### Agenda: Tuesday, November 9, 2021 from 10:00 AM – 2:00 PM Eastern Time

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<th>TIME</th>
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| 10:00 AM – 10:20 AM | • Welcome  
                   • Roll Call  
                   • Opening Remarks  
                   • Committee Business | Cynthia M. Powell, MD, MS, FACMG, FAAP  
                                      Committee Chair  
                                      Mia Morrison, MPH  
                                      Designated Federal Official  
                                      Health Resources and Services Administration |
| 10:20 AM – 11:35 AM | Mucopolysaccharidosis Type II Evidence-Based Review – Phase 2 Update | Alex R. Kemper, MD, MPH, MS  
                                      Lead, Evidence-Based Review Group  
                                      Lisa A. Prosser, PhD  
                                      Member, Evidence-Based Review Group |
| 11:35 AM – 11:50 AM | Overview of Immediately Actionable Committee Process Updates:  
                   • Condition nomination form  
                   • Methods for assessing published and unpublished evidence, reporting cost estimates  
                   • Decision matrix guidance | Cynthia M. Powell, MD, MS, FACMG, FAAP  
                                      Committee Chair |
| 11:50 AM – 12:05 PM | • Committee Discussion  
                   • **Vote on whether or not to approve immediately actionable updates** | Cynthia M. Powell, MD, MS, FACMG, FAAP  
                                      Committee Chair |
| 12:05 PM – 12:15 PM | Review of the Committee’s Evidence-Based Review and Decision-Making Processes: Recap of Key Issues Identified for Future Consideration | Cynthia M. Powell, MD, MS, FACMG, FAAP  
                                      Committee Chair |
| 12:15 PM – 12:45 PM | Break | |
| 12:45 PM – 1:15 PM | Public Comment | |
| 1:15 PM – 2:00 PM | HRSA Newborn Screening Portfolio Evaluation: Current and Future Needs of the Newborn Screening System | Melissa Raspa, PhD  
                                      Senior Scientist and Director  
                                      Genomics, Ethics and Translational Research Program  
                                      RTI International |
| 2:00 PM | End of Day 1 |
The Advisory Committee on Heritable Disorders in Newborns and Children

WEBINAR
November 9-10, 2021

Workgroup Meetings: Tuesday, November 9, 2021 from 3:00 PM – 4:30 PM Eastern Time

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| 3:00 PM – 4:30 PM | • Education and Training Workgroup  
|               | • Follow-up and Treatment Workgroup            
|               | • Laboratory Standards and Procedures Workgroup |

Agenda: Wednesday, November 10, 2021 from 10:00 AM – 1:15 PM Eastern Time

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| 10:00 AM – 10:10 AM | • Welcome  
|                 | • Roll Call                                     | Cynthia M. Powell, MD, MS, FACMG, FAAP  
|                 |                                                | Committee Chair  
|                 |                                                | Mia Morrison, MPH  
|                 |                                                | Designated Federal Official  
|                 |                                                | Health Resources and Services Administration  |
| 10:10 AM – 10:55 AM | Guanidinoacetate Methyltransferase (GAMT) Deficiency Evidence-Based Review – Phase 1 Update | Alex R. Kemper, MD, MPH, MS  
|                 |                                                | Lead, Evidence-Based Review Group  |
| 10:55 AM – 11:05 AM | Education and Training Workgroup Update        | Jane M. DeLuca, PhD, RN, CPNP  
|                 |                                                | Committee Member  
|                 |                                                | Chair, Education and Training Workgroup  |
| 11:05 AM – 11:15 AM | Follow-up and Treatment Workgroup Update       | Jeffrey P. Brosco, MD, PhD  
|                 |                                                | Committee Member  
|                 |                                                | Chair, Follow-up and Treatment Workgroup  |
| 11:15 AM – 11:25 AM | Laboratory Standards and Procedures Workgroup Update | Kellie B. Kelm, PhD  
|                 |                                                | Ex-Officio Committee Member  
|                 |                                                | Chair, Laboratory Standards and Procedures Workgroup  |
| 11:25 AM – 11:40 AM | Discussion: Workgroup Ideas                  | Cynthia M. Powell, MD, MS, FACMG, FAAP  
|                 |                                                | Committee Chair  |
| 11:40 AM – 12:00 PM | Break                                          | Melissa Wasserstein, MD  
|                 |                                                | Chief, Division of Pediatric Genetic Medicine  
|                 |                                                | Professor of Pediatrics and Genetics  
|                 |                                                | The Children's Hospital at  |
| 12:00 PM – 1:00 PM | Newborn Screening Pilot Programs               |                                           |
|                 | • ScreenPlus – New York                        |                                           |
|                 | • Early Check – North Carolina                 |                                           |
## Webinar
November 9-10, 2021

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|              |                | Montefiore  
The University Hospital for Albert Einstein College of Medicine  |
|              |                | **Don Bailey, PhD**  
Distinguished Fellow  
Genomics, Bioinformatics and Translational Research Center  
RTI International |
| 1:00 PM – 1:15 PM | New Business   | **Cynthia M. Powell, MD, MS, FACMG, FAAP**  
Committee Chair                                                     |
| 1:15 PM      | Adjourn        |                                                                           |