

# The Advisory Committee on Heritable Disorders in Newborns and Children

## WEBINAR November 14, 2024

Agenda: Thursday, November 14, 2024, from 10:00 AM – 3:50 PM Eastern Time

TIME	TOPIC	PRESENTER
10:00 AM – 10:15 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>Ned Calonge, MD, MPH</b> Committee Chair</p> <p><b>CDR Leticia Manning, MPH</b> Designated Federal Official</p>
10:15 AM – 10:35 AM	<b>Newborn Screening Health Information Center Overview</b>	
	<p><b>Session Purpose:</b> To provide an overview of the NBSIC website (<a href="https://www.hrsa.gov/newborn-screening">Home   Newborn Screening Information Center (NBSIC) (hrsa.gov)</a>) that provides up-to-date information and resources about newborn screening.</p>	<p><b>Molly Lynch, MPH</b> Project Director, Content Development for the NBSIC RTI International</p>
10:35 AM – 10:45 AM	<b>Committee Discussion</b>	
10:45 AM – 11:30 AM	<b>Research Funding Opportunities to Document Lived Experience of Patients and Families</b>	
	<p><b>Session Purpose:</b> To provide information on funding opportunities for research on rare diseases that includes family and/or lived experience perspectives. It is essential that the family and youth voice be included in the research literature so that the ACHDNC has as complete information as possible through the evidence review process.</p>	<p><b>Zhanzhi (Mike) Hu, PhD</b> Cofounder Project Guardian</p> <p><b>Nora McGhee, PhD</b> Associate Director, Clinical Comparative Effectiveness Research Patient Centered Outcomes Research Institute, PCORI</p> <p><b>Robyn Sagatov, PhD, MHS, RDN</b> Division of Priority Populations, Office of Extramural Research, Education, and Priority Populations Agency for Healthcare Research and Quality</p> <p><b>Melissa Parisi, MD, PhD</b> Chief, Intellectual and Developmental Disabilities Branch <i>Eunice Kennedy Shriver</i> National Institute of Child Health and Human Development</p> <p><b>Catharine Riley, PhD, MPH</b> National Center on Birth Defects and Developmental Disabilities Centers for Disease Control and Prevention</p>

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<b>11:30 AM – 11:50 AM</b>	<b>Committee Discussion</b>	
<b>11:50 AM – 12:30 PM</b>	<b>Lunch</b>	
<b>12:30 PM – 1:15 PM</b>	<b>Public Comments</b>	
<b>1:15 PM – 1:35 PM</b>	<b>Family Outcomes of Newborn Screening: Project Update</b>	
	<p><b>Session Purpose:</b> To provide an update on the development of an assessment tool to document family outcomes of newborn screening. This is part of the larger work to implement the <i>Blueprint for Change</i> by measuring what matters to youth and families.</p>	<p><b>Don Bailey, Jr., PhD, Med</b> RTI International</p> <p><b>Elizabeth Reynolds, PhD</b> Research Public Health Analyst, Genomics, Ethics, and Translational Research (GET) program RTI International</p>
<b>1:35 PM – 1:50 PM</b>	<b>Committee Discussion</b>	
<b>1:50 PM – 2:10 PM</b>	<b>Metachromatic Leukodystrophy (MLD) Evidence-Based Review Update</b>	<p><b>Alex R. Kemper, MD, MPH, MS</b> Lead, Evidence-Based Review Group</p>
<b>2:10 PM – 2:20 PM</b>	<b>Committee Discussion</b>	
<b>2:20 PM – 2:35 PM</b>	<b>Break</b>	
<b>2:35 PM – 2:55 PM</b>	<b>Laboratory Developed Tests</b>	
	<p><b>Session Purpose:</b> To provide information on the impact of the Food and Drug Administration’s final rule and phaseout policy to increase oversight of laboratory developed tests (LDTs).</p>	<p><b>Peter Kyriacopoulos</b> Chief Policy Officer Association of Public Health Laboratories</p> <p><b>Scott Shone, PhD, HCLD (ABB)</b> Public Health Laboratory Director North Carolina Department of Health and Human Services</p>

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<b>2:55 PM – 3:05 PM</b>	<b>Committee Discussion</b>	
<b>3:05 PM – 3:20 PM</b>	<b>Higher-Tier Screening Ad Hoc Topic Group Update</b>	
	<b>Session Purpose:</b> To share updates and information on proposed next steps for the higher-tier testing ad hoc topic group.	<b>Shawn Moloney, MPH, MLS(ASCP)<sup>CM</sup></b> Newborn Screening Laboratory Manager Michigan Department of health and Human Services
<b>3:20 PM – 3:30 PM</b>	<b>Committee Discussion</b>	
<b>3:30 PM – 3:45 PM</b>	<b>New Business</b>	
<b>3:45 PM – 3:50 PM</b>	<b>Adjourn</b>	