

Genetic Diseases Program Advisory Committee Meeting

October 28, 2022, 2:00 p.m. – 3:30 p.m.

Location: <https://ldhophbfhgenetics.zoom.us/j/82949587366>

Agenda

- I. Welcome & Introductions
- II. Review of the Committee Minutes from 2/4/22
- III. Review and discussion of the current newborn screening system operations and performance
 - Newborn Screening MCH Block Grant Data – Ngoc Huynh
 - Update on implementation of new conditions (SMA, MPS I and Pompe) – Cheryl Harris
 - Laboratory Updates – Alyson Saadi
 - Status of Testing Added Conditions
 - Timeliness Improvements
- IV. Discussion of conditions under GDPAC review
 - X-linked adrenoleukodystrophy (X-ALD)
 - Overview X-ALD – bluebird bio
 - X-ALD readiness review and recommendations
- V. Discussion of conditions to initiate GDPAC review
 - Mucopolysaccharidosis type II (MPS II)
 - Discussion of recent nomination (see ACHDNC: [Chair Letter to the HHS Secretary](#))
 - Timeline for readiness review activities
- VI. Other Business
 - Legislative Updates –
 - 2022 - Establishment of the Sickle Cell Registry
 - 2023 – Legislative Proposal for the Newborn Screening Rule
 - Rare Diseases Advisory Committee Update – Jantz Malbrue
- VII. Upcoming Meetings, Webinars and Workshops
 - HHS Advisory Committee on Heritable Disorders in Newborns & Children Meeting
 - November 3-4, 2022
 - Registration Link: <https://www.achdncmeetings.org/registration/>
- VIII. 2023 Meeting Dates
 - January 27
 - April 28
 - July 27
 - October 27
- IX. Announcements
- X. Adjournment