Tentative Agenda  
As of Dec. 12, 2018

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)

9:00 a.m. NCATS Office of Rare Diseases Research (ORDR) Update  
Anne R. Pariser, M.D., Director, ORDR, NCATS, NIH

9:20 a.m. Zebbie Award Presentation  
Anne R. Pariser, M.D., Director, ORDR, NCATS, NIH

9:30 a.m. Session 1: The Collective Research Model with the NIH Rare Diseases Clinical Research Network (RDCRN)  
Panel members will discuss the importance of collaborative research approaches for rare diseases. A diverse panel will share case studies and illustrative examples from the RDCRN. The RDCRN integrates academic investigators, patient groups, trainees, NIH scientific staff and others to accelerate rare diseases research.

Moderator: Tiina K. Urv, Ph.D., Program Director, ORDR, NCATS, NIH  
Panelists: TBD

10:30 a.m. Break

10:45 a.m. NIH Director Remarks  
Francis S. Collins, M.D., Ph.D., Director, NIH

11:00 a.m. Session 2: The Power of Patients — Harnessing Quality Registries to Understand Your Rare Disease  
An essential step in improving the diagnosis and treatment of a rare disease is to establish an understanding of the disease’s natural history. Natural history studies often provide foundational information for clinical research and drug development programs, and these studies often rely on active partnerships with patient organizations. Panel members will discuss their experiences in developing registries to understand rare diseases.

Moderator: Eric W.K. Sid, M.D., M.H.A., Presidential Management Fellow, ORDR, NCATS, NIH  
Panelists: TBD
**Noon**  
Lunch (*on your own*)  
- Themed Discussion Rooms  
- Poster Session & Exhibits  
- NIH CC Tours  
- Art/Photography Exhibition  
- Videos

**1:30 p.m. Session 3: Success Factors for Rare Cancer Research — Building Strong Foundations**  
Panelists will discuss the similarities and differences in the challenges that researchers face in rare cancers as compared to other rare diseases. Experts from NIH’s National Cancer Institute (NCI) and leading rare cancer advocates will describe their efforts to coordinate research both nationally and internationally, as well as different models of success for rare cancer advocacy and the role of social media in building rare cancer communities and recruiting patients for studies.

**NCI Organizers:** Karlyne M. Reilly, Ph.D., Abby B. Sandler, Ph.D., and Jack J. Welch, M.D., Ph.D.

**Moderator:** TBD

**Panelists:** TBD

**2:30 p.m.**  
Break

**2:50 p.m. Session 4: No Disease Left Behind, No Patient Left Behind**  
New technologies such as gene editing and genome therapy have potentially broad implications for many rare diseases. However, for clinical trials, there are practical challenges that need to be addressed to ensure that these technologies are accessible to all patients that might benefit from them. Panelists, including scientists, patients and other stakeholders, will discuss these issues.

**Moderator:** Philip John (P.J.) Brooks, Ph.D., Program Director, ORDR, NCATS, NIH

**Panelists:**  
- John F. Tisdale, M.D., Chief, Cellular and Molecular Therapeutics Branch, National Heart, Lung, and Blood Institute, NIH

**3:50 p.m. Closing Remarks**  
Christopher P. Austin, M.D., Director, NCATS, NIH; Chair, IRDiRC

**4:00 p.m.** Adjournment