

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 • Condition nomination form • Methods for assessing published and unpublished evidence • Decision matrix guidance 	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

3:00 PM – 4:30 PM	<ul style="list-style-type: none"> • Education and Training Workgroup • Follow-up and Treatment Workgroup • Laboratory Standards and Procedures Workgroup
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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"> • Welcome • Roll Call 	<p>Cynthia M. Powell, MD, MS, FACMG, FAAP Committee Chair</p> <p>Mia Morrison, MPH Designated Federal Official Health Resources and Services Administration</p>
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	
12:00 PM – 1:00 PM	Newborn Screening Pilot Programs <ul style="list-style-type: none"> • ScreenPlus – New York • Early Check – North Carolina 	<p>Melissa Wasserstein, MD Chief, Division of Pediatric Genetic Medicine Professor of Pediatrics and Genetics The Children’s Hospital at Montefiore The University Hospital for Albert Einstein College of Medicine</p>

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TIME	TOPIC	PRESENTER
		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	