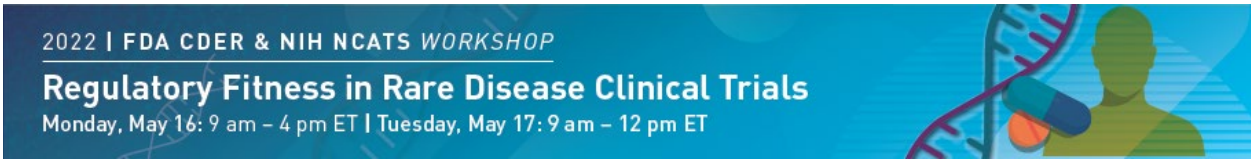



Event Website: https://www.fda.gov/drugs/news-events-human-drugs/fda-cder-nih-ncats-regulatory-fitness-rare-disease-clinical-trials-workshop-05162022

VideoCast (Day 1): https://videocast.nih.gov/watch=46190

**Day 1 (May 16, 2022) Timestamps**

**0:00:15** Welcoming Remarks – *Kerry Jo Lee, M.D. and Philip John (P.J.) Brooks, Ph.D.*

**Session 1**

**0:04:03** Approach to Demonstrating Substantial Evidence of Effectiveness for Rare Disease Drug Products
**0:05:17** Approach to Demonstrating Substantial Evidence of Effectiveness for Rare Disease Drug Development: Overview Considerations – *Janet Maynard, M.D., M.H.S*.
**0:25:45** Demonstrating Substantial Evidence of Effectiveness – *Jennifer Rodriguez Pippins, M.D., M.P.H.*
**0:38:38** Role of Translational Science in Rare Disease Drug Development – *Jeff Siegel, M.D.*
**0:56:37** Session 1 Q&A

**Session 2**

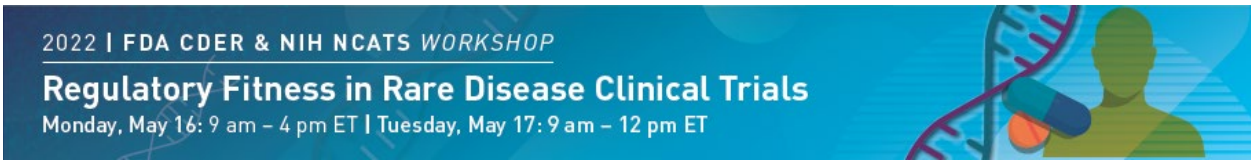
**1:37:35** Case Studies — An Academic Perspective
**1:39:10** Hutchinson-Gilford Progeria Syndrome: An ultra-rare disease pathway to drug approval – *Leslie B. Gordon, M.D., Ph.D.*
**2:03:52** sBLA: Anakinra and Rilonacept in DIRA – *Raphaela T. Goldbach-Mansky, M.D., M.H.S.*
**2:21:21** Baricitinib in CANDLE Patients – *Bita Shakoory, M.D.*
**2:49:41** Session 2 Q&A

**Session 3**

**2:57:21** Core Principles for Clinical Trials
**2:58:31** Dose Optimization for Rare Diseases – *Jie (Jack) Wang, Ph.D.*
**3:16:55** Core Principles for Clinical Trials – *Katie Donohue, M.D., M.Sc.*
**3:38:15** Statistical Considerations in Rare Disease Clinical Trials – *Yan Wang, Ph.D.*
**4:02:10** Session 3 Q&A

**Session 4**

**4:20:04** Case Studies — Real World Experiences
**4:20:56** From biomarker to study to basket: trials and tribulations of advancing science from the bedside or bench to trials: two models in academia – *Andrea L. Gropman, M.D*.
**4:40:28** Brittle Bone Disorders Consortium: Translating Discoveries to Therapy and Clinical trial Readiness – *Brendan H.L. Lee, M.D., Ph.D.*
**5:03:27** Resolving Disease Heterogeneity for Targeted Therapies in Rare Glomerular Disease: From syndrome disease classes to precision medicine trials – *Matthias Kretzler, M.D.*
**5:21:58** Session 4 Q&A

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VideoCast (Day 2): https://videocast.nih.gov/watch=46191

**Day 2 (May 17, 2022) Timestamps**

**0:00:25** Welcome **–** *Kerry Jo Lee, M.D.*

**Session 5**

**0:03:15** The Nuts and Bolts of Investigational New Drug (IND) Applications and Additional Considerations
**0:05:20** Understanding the Investigational New Drug (IND) Application Process – *Mari Suzuki, M.D. and Margaret Kober, R.Ph., M.B.A.*
**0:43:13** Pediatric Considerations in Rare Disease Drug Development – *Shamir Tuchman, M.D., M.P.H.*
**1:07:06** Nonclinical Perspective on the Development of Drugs for Rare Diseases – *Arianne L. Motter, Ph.D., DABT*
**1:33:10** Session 5 Q&A

**Session 6**

**2:02:07** Additional Pathways to Interact with FDA CDER
**2:03:07** Critical Path Innovation Meetings (CPIM) – *Chekesha Clingman-Henry, Ph.D., M.B.A.*
**2:11:46** Patient-Focused Drug Development – *Robyn Bent, R.N., M.S.*
**2:29:00** Session 6 Q&A

**2:43:03** Closing Remarks – *Kerry Jo Lee, M.D. and Alice Chen Grady, M.D.*