



ACHDNC
*Secretary's Advisory Committee
on Heritable Disorders in
Newborns and Children*

Advisory Committee on Heritable Disorders in Newborns and Children Meeting

August 1-2, 2019

Cynthia M. Powell, M.D.
Professor of Pediatrics and Genetic



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ACHDNC Business

- **Welcome**
- **Roll Call**
- **Vote – April 2019 Minutes**



NEW ORGANIZATION REPRESENTATIVES

Association of Women's Health, Obstetric & Neonatal Nurses

Jacqueline Rychnovsky, PhD, RN, CPNP, FAANP

Vice President, Research, Policy and Strategic
Initiatives, AWHONN



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NEW ORGANIZATION REPRESENTATIVES

Child Neurology Society

Jennifer M. Kwon, MD MPH FAAN

Director, Pediatric Neuromuscular Program

American Family Children's Hospital

Professor of Child Neurology, University of Wisconsin School of Medicine & Public Health



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THANK YOU

OUTGOING ORGANIZATION REPRESENTATIVES

**American College of
Obstetricians & Gynecologists**
Britton Rink, MD, MS
Mount Carmel Health Systems



Society for Inherited Metabolic Disorders
Shawn E. McCandless, MD
Section Head, Genetics and Metabolism
Children's Hospital Colorado



Department of Defense
COL Adam Kanis, MD, PhD, FACMG
Lieutenant Colonel, Medical Corps, US Army
Consultant to the (Army) Surgeon General
for Clinical Genetics, Department of
Pediatrics, Tripler Army Medical Center



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Children's

NEW ORGANIZATION REPRESENTATIVE

AMERICAN COLLEGE OF OBSTETRICIANS & GYNECOLOGISTS

Steven J. Ralston, MD, MPH
Chair, OB/GYN
Pennsylvania Hospital



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NEW ORGANIZATION REPRESENTATIVE SOCIETY FOR INHERITED METABOLIC DISORDERS

Georgianne Arnold, MD

Clinical Research Director, Division of Medical Genetics
UPMC Children's Hospital of Pittsburgh



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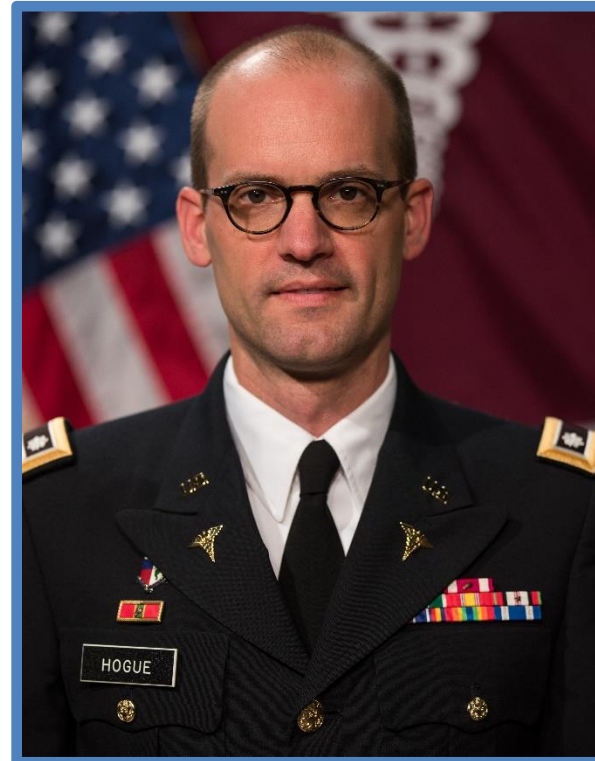


NEW ORGANIZATION REPRESENTATIVE

DEPARTMENT OF DEFENSE

Jacob Hogue, MD

Lieutenant Colonel, Medical Corps, US Army
Chief, Genetics of the Madigan Army Medical Center



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ACHDNC REPORT

MEDICAL FOODS FOR INBORN ERRORS OF METABOLISM: THE CRITICAL NEED TO IMPROVE PATIENT ACCESS



FUTURE MEETINGS DATES

- **November 7-8, 2019 (in person and webcast)**
- **February 13-14, 2020**

- **Meeting dates through 2023 can be found on the Committee's website:**

<https://www.hrsa.gov/advisory-committees/heritable-disorders/meetings/index.html>



MEETING TOPICS

Thursday, August 1, 2019

- Improving Detection of Newborns at Risk for Homocystinuria and Congenital Adrenal Hyperplasia
- Public Comments related to the RUSP Condition Nomination and Evidence Review Process
- Presentations and Committee Discussion on the RUSP Condition Nomination and Evidence Review Process



MEETING TOPICS

Friday, August 2, 2019

- International Rare Disease Research Consortium (IRDiRC)
- Implementation of RUSP Conditions Report
- Linking Data Resources
- Public Comments
- Workgroup Updates



Workgroup Meetings

1. Discuss current gaps in the field, topics or issues the Workgroup(s) could help address, and specific projects ideas.

– Next steps:

- August Meeting: present summary of the discussion, including ideas for topics or projects, to the Committee tomorrow
- November Meeting: the Committee will consider ideas and next steps for the Workgroups (e.g. new charges).

2. Feedback on the components of the RUSP condition nomination and evidence review process discussed at today's meeting.



Advisory Committee on Heritable Disorders in Newborns and Children

- Established under the Public Health Service Act, Title XI, § 1109 (42 U.S.C. 300b-10), as amended by the Newborn Screening Saves Lives Reauthorization Act of 2014 (P.L. 113-240)
- All federal committees governed by the Federal Advisory Committee Act (FACA), Public Law 92-463, as amended (5 U.S.C. App. 2), and by 41 C.F.R. Parts 101-6 and 102-3
- FACA sets the standards for the establishment, utilization, and management of Federal Advisory committees

Ethics and Conflicts of Interest (CIOs)

- **Advisory to the Secretary of HHS**
- **Inquiries about the Committee**
- **When to recuse:**
 - In all matters likely to affect the financial interests of any organization with which you serve as an officer, director, trustee, or general partner unless you are also an employee of the organization or unless you have received a waiver from HHS authorizing them to participate.

Meeting Participation

- All Committee meetings are open to the public.
- If the public wish to participate in the discussion, the procedures for doing so are published in the Federal Register and announced at the opening of a meeting.
- The Notice of Meeting in Federal Register indicated two options for public comment:
 - Oral Comments – 6 requests received
 - Submit written statements – 1 written statement received
 - Committee members receive copies of all written statements submitted
- Any further public participation will be solely at the discretion of the Chair and DFO



5600 Fishers Lane Building Info

- **Visitors only have access the Pavilion, cafeteria, restrooms, and meeting rooms.**
 - All areas of the facility are restricted and require an escort by a HRSA staff member. No exceptions.
- **If you need to leave and re-enter, you will be required to go through security screening again and will require a HRSA escort to meet you at Security to escort you in the building.**
 - 15 minutes prior to and after the lunch break ends a HRSA escort will be at the main entrance for those who need to leave and return.
 - For other re-entry needs, please notify a HRSA staff member or a registration team member.



5600 Fishers Lane Building Info

- Visitors are not allowed to take any video or photography in the building.
- In case of emergency please exit through the front door of 5600 Fishers Lane, cross the street, and meet on the parking pad to the left. Your escorts will have a roster and ensure everyone is accounted for.
- Please do not take any non-essential items with you if there is an evacuation, as it could delay your reentry into the building.

