

# The Advisory Committee on Heritable Disorders in Newborns and Children

**HRSA Headquarters - 5600 Fishers Lane, Rockville, MD 20852 (Pavilion)  
April 23 - 24, 2019**

**AGENDA: Tuesday, April 23, 2019 from 10:00AM to 3:15PM**

<b>TIME</b>	<b>TOPIC</b>	<b>PRESENTER</b>
<b>10:00AM – 10:15AM</b>	<ul style="list-style-type: none"> <li>• <b>Welcome</b></li> <li>• <b>Roll Call</b></li> <li>• <b>Opening Remarks</b></li> <li>• <b>March 2019 Minutes</b></li> </ul>	<b>Joseph Bocchini, MD</b> Committee Chair  <b>Catharine Riley, PhD, MPH</b> Designated Federal Official Health Resources and Services Administration (HRSA)
<b>10:15AM – 11:00AM</b>	<b>New Disorders Readiness Tool</b>	<b>Yvonne Kellar-Guenther, PhD</b> Senior Research Scientist Center for Public Health Innovation
<b>11:00AM – 11:15AM</b>	<b>Public Comment</b>	
<b>11:15AM – 11:30AM</b>	<b>RUSP Condition Nomination and Evidence Review Process: Draft Approach and Timeline</b>	<b>Joseph Bocchini, MD</b> Committee Chair
<b>11:30AM – 12:30PM</b>	<b>Lunch</b>	
<b>12:30PM – 2:15PM</b>	<b>Evidence Review Process</b>	<b>Cynthia Powell, MD, MS, FACMG, FAAP</b> Incoming Committee Chair  <b>Alex R. Kemper, MD, MPH, MS</b> Lead, Evidence-based Reviews
<b>2:15PM – 3:15PM</b>	<b>Acknowledgements for Dr. Bocchini</b>	
<b>3:15PM</b>	<b>Adjourn*</b>	

***\*Workgroups meet on Tuesday, April 23, 2019 from 3:30pm – 5:00pm  
at 5600 Fishers Lane, Rockville, MD.***

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**April 23 - 24, 2019**

**AGENDA: Wednesday, April 24, 2019 from 9:30AM to 2:00PM**

<b>TIME</b>	<b>TOPIC</b>	<b>PRESENTER</b>
9:30AM - 9:45AM	<ul style="list-style-type: none"> <li>• Welcome</li> <li>• Roll Call</li> </ul>	<b>Joseph Bocchini, MD</b> Committee Chair <b>Catharine Riley, PhD, MPH</b> Designated Federal Official, HRSA
9:45AM - 10:15AM	<b>Newborn Screening Pilot Studies</b>	<b>Michael S. Watson, PhD, FACMG</b> Executive Director, American College of Medical Genetics and Genomics
10:15AM - 10:30AM	<b>Ad-Hoc Workgroup - Interpreting NBS Results</b>	<b>Mei Baker, MD</b> Committee Member
10:30AM - 11:00AM	<b>Cystic Fibrosis Registry</b>	<b>Bruce Marshall, MD</b> Senior Vice President of Clinical Affairs Cystic Fibrosis Foundation
11:00AM - 11:30AM	<b>Primary Immune Deficiency Treatment Consortium (PIDTC): Severe Combined Immunodeficiencies (SCID) Data Collection</b>	<b>Jennifer Puck, MD</b> Principle Investigator, PIDTC UCSF Department of Pediatrics Allergy, Immunology, and Blood and Marrow Transplantation Division
11:30AM- 12:15PM	<b>Lunch</b>	
12:15PM - 12:30PM	<b>Public Comment</b>	
12:30PM - 12:45PM	<b>Follow Up and Treatment Workgroup Update</b>	<b>Christopher Kus, MD, MPH</b> Co-Chair, Follow Up and Treatment Workgroup
12:45PM - 1:00PM	<b>Education and Training Workgroup Update</b>	<b>Beth Tarini, MD</b> Chair, Education and Training Workgroup
1:00PM - 1:15PM	<b>Laboratory Standards and Procedures Workgroup Update</b>	<b>Kellie Kelm, PhD</b> Chair, Laboratory Standards and Procedures Workgroup
1:15PM - 1:30PM	<b>RUSP Condition Nomination and Evidence Review Process: Follow up Discussion</b>	<b>Joseph Bocchini, MD</b> Committee Chair
1:30PM - 1:55PM	<b>On the Horizon</b>	<b>Cynthia Powell, MD</b> Incoming Committee Chair
1:55PM - 2:00PM	<ul style="list-style-type: none"> <li>• New Business</li> <li>• Passing of the Gavel</li> </ul>	<b>Joseph Bocchini, MD</b> Outgoing Committee Chair <b>Cynthia Powell, MD</b> Incoming Committee Chair
2:00PM	<b>Adjourn</b>	