Metabolics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sevuparin	n/a	3/17/2015	Treatment of sickle cell disease	Dilaforette AB
	sialic acid	n/a	9/23/2011	Treatment of hereditary inclusion body myopathy.	Ultragenyx Pharmaceutical, Inc.
	sildenafil	Revatio	7/28/2011	Treatment of pediatric (defined as children less than 17 years of age) pulmonary arterial hypertension	Pfizer, Inc.
	silibinin-C-2',3-dihydrogensuccinate, disodium salt	Legalon(R) Sil	9/11/2014	Treatment of amatoxin poisoning, which includes the prevention and treatment of amatoxin induced hepatic failure	Meda Pharmaceuticals, Inc.
	siltuximab	Sylvant	5/26/2006	Treatment of Castleman's disease	Janssen Biotech, Inc.
	simtuzumab	n/a	1/5/2015	Treatment of primary sclerosing cholangitis	Gilead Sciences, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	single stranded, chemically modified oligonucleotide that binds to and inhibits the function of micro RNA-21	n/a	7/17/2014	Treatment of Alport syndrome	Regulus Therapeutics, Inc.
	siponimod	n/a	7/10/2014	Treatment of dermatomyositis	Novartis Pharmaceuticals Corporation
	sirolimus	n/a	11/17/2011	Treatment of lymphangioleiomyomatosis	Cote Orphan Consulting, LLC
	sirolimus	n/a	6/25/2014	Treatment of lymphangioleiomyomatosis	LAM Therapeutics, Inc.
	sirolimus	Rapamune	10/31/2012	Treatment of lymphangioleiomyomatosis	Pfizer, Inc.
	sirolimus	n/a	3/18/2013	Treatment of pachyonychia congenita	TransDerm, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sirolimus	n/a	11/4/2011	Treatment of chronic/refractory anterior noninfectious uveitis, noninfectious intermediate uveitis, noninfectious panuveitis and non-infectious, uveitis affecting the posterior of the eye (NICUPS).	Santen Inc.
	sirolimus in an implantable collagen matrix	Coll-R, Sirogen	5/10/2012	Prevention of arteriovenous(AV) fistula or AV graft failure in patients with end stage renal disease, receiving hemodialysis or preparing for hemodialysis	Vascular Therapies, LLC
	sitimagene ceradenovec	Cerepro	7/31/2001	Use with gancyclovir in the treatment of malignant glioma	Finvector Vision Therapies Ltd

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	skin tissue	Stratagraft	5/21/2012	Treatment of hospitalized patients with complex skin defects resulting from partial and full thickness skin burns requiring excision and grafting	Stratatech Corporation
	small molecule FGFR4 inhibitor	n/a	9/14/2015	Treatment of hepatocellular cancer (HCC).	Blueprint Medicines Corporation
	small molecule inhibitor of histone methyltransferase DOT1L	n/a	8/15/2013	Treatment of Acute Myeloid Leukemia	Epizyme Inc.
	small molecule inhibitor of histone methyltransferase DOT1L	n/a	8/8/2013	Treatment of acute lymphoblastic leukemia (ALL)	Epizyme Inc.
	small molecule inhibitor of phosphodiesterase 10	n/a	9/26/2013	Treatment of Huntington's disease	Omeros Corporation
	small molecule normalizing the p53 function	n/a	6/12/2014	Treatment of ovarian cancer	Critical Outcome Technologies, Inc.
	smilagenin	Cogane	7/21/2011	Treatment of amyotrophic lateral sclerosis	Junaxo, Inc.
	sobetirome	n/a	4/29/2011	Treatment of X-linked adrenoleukodystrophy	NeuroVia, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sodium 2, 2 dimethylbutyrate	n/a	7/25/2008	Treatment of sickle cell disease.	HemaQuest Pharmaceuticals, Inc.
	sodium 2-hydroxylinoleate	n/a	8/25/2015	Treatment of neuroblastoma	Ability Pharmaceuticals, SL
	sodium 4-phenylbutyrate	n/a	10/18/2011	Treatment of spinal muscular atrophy	GMP-Orphan SAS
	sodium 4-{ [9-chloro-7-(2-fluoro-6-methoxyphenyl)-5H-pyrimido [5,4-d] [2] benzazepin-2-yl]-2-methoxybenzoate	n/a	4/3/2009	Treatment of ovarian cancer	Millennium Pharmaceuticals, Inc.
	sodium ascorbate and menadione sodium bisulfite	Apatone(R)	4/15/2013	Treatment of autosomal dominant polycystic liver disease	IC-Medtech Corporation
	sodium ascorbate and menadione sodium bisulfite	Apatone	4/9/2015	Treatment of noninfected painful total joint without mechanical complication	IC-MedTech Corporation
	sodium ascorbate and menadione sodium bisulfite	Apatone	4/15/2013	Treatment of autosomal dominant polycystic kidney disease	IC-MedTech Corporation
	sodium benzoate and clozapine	n/a	12/22/2011	Treatment of treatment-resistant schizophrenia	Guochuan Emil Tsai, MD, PhD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sodium chlorite	n/a	8/22/2011	For slowing the progression of amyotrophic lateral sclerosis.	Neuraltus Pharmaceuticals, Inc.
	sodium dichloroacetate	n/a	7/3/2003	Use as an antidote in the management of systemic monochloroacetic acid poisoning	EBD Group
	sodium dichloroacetate	n/a	11/29/2010	For pulmonary arterial hypertension.	Peter W. Stackpoole, PhD, MD
	sodium fusidate	n/a	10/23/2013	Treatment of patients with prosthetic joint infections	Cempra Pharmaceuticals, Inc.
	sodium nitrite	n/a	7/8/2008	Treatment of pulmonary arterial hypertension	Airess Pharmaceuticals, Inc.
	sodium nitrite	n/a	9/3/2009	Prevention of ischemia reperfusion injury to donor organ tissue associated with solid organ transplantation	Hope Pharmaceuticals
	sodium nitrite	n/a	1/9/2012	Treatment of chlorine gas poisoning	Hope Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sodium nitrite and EDTA	n/a	11/10/2015	Treatment of Pseudomonas aeruginosa pulmonary infections in patients with cystic fibrosis	Arch Biopartners, Inc.
	sodium nitrite and sodium thiosulfate	Nithiodote	4/9/2008	Treatment of known or suspected cyanide poisoning	Hope Pharmaceuticals
	sodium phenylbutyrate	Buphenyl	11/22/1993	Treatment of urea cycle disorders: carbamylphosphate synthetase deficiency, ornithine transcarbamylase deficiency, and argininosuccinic acid synthetase deficiency.	Medicis Pharmaceutical Corp.
	sodium phenylbutyrate	n/a	7/2/1992	Treatment for sickling disorders, which include S-S hemoglobinopathy, S-C hemoglobinopathy, and S-thalassemia hemoglobinopathy.	Medicis Pharmaceutical Corp.
	sodium phenylbutyrate	Pheburane	6/6/2013	Treatment of urea cycle disorders	Lucane Pharma SA

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sodium phenylbutyrate	n/a	1/19/2010	Treatment of urea cycle disorder.	Navinta LLC
	sodium phenylbutyrate	n/a	8/19/2014	Treatment of maple syrup urine disease	Acer Therapeutics, Inc.
	sodium stibogluconate	n/a	10/28/2009	Treatment of cutaneous leishmaniasis	Surgeon General of the US Army
	sodium sulfate, potassium sulfate, and magnesium sulfate	Suprep	10/31/2012	For cleansing of the colon in preparation for colonoscopic diagnosis of colonic disease in children and adolescents	Braintree Laboratories, Inc.
	sodium sulfate, potassium sulfate, and magnesium sulfate; PEG-3350, sodium chloride, sodium bicarbonate and potassium chloride	Suclear	9/4/2013	For use in cleansing of the colon in preparation for colonoscopy in children and adolescents	Braintree Laboratories, Inc.
	sodium thiosulfate	n/a	12/2/2010	Treatment of extravasation of meclorethamine hydrochloride into subcutaneous tissues.	Hope Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sodium thiosulfate	n/a	11/9/2011	Treatment of uremic and non-uremic calciphylaxis	Hope Pharmaceuticals
	sodium thiosulfate	n/a	10/13/2011	Prevention of platinum-induced ototoxicity in pediatric patients	Hope Pharmaceuticals
	sodium thiosulfate	n/a	11/19/2012	Treatment of calciphylaxis	Edinburg BioQuarter
	sodium thiosulfate	n/a	3/3/2011	Treatment of sulfur mustard poisoning	Hope Pharmaceuticals
	sodium thiosulfate	n/a	10/28/2014	Treatment of dermatomyositis	Hope Pharmaceuticals
	sodium valproate	n/a	8/5/2015	Treatment of Wolfram syndrome	The University of Birmingham
	solvent/detergent treated non-blood-group specific human coagulation active plasma	Uniplas	12/12/2005	Treatment of thrombotic thrombocytopenic purpura	Octapharma USA, Inc.
	somatorelin	Somatrel	8/8/1989	Diagnostic measure of the capacity of the pituitary gland to release growth hormone.	Ferring Laboratories, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	somatropin [rDNA]	Genotropin	12/27/2000	Treatment of growth failure in children who were born small for gestational age.	Pharmacia and Upjohn Company
	sonidegib	n/a	3/23/2015	Treatment of medulloblastoma	Novartis Pharmaceuticals Corp.
	sorafenib	Nexavar	12/12/2011	Treatment of medullary thyroid cancer, anaplastic thyroid cancer, and recurrent or metastatic follicular or papillary thyroid cancer	Bayer HealthCare Pharmaceuticals, Inc.
	sotalol (IV)	So-Aqueous	7/25/2008	For ventricular tachycardia, ventricular fibrillation, or the maintenance of sinus rhythm in patients converted from atrial fibrillation or atrial flutter when oral administration is not possible.	Academic Pharmaceuticals

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sotalol hydrochloride	n/a	2/10/2014	Treatment of life-threatening ventricular arrhythmias in pediatric patients	Arbor Pharmaceuticals, LLC
	sotatercept	n/a	12/5/2013	Treatment of beta-thalassemia intermedia and major	Celgene Corporation
	sotatercept	n/a	4/28/2014	Treatment of anemias associated with myelodysplastic syndrome and myelodysplastic/myeloproliferative neoplasms.	Celgene Corporation
	sparsentan	n/a	1/5/2015	Treatment of Focal Segmental Glomerulosclerosis	Retrophin, LLC
	spherical carbon adsorbent	n/a	12/19/2007	Treatment of chronic pouchitis	Ocera Therapeutics, Inc.
	spironolactone	Aldactone	5/22/2014	Use in pediatric patients with primary hyperaldosteronism	CMP Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	squalamine lactate	n/a	5/11/2001	Treatment of ovarian cancer refractory or resistant to standard chemotherapy	Genaera Corporation
	staphylococcal aureus protein A	n/a	6/10/2015	Treatment of immune thrombocytopenic purpura	Protalex, Inc.
	stem/progenitor cells derived from ex vivo expanded allogeneic umbilical cord blood cells	Nicord(R)	11/13/2014	Treatment of myelodysplastic syndrome.	Gamida Cell Ltd.
	stem/progenitor cells derived from ex vivo expanded allogeneic umbilical cord blood cells	Nicord(R)	11/13/2014	Treatment of Hodgkin lymphoma.	Gamida Cell Ltd.
	stem/progenitor cells derived from ex vivo expanded allogeneic umbilical cord blood cells	Nicord(R)	11/13/2014	Treatment of acute lymphoblastic leukemia.	Gamida Cell Ltd.
	stiripentol	Diacomit	10/30/2008	Treatment of Dravet syndrome	Biocodex
	sulfamidase	n/a	5/22/2008	For treatment of Sanfilippo Syndrome (MPS IIIA)	Shire Human Genetic Therapies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	sulfonated monophosphorylated mannose oligosaccharide	n/a	2/17/2012	Treatment of hepatocellular carcinoma	Medigen Biotechnology Corporation
	sulthiame	n/a	7/25/2013	Treatment of patients with benign epilepsy of childhood with centrotemporal spikes (BECTS) also known as rolandic epilepsy	Marathon Pharmaceuticals, LLC
	superoxide dismutase, gliadin	Etr019	4/30/2010	Treatment of amyotrophic lateral sclerosis	Verius Limited
	synthetic 14-mer phosphorothioate antisense oligonucleotide directed against TGF-beta2 mRNA	n/a	6/4/2014	Prevention of scarring post glaucoma filtration surgery	Isarna Therapeutics GmbH
	synthetic double-stranded (hybridized duplex) ribonucleic acid oligonucleotide specific to hydroxyacid oxidase 1 gene	n/a	4/22/2015	Treatment of primary hyperoxaluria type 1	Dicerna Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	synthetic double-stranded siRNA oligonucleotide against antithrombin (AT) mRNA	n/a	8/16/2013	Treatment of hemophilia A	Alnylam Pharmaceuticals
	synthetic double-stranded siRNA oligonucleotide against antithrombin mRNA	n/a	8/12/2013	Treatment of hemophilia B	Alnylam Pharmaceuticals
	synthetic double-stranded siRNA oligonucleotide against p53 mRNA	n/a	12/23/2009	Prophylaxis of delayed graft function in renal transplant patients	Quark Pharmaceuticals, Inc.
	synthetic double-stranded siRNA oligonucleotide against transthyretin (TTR) mRNA	n/a	6/14/2012	Treatment of familial amyloidotic polyneuropathy	Alnylam Pharmaceuticals, Inc.
	synthetic oligomer of 16 nucleotides	n/a	1/13/2015	Treatment of myotonic dystrophy Type I	Isis Pharmaceuticals, Inc.
	synthetic peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	n/a	10/12/2011	Treatment of necrotizing soft tissue infections (NSTI)	Atox Bio, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	synthetic peptide; cyclo-Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-ALA-Lys-Pro-Trp-Tyr-Cys	n/a	1/16/2013	Treatment of high altitude pulmonary edema	Apeptico Forschung und Entwicklung GmbH
	synthetic signal peptide of human mucin-1 (amino acids 1-21)	n/a	6/16/2015	Treatment of multiple myeloma.	Vaxil Bio Therapeutics Ltd.
	synthetic surfactant comprised of DPPC, POPG Na, synthetic SP-C analogue and synthetic SP-B analogue	n/a	3/16/2012	Treatment of preterm neonatal respiratory distress syndrome	Chiesi USA, Inc.
	tabalumab	n/a	11/19/2012	Treatment of multiple myeloma	Eli Lilly and Company
	taberminogene vadenovec	Trinam	10/24/2000	Prevention of complications due to neointimal hyperplasia disease in certain vascular anastomoses.	Finvector Vision Therapies, Ltd.
	tacrolimus	n/a	3/16/2015	Treatment of pulmonary arterial hypertension.	Selten Pharma, Inc.
	tacrolimus	n/a	5/4/2015	Treatment of pulmonary arterial hypertension	Stanford University School of Medicine

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tacrolimus	n/a	7/6/2012	Treatment of hemorrhagic cystitis	Lipella Pharmaceuticals Inc.
	tacrolimus	Envarsus Xr	12/20/2013	Prophylaxis of organ rejection in patients receiving allogeneic kidney transplant	Veloxis Pharmaceuticals, Inc.
	tadalafil	Adcirca	12/18/2006	Treatment of pulmonary arterial hypertension	Eli Lilly and Company
	tadalafil	Cialis(R); Adcirca(R)	5/4/2015	Treatment of Duchenne Muscular Dystrophy (DMD)	Eli Lilly and Company
	tafamidis	n/a	5/23/2006	Treatment of familial amyloid polyneuropathy	Pfizer, Inc.
	tafamidis meglumine	n/a	2/17/2012	Treatment of symptomatic transthyretin (TTR) amyloid cardiomyopathy.	Pfizer, Inc.
	tafenoquine	n/a	1/15/2013	Treatment of malaria	Glaxo Group Limited, England
	talactoferrin alfa	n/a	8/20/2003	For the prevention of graft-versus-host disease	Agennix, Inc.
	talactoferrin alfa	n/a	8/20/2003	For the treatment of graft versus host disease	Agennix, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	talarazole	n/a	3/16/2012	Treatment of congenital ichthyosis	Stiefel Laboratories, Inc.
	talimogene laherparepvec	n/a	3/14/2011	Treatment of stage IIb-stage IV melanoma	BioVex, Inc. (subsidiary of Amgen)
	tarextumab	n/a	1/26/2015	Treatment of small cell lung cancer	OncoMed Pharmaceuticals, Inc.
	tarextumab	n/a	1/26/2015	Treatment of pancreatic cancer	OncoMed Pharmaceuticals, Inc.
	tasimelteon	n/a	4/30/2010	Treatment of sleep-wake disorder in Smith-Magenis syndrome associated with diurnal melatonin secretion	Vanda Pharmaceuticals, Inc.
	tasimelteon	Hetlioz	1/19/2010	Non-24-hour sleepwake disorder in blind individuals without light perception	Vanda Pharmaceuticals, Inc.
	taurine	n/a	3/22/2010	Treatment of cystathionine beta-synthase deficient homocystinuria	Johan L. Van Hove, MD, PhD

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	technetium Tc 99m tilmanocept	Lymphoseek	9/17/2014	Use in sentinel lymph node detection (SLN) with a hand-held gamma-counter, with scintigraphic imaging, in patients with cancer of the head and neck	Navidea Biopharmaceuticals
	tecovirimat	n/a	12/18/2006	post exposure prophylaxis against smallpox	SIGA Technologies, Inc.
	tecovirimat	n/a	12/27/2006	Treatment of smallpox.	SIGA Technologies, Inc.
	tecovirimat	n/a	9/29/2010	Treatment of orthopoxvirus infections.	SIGA Technologies, Inc.
	teduglutide [rDNA origin]	Gattex	6/29/2000	Treatment of short bowel syndrome.	NPS Pharmaceuticals, Inc.
	telatinib	n/a	5/17/2010	Treatment of gastric cancer	ACT Biotech Inc.
	temocillin sodium	Negaban	4/21/2004	Treatment of pulmonary infections caused by Burkholderia cepacia	Belpharma S.A.
	temozolomide	Temodar	10/5/1998	Treatment of recurrent malignant glioma.	Schering-Plough Research Institute
	temozolomide	n/a	11/25/2015	Treatment glioblastoma multiforme in pediatric patients	AmpliPharm Pharmaceuticals, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tenofovir	Viread	3/17/2009	Treatment of pediatric HIV infection.	Gilead Sciences, Inc.
	teprotumumab	n/a	5/6/2013	Treatment of active (dynamic) phase Grave's orbitopathy	River Vision, Inc.
	terguride	Mysalfon, Teluron	5/17/2013	Treatment of systemic sclerosis	Serodapharm UG
	teriparatide	Forteo	4/18/2014	Treatment of hypoparathyroidism	Entera Bio, Ltd.
	terlipressin	n/a	10/29/2004	Treatment of Hepatorenal Syndrome	Ikaria (INO Therapeutics)
	tesetaxel	n/a	12/22/2008	Treatment of gastric cancer	Genta Inc.
	tesetaxel	n/a	11/21/2008	Treatment of stages IIB, IIC, III, and stage IV melanoma	Genta, Inc.
	testosterone undecanoate (oral)	n/a	2/13/2013	Treatment of constitutional delay in growth and puberty in adolescent boys (14-17 yrs of age)	SOV Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tetra substituted porphyrin derivative containing manganese (III)	n/a	1/17/2014	For use in patients exposed to radiation following a nuclear accident or detonation in order to treat or mitigate acute radiation syndrome.	Aeolus Pharmaceuticals, Inc.
	tetra-substituted porphyrin derivative containing manganese (III)	n/a	3/16/2015	Treatment of idiopathic pulmonary fibrosis	Aeolus Pharmaceuticals
	tetrabenazine	n/a	7/1/2009	Treatment of Tourette's Syndrome in school-age children, ages 5-16	Valeant International (Barbados) SRL
	tetracosactide hexaacetate (beta 1-24-corticotrophin)	Synacthen Depot, S. Retard	10/31/2012	Treatment of infantile spasms	Cerium Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	thalidomide	n/a	9/19/1988	1. Treatment of graft versus host disease in patients receiving bone marrow transplantation 2. Prevention of graft versus host disease in patients receiving bone marrow transplantation	Pediatric Pharmaceuticals, Inc.
	theranost 68Ga-RGD	n/a	12/8/2014	Diagnostic for clinical management of patients with neuroblastoma	Advanced Imaging Projects, LLC
	thioureidobutyronitrile	Kevetrin	11/17/2015	Treatment of retinoblastoma	Cellceutix Corporation
	thioureidobutyronitrile	Kevetrin	7/14/2015	Treatment of ovarian cancer	Cellceutix Corporation
	thymopentin	n/a	2/4/2011	Treatment of sarcoidosis.	Mondobiotech Laboratories AG
	thymosin beta 4	n/a	12/31/2013	Treatment of patients with neurotrophic keratopathy	ReGenTree, LLC
	tigecycline	n/a	10/23/2013	Treatment of acute myeloid leukemia.	Trillium Therapeutics, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tin ethyl etiopurpurin	n/a	11/4/2003	Prevention of access graft disease in hemodialysis patients	Miravant Medical Technologies
	tinidazole	Tindamax	4/18/2002	Treatment of giardiasis	Presutti Laboratories, Inc.
	tipelukast	n/a	10/20/2014	Treatment of idiopathic pulmonary fibrosis	MediciNova, Inc.
	tiprelestat	Elafin	12/28/2012	Treatment of pulmonary arterial hypertension	Proteo Biotech AG
	tiptorelin pamoate	n/a	8/20/2012	Treatment of central precocious puberty	Debiopharm
	tirapazamine	n/a	10/23/2002	Treatment of head and neck cancer	Sanofi-Aventis US, Inc.
	tirasemtiv	n/a	3/2/2010	Treatment of amyotrophic lateral sclerosis (ALS)	Cytokinetics Inc.
	tisagenlecleucel- T	n/a	2/3/2015	Treatment of diffuse large B- cell lymphoma	Novartis Pharmaceuticals Corporation
	tivantinib	n/a	10/16/2013	Treatment of hepatocellular carcinoma	Daiichi Sankyo Pharma Development
	tocilizumab	Actemra	4/17/2013	Treatment of systemic sclerosis	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tocilizumab	Actemra	7/31/2012	Treatment of pediatric patients (age 16 years and younger) with polyarticular-course juvenile idiopathic arthritis	Genentech, Inc.
	tolerogen	n/a	2/5/2015	Treatment of myasthenia gravis	Toleranzia AB
	tolvaptan	Samsca	4/6/2012	Treatment of autosomal dominant polycystic kidney disease	Otsuka Pharmaceuticals Co., Ltd.
	topiramate injection	n/a	7/24/2013	Treatment of partial onset or primary generalized tonic-clonic seizures for hospitalized epilepsy patients or epilepsy patients being treated in an emergency care setting who are unable to take oral topiramate	CURx Pharmaceuticals, Inc.
	trabectedin	Yondelis	3/29/2005	Treatment of patients with ovarian cancer	Janssen Research & Development, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	trabectedin	Yondelis	9/30/2004	Treatment of soft tissue sarcoma	Janssen Research & Development, LLC
	trabedersen	Oncomun	6/5/2002	Treatment of malignant glioma	Isarna Therapeutics GmbH
	trabedersen	n/a	8/22/2011	Treatment of Stage IIB through Stage IV malignant melanoma.	Isarna Therapeutics GmbH
	trabedersen	n/a	7/21/2009	Treatment of pancreatic cancer.	Isarna Therapeutics GmbH
	tralokinumab	n/a	7/24/2012	Treatment of idiopathic pulmonary fibrosis	MedImmune Ltd.
	trametinib	Mekinist	12/20/2010	Treatment of Stage IIb through Stage IV melanoma	Novartis Pharmaceuticals Corp.
	trametinib and dabrafenib	Mekinist And Tafinlar	9/20/2012	Treatment of Stage IIb through IV melanoma.	Novartis Pharmaceuticals Corp.
	tranilast	Rizaben	12/23/2010	Prevention of scarring following glaucoma filtration surgery	Altacor Ltd
	trans sodium crocetinate	n/a	7/21/2011	Treatment of glioblastoma in conjunction with radiotherapy	Diffusion Pharmaceuticals, LLC
	trans sodium crocetinate	n/a	12/3/2012	Treatment of brain metastasis	Diffusion Pharmaceuticals, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	transforming growth factor-beta receptor 1 kinase inhibitor	n/a	3/11/2013	Treatment of glioma	Eli Lilly and Company
	transforming growth factor-beta receptor 1 kinase inhibitor	n/a	4/1/2013	Treatment of hepatocellular carcinoma	Eli Lilly and Company
	trastuzumab	Herceptin	12/14/1999	Treatment of patients with pancreatic cancer that overexpress p185HER2.	Genentech, Inc.
	trastuzumab	Herceptin	10/13/2009	Treatment of HER2-overexpressing advanced adenocarcinoma of the stomach, including gastroesophageal junction	Genentech, Inc.
	trastuzumab emtansine	Kadcyla Kadcyla Kadcyla Kadcyla Kadcyla®	10/25/2013	For the treatment of gastric cancer including gastroesophageal junction cancer.	Genethetech, Inc.
	trebananib	n/a	8/30/2013	Treatment of ovarian cancer	Amgen, Inc.
	trehalose	Cabaletta	10/25/2013	Treatment of oculopharyngeal muscular dystrophy	BIOBLAST PHARMA LTD.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	trehalose	n/a	11/17/2014	Treatment of spinal cerebellar ataxia type 3 (also known as SCA3 or Machado Joseph disease)	BioBlast Pharma Ltd
	tremelimumab	n/a	3/18/2015	Treatment of malignant mesothelioma	MedImmune, LLC
	tremelimumab	n/a	9/18/2006	Treatment of stage IIb to stage IV metastatic melanoma	MedImmune
	treosulfan	n/a	4/8/2015	Conditioning treatment prior to hematopoietic stem cell transplantation (HSCT) in malignant and non-malignant diseases in adults and pediatric patients.	Medac GmbH
	treprostinil	Remodulin	6/4/1997	Treatment of pulmonary arterial hypertension.	United Therapeutics Corp.
	treprostinil (inhalational)	Tyvaso	6/17/2010	Treatment of pulmonary arterial hypertension	LungRx, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	tri-antennary glycotriptide derivative of 5-fluorodeoxyuridine monophosphate	n/a	11/23/2001	Treatment for hepatocellular carcinoma	Cell Works Inc.
	tricitabine	n/a	2/1/2008	Treatment of multiple myeloma	VioQuest Pharmaceuticals, Inc.
	trientine hydrochloride	Syprine(R)	11/2/2010	Treatment of manganism.	Cerberus Princeton, LLC
	trifarotene	n/a	6/6/2014	Treatment of congenital ichthyosis	Galderma R&D, LLC
	trihexanoin	n/a	10/21/2014	Treatment of glucose transporter type-1 deficiency syndrome	Ultragenyx Pharmaceutical, Inc.
	trihexanoin	n/a	4/15/2015	Treatment of fatty acid oxidation disorders	Ultragenyx Pharmaceutical, Inc.
	triterpenoid saponin	n/a	7/23/2014	Treatment of mantle cell lymphoma	Avicin Therapeutics, Ltd.
	tryptophan hydroxylase (TPH) inhibitor	n/a	3/9/2012	Management of symptoms of carcinoid syndrome associated with carcinoid tumor	Lexicon Pharmaceuticals, Inc.
	tumor-infiltrating lymphocytes; LN-144	n/a	6/9/2015	Treatment of malignant melanoma Stages IIb to IV	Lion Biotechnologies, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ubenimex	n/a	11/19/2015	Treatment of pulmonary arterial hypertension.	Eiger BioPharmaceuticals, Inc.
	ubiquinol	Ubi-Q-NoI, Li-Q-NoI	4/12/2004	Treatment of Huntington's Disease	Gel-Tec, Division of Tishcon Corp.
	ublituximab	n/a	8/6/2010	Treatment of chronic lymphocytic leukemia	TG Therapeutics, Inc.
	ublituximab	n/a	9/5/2013	Treatment of Extranodal marginal zone lymphoma (mucosa-associated lymphatic tissue, MALT)	TG Therapeutics, Inc.
	ublituximab	n/a	9/5/2013	Treatment of Nodal marginal zone lymphoma	TG Therapeutics, Inc.
	udenafil	n/a	8/31/2015	Treatment of single ventricle congenital heart disease with Fontan physiology	Mezzion Pharma Co. Ltd.
	ulocuplumab	n/a	1/12/2015	Treatment of acute myeloid leukemia	Bristol-Myers Squibb Company
	unoprostone isopropyl	Rescula	9/16/2010	Treatment of retinitis pigmentosa	R-Tech Ueno, Ltd.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	urea	n/a	11/7/2011	rare congenital ichthyoses (CHILD syndrome, collodion baby, congenital ichthyosiform erythroderma, Conradi-flunermann, epidermolytic hyperkeratosis, erythrokeratoderma variabilis, harlequin ichthyosis, KID syndrome, lamellar ichthyosis, Netherton syndrome, neutral lipid storage disease, Sjorgren-Larsson	Orenova Group, LLC
	uridine triacetate	n/a	5/1/2009	An antidote in the treatment of 5-fluorouracil poisoning	Wellstat Therapeutics Corp.
	uridine triacetate	n/a	9/3/2009	Treatment of mitochondrial disease	Wellstat Therapeutics Corporation
	uridine triacetate	n/a	8/9/2013	Treatment of hereditary orotic aciduria	Wellstat Therapeutics, Inc.
	ursodiol	Ursofalk Suspension	10/23/2007	Treatment of cystic fibrosis liver disease	Asklepiion Pharmaceuticals, LLC

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	ustekinumab	Stelara	11/29/2010	Treatment of type 1 diabetes mellitus patients with residual beta-cell function.	Johnson & Johnson Pharmaceutical & Development LLC
	vadastuximab talirine	n/a	11/10/2015	Treatment of acute myeloid leukemia.	Seattle Genetics, Inc.
	valine-valine-ganciclovir	n/a	5/21/2007	Treatment of primary keratoconjunctivitis and recurrent epithelial keratitis due to HSV1 and HSV2.	Verenta Pharmaceuticals, Inc.
	valsartan oral solution	n/a	10/28/2015	Treatment of hypertension in pediatric patients 0 through 16 years of age	Carmel Biosciences
	vancomycin	n/a	12/27/2012	Treatment of endophthalmitis	Fera Pharmaceuticals, LLC
	vancomycin hydrochloride (inhalational)	n/a	9/20/2012	Treatment of persistent methicillin-resistant S. aureus lung infection in patients with cystic fibrosis	Savara Pharmaceuticals, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	vandetanib	Caprelsa(R)	10/21/2005	Treatment of patients with follicular thyroid carcinoma, medullary thyroid carcinoma, anaplastic thyroid carcinoma, and locally advanced and metastatic papillary thyroid carcinoma	AstraZeneca Pharmaceutical LP
	vapreotide	Octastatin	1/10/2000	Treatment of gastrointestinal and pancreatic fistulas.	Debiopharm S.A.
	vapreotide	Sanvar	1/10/2000	Treatment of esophageal variceal hemorrhage patients with portal hypertension.	Debiovision, Inc.
	vapreotide	Octastatin	3/6/2000	Prevention of early postoperative complications following pancreatic resection.	Debiopharm S.A.
	vapreotide	Sanvar	11/4/2003	Treatment of acromegaly	H3 Pharma, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016**  
**Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	vascular endothelial growth factor 165b	n/a	6/24/2008	Treatment of advanced melanoma stages IIb through IV.	PhiloGene, Inc.
	vasoactive intestinal peptide (VIP)-elastin-like peptide (ELP) fusion protein	n/a	5/13/2014	Treatment of pulmonary arterial hypertension, WHO Group 1	PhaseBio Pharmaceuticals, Inc.
	vatiquinone	Vincerinone	6/4/2014	Treatment of Leigh Syndrome	Edison Pharmaceuticals, Inc.
	vatiquinone	Vincerinone	11/17/2014	Treatment of Rett syndrome	Edison pharmaceuticals, Inc.
	vatiquinone	Vincerinone	1/31/2014	Treatment of Friedreich's ataxia	Edison pharmaceuticals, Inc.
	velaglucerase-alfa	Vpriv	6/8/2009	Treatment of Gaucher disease	Shire Human Genetics Therapies, Inc.
	veliparib	n/a	12/17/2014	Treatment of brain metastases when used in combination with DNA-damaging agents	AbbVie Inc.
	veliparib	n/a	5/9/2008	Treatment of glioblastoma multiforme when used in combination with DNA-damaging agents	AbbVie, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	veliparib	n/a	11/20/2009	Treatment of hepatocellular carcinoma in combination with DNA-damaging agents	AbbVie, Inc.
	veliparib	n/a	9/3/2009	Treatment of epithelial ovarian cancer in combination with DNA-damaging agents	AbbVie, Inc.
	veltuzumab	n/a	11/17/2014	Treatment of pemphigus	Immunomedics, Inc.
	veltuzumab	n/a	7/28/2015	Treatment of immune thrombocytopenic purpura.	Immunomedics, Inc.
	veltuzumab	n/a	8/28/2008	Treatment of chronic lymphocytic leukemia	Immunomedics, Inc.
	vemurafenib	Zelboraf	11/26/2013	Treatment of anaplastic thyroid carcinoma and advanced papillary thyroid cancer whose tumors harbor a BRAF V600 mutation	Genentech, Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	vemurafenib	Zelboraf	8/26/2014	Treatment of hairy cell leukemia (HCL).	Genentech, Inc.
	vemurafenib	Zelboraf	12/20/2010	Treatment of patients with IIb to Stage IV melanoma positive for the BRAF(v600) mutation	Hoffmann-La Roche, Inc.
	vemurafenib	Zelboraf	9/8/2014	Treatment of patients with non-small cell lung cancer (NSCLC) with BRAF V600E mutation	Genentech, Inc.
	verteporfin	Visudyne(R)	3/9/2012	Treatment of chronic or recurrent central serous chorioretinopathy	Valeant Pharmaceuticals North America LLC
	vibriolysin	Vibrilase	6/16/2006	Debridement of severe, deep dermal burns in hospitalized patients	BioMarin Pharmaceutical Inc.
	vigabatrin	Sabril	6/12/2000	Treatment of infantile spasms.	H. Lundbeck A/S
	vinCRIS <sup>te</sup> sulfate LIPOSOME injection	Marqibo	1/8/2007	Treatment of acute lymphoblastic leukemia	Talon Therapeutics, Inc.
	vincristine sulfate liposomes	Marqibo	6/24/2008	Treatment of metastatic uveal melanoma.	Talon Therapeutics

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	vintafolide	n/a	12/16/2013	Treatment of ovarian cancer	Endocyte, Inc.
	vitamin A palmitate	n/a	7/14/2015	Prevention of bronchopulmonary dysplasia.	Advent Therapeutics, Inc.
	volanesorsen sodium, apolipoprotein C-III antisense oligonucleotide	n/a	6/23/2015	treatment of familial chylomicronemia syndrome	Isis Pharmaceuticals, Inc.
	volasertib	n/a	4/14/2014	Treatment of acute myeloid leukemia	Boehringer Ingelheim Pharmaceuticals, Inc.
	von Willebrand Factor Human Concentrate	Wilfactin	5/29/2014	Treatment of Von Willebrand Disease	rEVO Biologics, Inc.
	vorinostat	Zolinza	3/16/2004	Treatment of T-cell non-Hodgkin's lymphoma	Merck & Co., Inc.
	vosaroxin	n/a	10/28/2009	Treatment of acute myeloid leukemia	Sunesis Pharmaceuticals, Inc.
	xenon gas	n/a	5/18/2015	To improve neurological outcome in hospitalized cardiac arrest patients.	Neuroprotexon
	xenon gas	n/a	12/3/2014	Treatment of hypoxic ischemic encephalopathy	Neuroprotexon
	zidovudine	Retrovir	7/17/1985	Treatment of AIDS	Glaxo Wellcome Inc.

**Orphan Drug Designations and Approvals List as of 12-01-2016  
Governs January 1, 2016 - March 31, 2016**

Row Num	Generic Name	Trade Name	Designation Date	Designation	Contact Company
	zoledronate D,L-lysine monohydrate (ZLM)	n/a	4/15/2015	Treatment of complex regional pain syndrome (CRPS)	Thar Pharmaceuticals
	zoledronic acid	Zometa, Reclast, Aclasta	5/6/2013	Treatment of complex regional pain syndrome (CRPS).	Axsome Therapeutics, Inc.
	{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl](3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide	n/a	4/10/2014	Treatment of ovarian cancer	VentiRx Pharmaceuticals, Inc.
		Dysport(R)	12/5/1991	Treatment of essential blepharospasm	Ipsen Biopharmaceuticals, Inc.
		Nicord	4/28/2014	For the treatment of acute myeloid leukemia	Gamida Cell Ltd
		Biothrax	4/11/2014	For post-exposure prophylaxis of anthrax disease resulting from suspected or confirmed Bacillus anthracis exposure	Emergent Product Development Gaithersburg, Inc.