Tentative Agenda  
As of Dec. 19, 2017

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  
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  
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  
- Kathleen Brewer, Founder and President, Erdheim-Chester Disease (ECD) Global Alliance  
- James O’Leary, M.B.A., Chief Innovation Officer, Genetic Alliance

11:45 a.m.  Lunch and Poster Session/NIH CC Tours
1:15 p.m.  **Session 3: Gene Therapy**

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

*Panelists:*
- Maria Kefalas, Co-Founder, Calliope Joy Foundation
- 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**

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