7:30 a.m.  Registration and Poster/Exhibit Booth Setup

8:30 a.m.  Welcome
Christopher P. Austin, M.D., Director, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH); Chair, International Rare Diseases Research Consortium (IRDiRC)

James K. Gilman, M.D., Chief Executive Officer, NIH Clinical Center (CC)

8:50 a.m.  NCATS Office of Rare Diseases Research (ORDR) Update
Petra Kaufmann, M.D., M.Sc., Director, ORDR, NCATS, NIH
Anne Pariser, M.D., Deputy Director, ORDR, NCATS, NIH

9:00 a.m.  NIH Director Remarks
Francis S. Collins, M.D., Ph.D., Director, NIH (invited)

9:30 a.m.  Session 1: Gene Editing
Panel members will discuss the groundbreaking work and collaborative approaches that led to the first-in-human gene editing clinical trial for Hunter Syndrome, collectively providing clinical research, patient, and industry perspectives.

Panelist:
- Chester B. Whitley, Ph.D., M.D., Professor, Department of Pediatrics and Department of Experimental and Clinical Pharmacology, University of Minnesota; Principal Investigator, Lysosomal Disease Network

10:15 a.m.  Break

10:45 a.m.  Session 2: Collaborating for Successful Research
Patient groups play an essential role in advancing rare disease research. Patient organization representatives will discuss how collaborations improve understanding of these disorders and help advance development of new therapies.

Moderator: Marshall Summar, M.D., Chairman of the Board of Directors, National Organization of Rare Disorders; Chief, Division of Genetics and Metabolism, Children’s National Health System; Director, Rare Disease Institute

Panelists:
- Margie Frazier, Ph.D., LISW-S, Executive Director, Batten Disease Support and Research Association
- Theresa V. Strong, Ph.D., Co-Founder and Director of Research Programs, Foundation for Prader-Willi Research
- Salvatore La Rosa, Vice President, Research and Development, Children’s Tumor Foundation
11:45 a.m.  Lunch and Poster Session/NIH CC Tours

1:15 p.m.  **Session 3: Gene Therapy**
Gene therapy for rare diseases is moving forward rapidly. Panelists will share their perspectives on gene therapy scientific advances and research gaps, and on how to advance gene therapies for more rare diseases.

**Moderator:** Philip John (P.J.) Brooks, Ph.D., Program Director, Division of Clinical Innovation, NCATS, NIH

**Panelists:**
- Maria Kefalas, Co-Founder, Calliope Joy Foundation
- Jeffrey D. Marrazzo, M.B.A., M.P.A., Co-Founder and Chief Executive Officer, Spark Therapeutics, Inc.
- Kristin Smedley, President, Curing Retinal Blindness Foundation
- Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, Food and Drug Administration

2:00 p.m.  **Session 4: Engaging the Next Generation of the Rare Diseases Community**
This panel focuses on the perspective of youth and young adults with rare diseases. The panelists will share personal experiences and discuss how their generation can support research, access to care, or advocate for change using the power of social media, digital communications, and modern technology.

**Moderator:** Eric Sid, M.D., M.H.A., Presidential Management Fellow, ORDR, NCATS, NIH

**Panelist:**
- Shira Strongin, Founder, Sick Chicks

2:45 p.m.  Break

3:15 p.m.  **Congressional Remarks**

3:45 p.m.  **Summary and Highlights**
Petra Kaufmann, M.D., M.Sc., Director, ORDR, NCATS, NIH
Anne Pariser, M.D., Deputy Director, ORDR, NCATS, NIH

3:55 p.m.  **Closing Remarks**
Christopher P. Austin, M.D., Director, NCATS, NIH; Chair, IRDiRC
James K. Gilman, M.D., Chief Executive Officer, NIH CC

4:00 p.m.  Adjournment