

**Advisory Committee on Heritable
Disorders in Newborns and Children
Webinar Meeting
August 2, 2018**



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ACHDNC Business

- **Welcome**
- **Roll Call**
- **Vote – May Minutes**
- **Committee Business**
 - **SMA Update**
 - **Organization Representatives**
 - **Evidence-based Review Process**
 - **Implementation of New Conditions**
 - **Future meetings dates**
 - **Today's Meeting Topics**

ROLL CALL

VOTE – MAY MINUTES

NEW ADDITION TO THE RUSP

**SPINAL MUSCULAR ATROPHY (SMA)
DUE TO HOMOZYGOUS DELETION OF
EXON 7 IN SMN1**

CALL FOR ORGANIZATION REPRESENTATIVES

Organizations eligible include:

- Public health constituencies (state and local)
 - Hospitals and medical home
 - Professional societies with an emphasis on public health
 - Organizations that support state & local public health programs
- Primary Care
 - Professional societies (clinical and scientific)
- Specialty/Subspecialty
 - Professional societies (clinical and scientific)
- Other federal agencies
- Consumer/family organizations, advocacy groups with a broad focus and constituencies

NEW ORGANIZATIONAL REPRESENTATIVE

**SOCIETY OF INHERITED METABOLIC
DISORDERS (SIMD)**

SHAWN E. MCCANDLESS, MD

EVIDENCE-BASED REVIEW PROCESS

Take a closer look at the condition nomination process and evidence-based review process.

IMPLEMENTATION OF NEW CONDITIONS

Evaluate states' ability to implement the screening of a new condition and cost implications of implementing population based screening.

FUTURE MEETINGS DATES

- November 1-2, 2018 (in person and webcast)
- April 22-23, 2019 (in person and webcast - new dates)
- Meeting dates through 2020 can be found on the Committee's website:
<https://www.hrsa.gov/advisory-committees/heritable-disorders/meetings/index.html>

MEETING TOPICS

THURSDAY, AUGUST 2, 2018

- **Risk Assessment in Newborn Screening**
- **Improving Timeliness in Newborn Screening**
- **Workgroup Updates**
- **Report on Long Term Follow Up in Newborn Screening**
- **Report on Technology in Newborn Screening**

Advisory Committee on Heritable Disorders in Newborns and Children

- **Established under the Public Health Service Act, Title XI, § 1109 (42 U.S.C. 300b-10), as amended by the Newborn Screening Saves Lives Reauthorization Act of 2014 (P.L. 113-240)**
- **All federal committees governed by the Federal Advisory Committee Act (FACA), Public Law 92-463, as amended (5 U.S.C. App. 2), and by 41 C.F.R. Parts 101-6 and 102-3**
- **FACA sets the standards for the establishment, utilization, and management of Federal Advisory committees**

Ethics and Conflicts of Interest (CIOs)

- **Advisory to the Secretary of HHS**
- **Inquiries about the Committee**
- **When to recuse:**
 - In all matters likely to affect the financial interests of any organization with which you serve as an officer, director, trustee, or general partner unless you are also an employee of the organization or unless you have received a waiver from HHS authorizing them to participate.

Meeting Participation

- All Committee meetings are open to the public.
- If the public wish to participate in the discussion, the procedures for doing so are published in the Federal Register and announced at the opening of a meeting.
- The Notice of Meeting in Federal Register indicated two options for public comment:
 - Oral Comments - no requests were received for this meeting
 - Submit written statements – no written statements were received for this meeting
 - Committee members receive copies of all written statements submitted
- Any further public participation will be solely at the discretion of the Chair and DFO

Webinar

- **Committee Members, Organization Representatives and Speakers:** please use the “raise hand” function if you have a comment or ask a question. The Chair will call on you in order of request.
 - Please unmute your phone line.
 - Please state your name first to ensure proper recording for the Committee transcript and minutes.
- **Meeting attendees:** We cannot receive audio through the Webinar.