REAGAN-UDALL FOUNDATION FOR THE FDA

Natural History Studies and Registries in the Development of Rare Disease Treatments

(hybrid public workshop)

May 13, 2024

10am **Welcome & Opening Remarks**
Patrizia Cavazzoni, MD, Center for Drug Evaluation and Research, FDA
Susan C. Winckler, RPh, Esq., Reagan-Udall Foundation for the FDA

10:15am "***What Are Registries and Natural History Studies?"***
Dominique Pichard, MD, MS, National Center for Advancing Translational Sciences, NIH

"Why Registries and Natural History Studies are Critical to Rare Disease Treatment Development"
Kerry Jo Lee, MD, Center for Drug Evaluation and Research, FDA

10:30am **Getting Started: Developing Registries and Designing Natural History Studies**
Leslie Gordon, MD, PhD, The Progeria Research Foundation
Eileen King, PhD, Cincinnati Children's Hospital Medical Center
Michael Wagner, PhD, Cincinnati Children's Hospital Medical Center
Kristen Wheeden, MBA, United Porphyrias Association

Q&A Session

11:25am **Addressing Challenges in Registry and Natural History Data Collection**
Benjamin Forred, MBA, ACRP-CP, Sanford Research
Zohreh Talebizadeh, PhD, Global Genes

Reactor Panel
Henry Kaminski, MD, George Washington University
Suzanne Pattee, Office of the Commissioner, FDA
Dominique Pichard, MD, MS, National Center for Advancing Translational Sciences, NIH

12:25pm **Funding Opportunities**
Tiina Urv, PhD, National Center for Advancing Translational Sciences, NIH
Katherine Needleman, PhD, RAC, Office of Orphan Products Development, FDA

12:40pm **LUNCH**

1:35pm **Collecting Fit for Purpose Data to Inform Regulatory Decision Making**
Jennifer Farmer, MS, Friedreich's Ataxia Research Alliance

Collin Hovinga, PharmD, MS, FCCP, Critical Path Institute

Reactor Panel
Benjamin Forred, MBA, ACRP-CP, Sanford Research
Donna Rivera, PharmD, MSc, Oncology Center of Excellence, FDA
Kimberly Smith, MD, MS, Center for Drug Evaluation and Research, FDA
Tiina Urv, PhD, National Center for Advancing Translational Sciences, NIH

2:35pm **Natural History Studies and Registries that Informed Regulatory Decision Making**
Example: Nulibry for molybdenum cofactor deficiency
Ronen Spiegel, MD, Emek Medical Center
Liza Squires, MD, Sentynl Therapeutics

Example: Lumasiran and Nedosiran for Primary Hyperoxaluria
John Lieske, MD, Mayo Clinic Hospital – Rochester

Reactor Panel
Catherine Lerro, PhD, MPH, Oncology Center for Excellence, FDA
Kirtida Mistry, MBBCh, DCH, MRCPCH, Center for Drug Evaluation and Research, FDA
Jill Morris, PhD, National Institute of Neurological Disorders and Stroke, NIH
Catherine Pilgrim-Grayson, MD, MPH, Center for Drug Evaluation and Research, FDA

3:45 pm **Closing Remarks**