Meeting Agenda

EVERYLIFE FOUNDATION FOR RARE DISEASES | Scientific Workshop

Making What Matters Count: Advancing Patient-Centered Rare Disease Therapy Development Methodologies, Tools, and Knowledge Management

May 12, 2026 | 9:00 am- 4:30 pm | National Press Club, Washington DC

Scan QR code for Speaker Biographies

9:00 am – 9:15 am: Opening Remarks

Welcome and Goals for the Day: **Jamie Sullivan**, MPH – Senior Vice President of Policy and Advocacy, EveryLife Foundation for Rare Diseases

9:15 am – 10:00 am: Opening Panel - Setting the Goals for the Day: *Roadmaps have been built but how do we create freeways?*

Moderator: Cartier Esham, PhD – Founder & CEO, Esham Strategies, LLC

Terry Jo Bichell, PhD, MPH – CEO, COMBINEDBrain
Ryan Fischer – Chief Operating Officer, Foundation for Angelman Syndrome Therapeutics
Kimberly Lin, MD – Attending Cardiologist and Associate Medical Director of the Heart Function, Transplant, and Ventricular Assist Device Program, Children's Hospital of Pennsylvannia
Kim Quaintance-Lunn – Vice President, Head of Regulatory Science and Execution, Alexion
Jewell Martin, MA, MBA, PMP, RAC – Director, US Regulatory Policy, BioMarin Pharmaceutical

10:00 am – 11:00 am: Finding the Throughlines: FDA Perspectives on Patterns in Rare Disease Development

Moderator: James Valentine, JD, MHS – Director, Hyman, Phelps, & McNamara, P.C.

Kathleen Davies, JD, MS – Senior Advisor, CDER, FDA
Emily Freilich, MD – Director, Division of Neurology 1, CDER, FDA
Rosa Sherafat, MD – Deputy Director, Office of Clinical Evaluation, Office of Therapeutic Products, CBER, FDA
Lynne Yao, MD – Director, Division of Pediatric and Maternal Health, CDER, FDA

11:00 am – 11:15 am: Break

11:15 am – 12:15 pm: Measuring What Matters: Enabling Earlier Endpoints to Unlock Accelerated Approval

Moderator: Kristin Van Goor, PhD, MPhil – Executive Director and US Head, Global Regulatory Policy and Innovation, Takeda Pharmaceutical

Sorin Fedeles, PhD, MBA – Executive Director, Polycystic Kidney Disease Outcomes Consortium, Critical Path Institute
Stephanie Fradette, PharmD – Senior Vice President, Head of Neuromuscular Development Unit, Biogen
Tim Franson, MD – Principal, Faegre Drinker Consulting
Peter Stein, MD – NextStage Pharma Consulting, LLC

12:15 pm – 1:00 pm: Lunch

1:00 pm – 2:00 pm: Measuring What Matters: Enabling Development and Utilization of Patient-Relevant Clinical Endpoints

Moderator: Jewell Martin, MA, MBA, PMP, RAC – Director, US Regulatory Policy, BioMarin Pharmaceutical

Elizabeth Berry-Kravis, MD, PhD – Professor of Pediatrics, Neurological Sciences and Anatomy/Cell Biology, Rush University Medical Center
Terry Jo Bichell, PhD, MPH – CEO, COMBINEDBrain
Jennifer Pangoulias – Head of Regulatory and Policy, Co-director of ABOM, Foundation for Angelman Syndrome Therapeutics
Charlene Son Rigby, MBA – CEO, Global Genes
Michelle Campbell, PhD, MS – Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, CDER, FDA

2:00 pm – 3:00 pm: Evaluating What Matters: Innovative Trial Design Approaches to Enable Benefit Capture and Broad Patient Inclusion

Moderator: Heather Gordish-Dressman, PhD, MS – Research Professor of Pediatrics and the Center for Health Outcomes Research and Delivery Science, Children's National Hospital

Ramona Belfiore-Oshan, PhD – Executive Director Duchenne Regulatory Science Consortium, Critical Path Institute
Jenny Carwile, ScD, MPH – Global Medical Director, Medical Evidence Generation, Sanofi
Kent Christopherson, PhD – Head of Global Medical Affairs, Orchard Therapeutics
Marshall Summar, MD – CEO, Uncommon Cures, LLC
Naomi Lowy, MD – Principal Drug Regulatory Expert, Hyman, Phelps, & McNamara P.C.

3:00 pm – 3:15 pm: Break

3:15 pm – 4:15 pm: Aligning on What Matters: Increasing Cross-Stakeholder Understandings About Evidentiary Thresholds and Regulatory Requirements

Moderator: Annie Kennedy – Chief Mission Officer, EveryLife Foundation for Rare Diseases

Michelle Campbell, PhD, MS – Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, CDER, FDA
Katherine Donigan, PhD, MPhil – Executive Director, Science and Regulatory Policy, Sarepta Therapeutics
Emily Milligan, MPH – Chief Executive Officer, Barth Syndrome Foundation
Cara O'Neill, MD, FAAP – Chief Scientific Officer, Cure Sanfilippo Foundation
Kristin Van Goor, PhD, MPhil – Executive Director and US Head, Global Regulatory Policy and Innovation, Takeda Pharmaceutical
Pei Wang, PhD – Senior Vice President, Clinical Development & CMC, Eureka Therapeutics

4:15– 4:30 pm: Returning to the Goals – Priority Actions to Achieve Workshop Goals

Nick Manetto – Principal, Faegre Drinker Consulting
Kim McCleary – Founder and CEO, KithCollective
James Valentine, JD, MHS – Director, Hyman, Phelps, & McNamara, P.C.

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