

# The Advisory Committee on Heritable Disorders in Newborns and Children

## WEBINAR November 9-10, 2021

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

TIME	TOPIC	PRESENTER
10:00 AM – 10:20 AM	<ul style="list-style-type: none"> <li>• Welcome</li> <li>• Roll Call</li> <li>• Opening Remarks</li> <li>• Committee Business</li> </ul>	<p><b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair</p> <p><b>Mia Morrison, MPH</b> Designated Federal Official Health Resources and Services Administration</p>
10:20 AM – 11:35 AM	<p><b>Mucopolysaccharidosis Type II Evidence-Based Review – Phase 2 Update</b></p>	<p><b>Alex R. Kemper, MD, MPH, MS</b> Lead, Evidence-Based Review Group</p> <p><b>Lisa A. Prosser, PhD</b> Member, Evidence-Based Review Group</p>
11:35 AM – 11:50 AM	<p><b>Overview of Immediately Actionable Committee Process Updates:</b></p> <ul style="list-style-type: none"> <li>• Condition nomination form</li> <li>• Methods for assessing published and unpublished evidence, reporting cost estimates</li> <li>• Decision matrix guidance</li> </ul>	<p><b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair</p>
11:50 AM – 12:05 PM	<ul style="list-style-type: none"> <li>• Committee Discussion</li> <li>• <i>Vote on whether or not to approve immediately actionable updates</i></li> </ul>	<p><b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair</p>
12:05 PM – 12:15 PM	<p><b>Review of the Committee's Evidence-Based Review and Decision-Making Processes: Recap of Key Issues Identified for Future Consideration</b></p>	<p><b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair</p>
12:15 PM – 12:45 PM	Break	
12:45 PM – 1:15 PM	Public Comment	
1:15 PM – 2:00 PM	<p><b>HRSA Newborn Screening Portfolio Evaluation: Current and Future Needs of the Newborn Screening System</b></p>	<p><b>Melissa Raspa, PhD</b> Senior Scientist and Director Genomics, Ethics and Translational Research Program RTI International</p>
2:00 PM	End of Day 1	

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

TIME	WORKGROUPS
3:00 PM – 4:30 PM	<ul style="list-style-type: none"> <li>• Education and Training Workgroup</li> <li>• Follow-up and Treatment Workgroup</li> <li>• Laboratory Standards and Procedures Workgroup</li> </ul>

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

TIME	TOPIC	PRESENTER
10:00 AM – 10:10 AM	<ul style="list-style-type: none"> <li>• Welcome</li> <li>• Roll Call</li> </ul>	<p><b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair</p> <p><b>Mia Morrison, MPH</b> Designated Federal Official Health Resources and Services Administration</p>
10:10 AM – 10:55 AM	<b>Guanidinoacetate Methyltransferase (GAMT) Deficiency Evidence-Based Review – Phase 1 Update</b>	<b>Alex R. Kemper, MD, MPH, MS</b> Lead, Evidence-Based Review Group
10:55 AM – 11:05 AM	<b>Education and Training Workgroup Update</b>	<b>Jane M. DeLuca, PhD, RN, CPNP</b> Committee Member Chair, Education and Training Workgroup
11:05 AM – 11:15 AM	<b>Follow-up and Treatment Workgroup Update</b>	<b>Jeffrey P. Brosco, MD, PhD</b> Committee Member Chair, Follow-up and Treatment Workgroup
11:15 AM – 11:25 AM	<b>Laboratory Standards and Procedures Workgroup Update</b>	<b>Kellie B. Kelm, PhD</b> Ex-Officio Committee Member Chair, Laboratory Standards and Procedures Workgroup
11:25 AM – 11:40 AM	<b>Discussion: Workgroup Ideas</b>	<b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair
11:40 AM – 12:00 PM	<b>Break</b>	
12:00 PM – 1:00 PM	<b>Newborn Screening Pilot Programs</b> <ul style="list-style-type: none"> <li>• ScreenPlus – New York</li> <li>• Early Check – North Carolina</li> </ul>	<p><b>Melissa Wasserstein, MD</b> Chief, Division of Pediatric Genetic Medicine Professor of Pediatrics and Genetics The Children’s Hospital at</p>

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<b>TIME</b>	<b>TOPIC</b>	<b>PRESENTER</b>
		Montefiore The University Hospital for Albert Einstein College of Medicine  <b>Don Bailey, PhD</b> Distinguished Fellow Genomics, Bioinformatics and Translational Research Center RTI International
<b>1:00 PM - 1:15 PM</b>	<b>New Business</b>	<b>Cynthia M. Powell, MD, MS, FACMG, FAAP</b> Committee Chair
<b>1:15 PM</b>	<b>Adjourn</b>	