

# The Advisory Committee on Heritable Disorders in Newborns and Children

## IN-PERSON/WEBINAR

**HRSA Headquarters - 5600 Fishers Lane, Rockville, MD 20852 (Pavilion)  
May 9-10, 2024**

**Agenda: Thursday, May 9, 2024, from 10:00 AM – 3:45 PM Eastern Time**

<b>TIME</b>	<b>TOPIC</b>	<b>PRESENTER</b>
<b>10:00 AM – 10:15 AM</b>	<ul style="list-style-type: none"> <li>• <b>Welcome</b></li> <li>• <b>Roll Call</b></li> <li>• <b>Opening Remarks</b></li> <li>• <b>Committee Business</b></li> </ul>	<b>Ned Calonge, MD, MPH</b> Committee Chair  <b>CDR Leticia Manning, MPH</b> Designated Federal Official
<b>10:15 AM – 11:00 AM</b>	<b>Public Comments</b>	
<b>11:00 AM – 11:10 AM</b>	<b>Break</b>	
<b>11:10 AM – 11:30 AM</b>	<b>Regulatory Process for the Review of Drugs for Rare Diseases</b>	<b>Anita Zaidi, MD</b> Lead Physician Center for Drug Evaluation and Research (CDER) U.S. Food and Drug Administration
<b>11:30 AM – 12:00 PM</b>	<b>Committee Discussion</b>	
<b>12:00 PM – 1:00 PM</b>	<b>Lunch</b>	
<b>1:00 PM – 1:30 PM</b>	<b>Duchenne Muscular Dystrophy Evidence-Based Review: Update</b>	<b>Alex R. Kemper, MD, MPH, MS</b> Lead, Evidence-Based Review Group
<b>1:30 PM – 1:50 PM</b>	<b>Committee Discussion</b>	
<b>1:50 PM – 2:00 PM</b>	<b>Committee Liaison Update</b> - <b>Committee Discussion and Vote</b>	
<b>2:00 PM – 2:20 PM</b>	<b>ACHDNC Decision Matrix Tool: Public Health System Assessment</b>	<b>Ned Calonge, MD, MPH</b> Committee Chair
<b>2:20 PM – 2:45 PM</b>	<b>Committee Discussion</b>	
<b>2:45 PM – 2:55 PM</b>	<b>Break</b>	
<b>2:55 PM – 3:10 PM</b>	<b>ACHDNC Nomination and Evidence Review Process</b>	<b>Ned Calonge, MD, MPH</b> Committee Chair
<b>3:10 PM – 3:30 PM</b>	<b>Committee Discussion</b>	
<b>3:30 PM – 3:45 PM</b>	<b>Awards and Acknowledgements</b>	
<b>3:45 PM</b>	<b>End Day 1</b>	

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<b>10:00 AM – 10:15 AM</b>	<ul style="list-style-type: none"> <li>• <b>Welcome</b></li> <li>• <b>Roll Call</b></li> <li>• <b>Opening Remarks</b></li> <li>• <b>Committee Business</b></li> </ul>	<b>Ned Calonge, MD, MPH</b> Committee Chair  <b>CDR Leticia Manning, MPH</b> Designated Federal Official
<b>10:15 AM – 10:35 AM</b>	<b>Qualitative Evidence Synthesis: GRADE-CERQual Approach For Assessing the Confidence in Synthesized Findings</b>	<b>Jane Noyes, MD, PhD</b> Professor in Health and Social Services Research and Child Health, Bangor University
<b>10:35 AM – 11:00 AM</b>	<b>Committee Discussion</b>	
<b>11:00 AM – 11:30 AM</b>	<b>Public Comments</b>	
<b>11:30 AM – 12:30 PM</b>	<b>Lunch</b>	
<b>12:30 PM – 1:45 PM</b>	<b>Newborn Screening Ad Hoc Topic Groups: Updates and Committee Discussion</b>	<b>Jelili Ojodu, MPH</b> Association of Public Health Laboratories
<b>1:45 PM – 1:55 PM</b>	<b>New Business</b>	
<b>1:55 PM – 2:00 PM</b>	<b>Adjourn</b>	