

# Advisory Committee on Heritable Disorders in Newborns and Children Meeting April 23 - 24, 2019



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Professor and Chairman  
Department of Pediatrics  
Louisiana State University Health –  
Shreveport

# ACHDNC Business

- **Welcome**
- **Roll Call**
- **Vote – March 2019 Minutes**

# NEW COMMITTEE MEMBERS

**Kyle Brothers, MD, PhD**

**Jane M. DeLuca PhD RN**



# **NEW ORGANIZATION REPRESENTATIVES**

# **NEW CONDITION NOMINATION FOR THE RUSP**

**A nomination team, led by the National CMV  
Foundation, submitted a nomination for:**

**congenital cytomegalovirus (CMV).**

# FUTURE MEETINGS DATES

- August 1-2, 2019 (in person and webcast)
- November 7-8, 2019 (in person and webcast)
- Meeting dates through 2023 can be found on the Committee's website:  
<https://www.hrsa.gov/advisory-committees/heritable-disorders/meetings/index.html>

# **MEETING TOPICS**

## **Tuesday, April 23, 2019**

- **New Disorders Readiness Tool**
- **RUSP Condition Nomination and Evidence Review Process: Draft Approach and Timeline**
- **Systematic Evidence Review Process**

# **MEETING TOPICS**

## **Wednesday, April 24, 2019**

- **Newborn Screening Pilot Studies**
- **Update from the Ad-hoc Workgroup on Interpreting NBS Results**
- **Rare Disease Registries**
  - **Cystic Fibrosis Foundation – CF Registry**
  - **Primary Immune Deficiency Treatment Consortium – SCID data collection**



# **MEETING TOPICS**

## **Wednesday, April 24, 2019**

- **Workgroup Updates**
  - **Follow Up and Treatment**
  - **Education and Training**
  - **Laboratory Standards and Procedures**
- **What is On the Horizon**

# **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 – 7 requests received
  - Submit written statements – 2 written statements received
    - Committee members receive copies of all written statements submitted
- Any further public participation will be solely at the discretion of the Chair and DFO

# 5600 Fishers Lane Building Info

- **Visitors only have access the Pavilion, cafeteria, restrooms, and meeting rooms.**
  - **All areas of the facility are restricted and require an escort by a HRSA staff member. No exceptions.**
- **If you need to leave and re-enter, you will be required to go through security screening again and will require a HRSA escort to meet you at Security to escort you in the building.**
  - **15 minutes prior to and after the lunch break ends a HRSA escort will be at the main entrance for those who need to leave and return.**
  - **For other re-entry needs, please notify a HRSA staff member or a registration team member.**

# 5600 Fishers Lane Building Info

- Visitors are not allowed to take any video or photography in the building.
- In case of emergency please exit through the front door of 5600 Fishers Lane, cross the street, and meet on the parking pad to the left. Your escorts will have a roster and ensure everyone is accounted for.
- Please do not take any non-essential items with you if there is an evacuation, as it could delay your reentry into the building.