PRE-MEETING WORKSHOP

NEWBORN SCREENING: TOWARD A SYSTEM THAT KEEPS PACE WITH GENE THERAPY ADVANCES

MAY 15, 2022
NEWBORN SCREENING: TOWARD A SYSTEM THAT KEEPS PACE WITH GENE THERAPY ADVANCES

SUNDAY, MAY 15, 2022
All times listed below ET

Co-chairs: Diane Berry, PhD, Sarepta
Meagan Perry, Orchard Therapeutics

Description
This workshop will dive into the key policy issues affecting the U.S. newborn screening system, given the anticipated wave of innovative therapy approvals. Topics will include modernizing the NBS process; addressing the challenges of data collection via NBS pilot programs; and evaluating emerging screening and clinical programs.
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Schedule
All times listed below ET

8:00–8:55 AM
Session 1 The Recommended Uniform Screening Panel (RUSP)—Current and Future Process
Potential Solutions to Prepare Newborn Screening for Transformative Therapies
Don Bailey, PhD, RTI International
Streamlining Future RUSP Applications
Terri Klein, National MPS Society
Roundtable Panel: The RUSP—Current and Future Process
Don Bailey, PhD, RTI International
Terri Klein, National MPS Society
Elisa Seeger, ALD Alliance
Moderated by: Stacey Frisk, Sarepta

8:55–9:45 AM
Session 2 State Implementation and Innovation
Hastening the State Uptake of Newly Approved Conditions
Erin Frey, Ultragenyx
What Can States Do Before a Condition is Added to the RUSP?
Julie Morrison, Illinois State Senate
Roundtable Panel: State Implementation and Innovation
Erin Frey, Ultragenyx
Annie Kennedy, EveryLife Foundation
Julie Morrison, Illinois State Senate
Scott Shone, PhD, North Carolina State Laboratory of Public Health
Moderated by Stacey Frisk, Sarepta
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9:45–10:15 AM
Break

10:15 AM–11:05 AM
Session 3  Innovation in Clinical Trial Design for Cell and Gene Therapies: What’s on the Horizon?
Opportunities of a Multi-disease Panel Approach
Melissa Wasserstein, MD, Montefiore Medical Center

State Pilot Programs: New York’s Experience
Joseph Orsini, PhD, New York Department of Health

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Roundtable Panel: Data Collection and Newborn Screening Program
Amy Brower, PhD, American College of Genetics and Genomics
Barbara Burton, MD, Lurie’s Children’s Hospital
Joseph Orsini, PhD, New York Department of Health
Melissa Wasserstein, MD, Montefiore Medical Center

Moderated by: Kent Christopherson, PhD, Orchard Therapeutics

11:05 AM–12:00 PM
Session 4 Evaluating Emerging Screening Methods and Clinical Programs

Evaluating Emerging Screening Methods and Clinical Programs
Stephen Kingsmore, MD, DSc, Rady Children’s Hospital

Evaluating Emerging Screening Methods and Clinical Programs
Richard Parad, MD, Brigham and Women’s Hospital

Roundtable Panel: Evaluating Emerging Screening Methods and Clinical Programs
Stephen Kingsmore, MD, DSc, Rady Children’s Hospital
Richard Parad, MD, Brigham and Women’s Hospital
Philip Reilly, MD, JD, Third Rock Ventures

Moderated by: Kent Christopherson, PhD, Orchard Therapeutics