

The Advisory Committee on Heritable Disorders in Newborns and Children

WEBINAR February 9-10, 2023

Agenda: Thursday, February 9, 2023 from 9:30 AM – 3:15 PM Eastern Time

TIME	TOPIC	PRESENTER
9:30 AM – 9:45 AM	<ul style="list-style-type: none"> • Welcome • Roll Call • Opening Remarks • Committee Business 	<p>Ned Calonge, MD, MPH Committee Chair</p> <p>Leticia Manning, MPH Acting Designated Federal Official Health Resources and Services Administration</p>
9:45 AM – 10:30 AM	Public Comment	
10:30 AM – 11:30 AM	Newborn Screening for Krabbe Disease: A Systematic Review of the Evidence (Part 1)	<p>Alex R. Kemper, MD, MPH, MS Lead, Evidence-Based Review Group</p>
11:30 AM – 11:45 AM	Break	
11:45 AM – 12:45 PM	Newborn Screening for Krabbe Disease: A systematic Review of the Evidence and Review of Public Health Assessment Survey Results (Part 2)	<p>Alex R. Kemper, MD, MPH, MS Lead, Evidence-Based Review Group</p> <p>Lisa A. Prosser, PhD Member, Evidence-Based Review Group</p>
12:45 PM – 1:30 PM	Lunch	
1:30 PM – 2:15 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>
2:15 PM – 3:10 PM	<p style="text-align: center;">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> 	<p>Ned Calonge, MD, MPH Committee Chair</p>
3:10 PM – 3:15 PM	Orientation to Workgroup Sessions	<p>Ned Calonge, MD, MPH Committee Chair</p>
3:15 PM	End of Day 1	

The Advisory Committee on Heritable Disorders in Newborns and Children

WEBINAR February 9-10, 2023

Workgroup Meetings: Thursday, February 9, 2023, from 3:45 PM – 5:00 PM Eastern Time

3:45 PM – 5:00 PM	<ul style="list-style-type: none"> • Education and Training Workgroup • Follow-up and Treatment Workgroup • Laboratory Standards and Procedures Workgroup
--------------------------	---

Agenda: Friday, February 10, 2023 from 9:30 AM – 2:00 PM Eastern Time

TIME	TOPIC	PRESENTER
9:30 AM – 9:45 AM	<ul style="list-style-type: none"> • Welcome • Roll Call • Committee Business 	<p>Ned Calonge, MD, MPH Committee Chair</p> <p>Leticia Manning, MPH Acting Designated Federal Official Health Resources and Services Administration</p>
9:45 AM – 10:15 AM	Prioritization and Capacity Workgroup Interim Update	Alex R. Kemper, MD, MPH, MS Lead, Evidence-Based Review Group
10:15 AM – 10:45 AM	Public Comment	
10:45 AM – 10:55 AM	Education and Training Workgroup Report	Jane M. DeLuca, PhD, RN, CPNP Committee Member Chair, Education and Training Workgroup
10:55 AM – 11:05 AM	Follow-up and Treatment Workgroup Report	Kyle Brothers, MD, PhD Committee Member Chair, Follow-up and Treatment Workgroup
11:05 AM – 11:15 AM	Laboratory Standards and Procedures Workgroup Report	Kellie B. Kelm, PhD Ex-Officio Committee Member Chair, Laboratory Standards and Procedures Workgroup
11:15 AM – 11:35 AM	Committee Discussion on Action Items	Ned Calonge, MD, MPH Committee Chair
11:35 AM – 12:00 PM	Lunch Break	

**The Advisory Committee on
Heritable Disorders in Newborns and Children**

**WEBINAR
February 9-10, 2023**

12:00 PM – 12:45 PM	Duchenne Muscular Dystrophy (DMD) Nomination Summary	Ned Calonge, MD, MPH Committee Chair
12:45 PM – 1:15 PM	Committee Discussion <ul style="list-style-type: none"> • Vote on whether or not to move DMD forward to full evidence review 	Ned Calonge, MD, MPH Committee Chair
1:15 PM – 1:45 PM	HRSA State Interoperability Program	Craig Newman, PhD Altarum Radley Remo, MPH Florida Department of Health Juan Vasquez, MHA Florida Department of Health Andy Rohrwasser, PhD, MBA Utah State Department of Health
1:45 PM – 1:55 PM	Committee Discussion	Ned Calonge, MD, MPH Committee Chair
1:55 PM – 2:00 PM	New Business	
2:00 PM	Adjourn	