



# Advancing Innovation in Rare Disease Research

Agenda subject to change.

\* All times are ET

## TUESDAY, APRIL 14, 2026

7:45AM **CONFERENCE REGISTRATION AND CONTINENTAL BREAKFAST**

8:30AM **NORD'S WELCOME & OPENING REMARKS**  
Pamela Gavin, CEO, NORD  
*Special Video Message from Becky Quick of CNBC*

8:45AM **DAY 1 KEYNOTE ADDRESS: FDA (Invited)**

### SECTION I: Clinical Research and Trial Design in Rare Disease

9:10AM **BAYESIAN AND ADAPTIVE TRIAL DESIGNS FOR ULTRA-RARE POPULATIONS**  
Bayesian and adaptive approaches offer alternative ways to design and analyze studies when patient populations are extremely small. This session will frame how these methods differ from traditional trial designs and why they are increasingly considered in ultra-rare disease research, including their potential to support learning over time, reduce patient burden, and make more efficient use of limited data.

**Speakers:**

Kelley Kidwell, Ph.D, Professor of Biostatistics, **University of Michigan School of Public Health**  
Olivia Morgan, Statistician, **FDA**

10:00AM **ALTERNATIVES TO PLACEBO CONTROLS IN RARE DISEASE TRIALS**  
Placebo-controlled trials are often impractical or ethically challenging in rare disease settings. This session will explore approaches that use alternative comparators, such as within-patient comparisons or external data sources, and discuss how these strategies can be used to generate interpretable evidence while addressing feasibility, ethical considerations, and regulatory expectations.

**Moderator:** FDA Representative

**Speakers:**

Oxana V. Crysler, MD, MHS, **University of Michigan**  
Wonyul Lee, FDA Representative, **FDA**

10:50AM **NETWORKING BREAK**

## TUESDAY, APRIL 14, 2026 (continued)

### 11:10AM **FUNDING RARE DISEASE RESEARCH**

Sustaining rare disease research requires funding models that account for small populations, long development timelines, and limited commercial incentives. This session will examine broad approaches to funding rare disease research and the roles that patient advocacy organizations, academic institutions, and industry partners can play across different stages of development.

**Speakers:**

Michael Hund, CEO, **EB Research Partnership**

### 12:00PM **NETWORKING LUNCH**

### 1:15PM **INNOVATIVE CLINICAL TRIAL DESIGNS**

Nontraditional trial designs are being used across therapeutic areas to address heterogeneity, small sample sizes, and evolving scientific understanding. This session will provide an overview of nontraditional clinical trial structures, such as umbrella, basket, and pragmatic trial designs, highlighting their applicability to rare disease research and their potential to improve efficiency, generalizability, and evidentiary yield.

**Speakers:**

Jaishri Blakeley, MD, **Johns Hopkins University / EUPEARL**

## SECTION II: Drug Development: From Clinical Trials to Clinical Care

### 2:05PM **FDA REGULATORY INNOVATION: PLAUSIBLE MECHANISM AND PLATFORM PATHWAYS**

For many rare diseases, especially those with strong biological rationale but limited clinical data, regulatory approaches may rely on alternative forms of evidence. This session will provide context on how concepts such as plausible mechanism and platform-based development are considered within the regulatory framework for therapies targeting small populations.

**Speakers:**

Judy Stecker, Regulatory Affairs Leader, **Former HHS**

### 3:15PM **ABANDONED, SHELVED, AND RESCUED THERAPIES FOR ULTRA-RARE DISEASES**

Drug development programs for ultra-rare diseases may be discontinued for reasons unrelated to scientific validity, including resource constraints or shifting priorities. Presenters will describe case studies of therapies that were abandoned for commercial or logistical reasons and later revived through academic leadership, public-benefit models, or through repositioning.

**Speakers:**

Paul Ayoub, PhD, MBA, Founder & Chief Executive Officer, **Rarity PBC**

Matthew Porteus, MD, PhD, Professor of Pediatrics, **Stanford University**

### 4:10PM **INNOVATIONS IN DRUG REPURPOSING FOR RARE DISEASES**

Drug repurposing seeks to identify new disease applications for existing therapies. This session will provide an overview of how repurposing approaches are being explored in rare diseases and why they may offer a pragmatic path to treatment development when traditional discovery and development models are not feasible.

**Speakers:**

Sarah Fuchs, Medical Officer, **FDA**

Kasha Morris, Co-Founder, **TANGO2 Research Foundation**

Heather Stone, Acting Director, Division of Rare Diseases and Medical Genetics, Health Science Policy Analyst, **FDA**

### 5:00PM **PRESENTATION OF THE 2026 MEDICAL & SCIENTIFIC TRAILBLAZER RARE IMPACT AWARDS®**

### 5:30PM **NETWORKING RECEPTION**

## WEDNESDAY, APRIL 15, 2026 (continued)

8:00AM **CONTINENTAL BREAKFAST**

9:00AM **DAY 1 RECAP AND OPENING REMARKS**

9:05AM **KEYNOTE ADDRESS**

Jay Bhattacharya, MD, PhD, Director, **National Institutes of Health**

### SECTION III: Leveraging Data for Innovation

9:50AM **DATA FOR ENDPOINT SELECTION**

Selecting appropriate endpoints is a central challenge in rare disease research, particularly when clinical outcomes are heterogeneous or poorly characterized. This session will explore how different data sources can inform endpoint selection and support endpoints that are clinically meaningful, feasible to measure, and interpretable for regulators and other decision-makers.

**Speakers:**

Amy Raymond, PhD, PMP, Therapeutic Strategy Lead, Rare Diseases and Cellular & Genetic Medicines, **Worldwide Clinical Trials**

10:50AM **NETWORKING BREAK**

11:10AM **REGISTRIES AND REAL-WORLD DATA AS DEVELOPMENT PLATFORMS**

Registries and real-world data sources are increasingly used to support multiple stages of rare disease research. This session will frame how these data assets can function as development platforms, informing natural history studies, clinical trial design, and evidence generation beyond traditional clinical trials.

**Speakers:**

Mayowa Osundiji, MBBS, PhD, Medical Geneticist, **Mayo Clinic**

Theresa Strong, Director of Research Programs, **Foundation for Prader-Willi Research**

Srilaskhmi Raj, PhD, Research Leader, **Albert Einstein College of Medicine**

12:10PM **ACCESS AND COVERAGE: DATA AND CLINICAL DEVELOPMENT**

Decisions about coverage and access are influenced by the type and strength of evidence generated during and after clinical development. This session will examine how clinical and real-world data intersect with payer and coverage considerations, and why early alignment between development strategies and evidence needs is particularly important in rare diseases.

**Speakers:**

Danny Yeh, PhD, Executive Director, Value Evidence, Center of Excellence, **Aesera**

1:00PM **CLOSING REMARKS**



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