

The Advisory Committee on Heritable Disorders in Newborns and Children

IN-PERSON/WEBINAR

HRSA Headquarters - 5600 Fishers Lane, Rockville, MD 20852 (Pavilion)

January 29 – 30, 2024

Agenda: Monday, January 29, 2024, from 10:00 AM – 4:30 PM Eastern Time

TIME	TOPIC	PRESENTER
10:00 AM – 10:15 AM	<ul style="list-style-type: none"> • Welcome • Roll Call • Opening Remarks • Committee Business 	Ned Calonge, MD, MPH Committee Chair CDR Leticia Manning, MPH Designated Federal Official
10:15 AM – 10:35 AM	Family Outcomes of Newborn Screening: Project Overview Update	Don Bailey, Jr., PhD, MEd RTI International
10:35 AM – 10:55 AM	Committee Discussion	
10:55 AM – 11:40 AM	Research on Family Perspectives	
	Families' Search for Meaning and Value in Rare Genetic Diagnoses	Sara Ackerman, PhD, MPH University of California, San Francisco
	The "Value of Values": Expanding Assessment of Net Benefits and Harms through Social Science Data	Aaron Goldenberg, PhD, MA, MPH Case Western Reserve University School of Medicine
11:40 AM – 12:15 PM	Committee Discussion	
12:15 PM – 1:00 PM	Lunch	
1:00 PM – 1:30 PM	Public Comments	
1:30 PM – 1:45 PM	Duchenne Muscular Dystrophy Evidence-Based Review: Phase 2 Update	Alex R. Kemper, MD, MPH, MS Lead, Evidence-Based Review Group
1:45 PM – 2:00 PM	Committee Discussion	
2:00 PM – 2:15 PM	ACHDNC Decision Matrix Tool: Public Health Assessment	Ned Calonge, MD, MPH Committee Chair
2:15 PM – 2:35 PM	Committee Discussion	
2:35 PM – 2:45 PM	Break	

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2:45 PM – 3:00 PM	ACHDNC Nomination Process Update	Ned Calonge, MD, MPH Committee Chair
3:00 PM – 3:10 PM	ACHDNC Nomination and Evidence-Based Review Process Proposal	Ned Calonge, MD, MPH Committee Chair
3:10 PM – 4:30 PM	Discussion	
4:30 PM	End Day 1	

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10:00 AM – 10:30 AM	<ul style="list-style-type: none"> • Welcome • Roll Call • Opening Remarks • Committee Business 	<p>Ned Calonge, MD, MPH Committee Chair</p> <p>CDR Leticia Manning, MPH Designated Federal Official</p>
10:30 AM – 11:15 AM	Public Comments	
11:15 AM – 11:25 AM	Break	
11:25 AM – 12:00 PM	Newborn Screening for Krabbe Disease: An Expedited Evidence-Based Review	<p>Alex R. Kemper, MD, MPH, MS Lead, Evidence-Based Review Group</p> <p>Lisa A. Prosser, PhD Member, Evidence-Based Review Group</p>
12:00 PM – 12:30 PM	Committee Discussion	
12:30 PM – 1:15 PM	Lunch	
1:15 PM – 1:35 PM	Committee Report: Newborn Screening for Krabbe Disease	<p>Shawn E. McCandless, MD Committee Member</p> <p>Jennifer M. Kwon, MD, MPH, FAAN Committee Member</p>
1:35 PM – 2:00 PM	<p>Committee Discussion</p> <ul style="list-style-type: none"> • <i>Vote on whether or not to recommend Krabbe Disease for inclusion on the Recommended Uniform Screening Panel</i> 	
2:00 PM – 2:10 PM	Break	
2:10 PM – 2:20 PM	NewSTEPS Ad Hoc Newborn Screening Workgroups: Update	Jelili Ojodu, MPH Association of Public Health Laboratories
2:20 PM – 2:30 PM	Committee Discussion	
2:30 PM – 2:40 PM	New Business	
2:40 PM – 2:45 PM	Adjourn	