



## 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

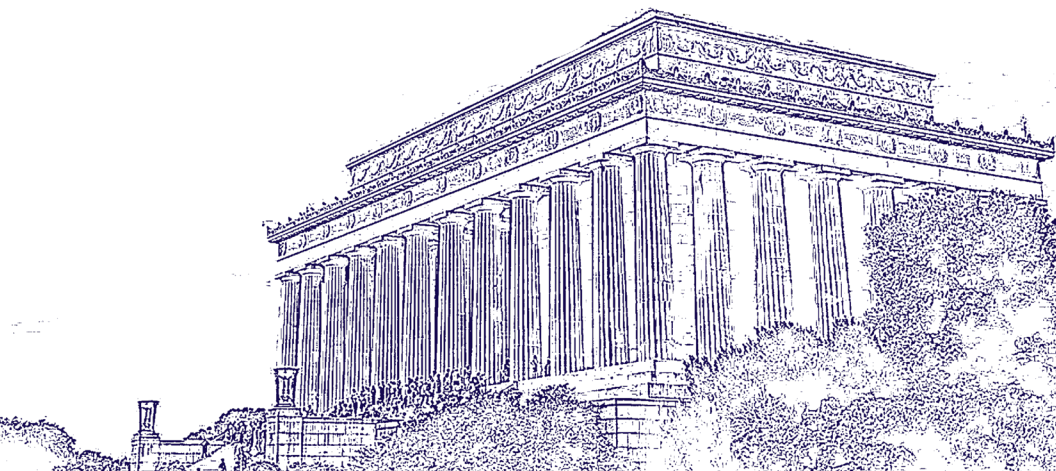
**SUNDAY, MAY 15, 2022**

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

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#### 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*

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

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*

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**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*



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