LET'S GET REAL: Harnessing Non-Proprietary Patient Registries and RWE to Accelerate Rare Disease Drug Development

SEPTEMBER 2020 WEBINAR SERIES

Hosted by Penn Medicine Orphan Disease Center and Amicus Therapeutics

This webinar series will review the role of patient registries and high-quality natural history data in advancing clinical development of rare disease therapeutics, with a focus on potentially curative technologies such as gene therapy and genome editing.

Session 1 - September 8, 2020
BEYOND THE TOWER OF BABEL: FDA LEADERSHIP ON DATA STANDARDS

Session 2 - September 15, 2020
LET'S STOP REINVENTING THE WHEEL: SCALING BEST PRACTICES FOR PATIENT REGISTRIES

Session 3 - September 22, 2020
LET'S GET REAL (WORLD EVIDENCE): APPLICATIONS FROM THE ONCOLOGY COMMUNITY

Session 4 - September 29, 2020
FROM BROOKLYN TO BEIJING: GLOBAL RARE DISEASE REGISTRIES AS GLOBAL LEARNING PLATFORMS

Hosted by

Amicus Therapeutics
At the Forefront of Therapies for Rare and Orphan Diseases

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the orphan disease center

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AGENDA AND SPEAKERS

SESSION 1 | BEYOND THE TOWER OF BABEL: FDA LEADERSHIP ON DATA STANDARDS

Tuesday, September 8, 2020
10:00am - Noon (Eastern)

Public Session	10:00am-10:15am	Patient Voice & Chief Provocateur	Tracy Dixon-Salazar, PhD, Director of Research and Strategy, Lennox-Gastaut Syndrome (LGS) Foundation Craig Lipset, Managing Partner, Clinical Innovation Partners
	10:15am-11:15am	Panel Response and Discussion	Anthony Philippakis, Chief Data Officer, Institute Scientist, Broad Institute of MIT and Harvard, Board Member, RARE-X Elizabeth Hart, Branch Chief, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration Eric Sid, Program Officer, Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health Suzanne Thornton-Jones, Chief Regulatory and Compliance Officer, University of Pennsylvania Gene Therapy Program
	11:15am	Public Session Wrap-Up and Adjourn	
Closed Session	11:30am-Noon	Small Group Digital Whiteboarding: Action Items and Next Steps	Moderator: Anne Pariser, Director, Office of Rare Diseases, National Center for Advancing Translational Sciences
	Noon	Full Adjourn	

SESSION 2 | LET'S STOP REINVENTING THE WHEEL: SCALING BEST PRACTICES FOR PATIENT REGISTRIES

Tuesday, September 15, 2020
10:00am - Noon (Eastern)

Public Session	10:00am-10:15am	Chief Provocateur	Andrew E. Mulberg, Adult and Pediatric IBD Drug Development Consultant; fmr Deputy Director of the Division of Gastroenterology and Inborn Errors Products, U.S. Food and Drug Administration (2010-16)
	10:15am-11:15am	Panel Response and Discussion	Eric Zuckerman, Board Chairman, Pediatric IBD Registry; Chair, Children's Registry for the Advancement of Therapeutics (CREATE) Jane Larkindale, Executive Director, Rare Disease Cures Accelerator, Critical Path Institute Laura Schanberg, Professor of Pediatrics, Department of Pediatrics, Duke University; Childhood Arthritis and Rheumatology Research Alliance (CARRA) Network Betsy Bogard, Board Member, RARE-X; Program Leader, Research Committee Chair, International FOP Association
	11:15am	Public Session Wrap-Up and Adjourn	
Closed Session	11:30am-Noon	Small Group Digital Whiteboarding: Action Items and Next Steps	Proposed Moderator: David Fajgenbaum, Penn Medicine, Orphan Disease Center
	Noon	Full Adjourn	

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AGENDA AND SPEAKERS

SESSION 3 | LET'S GET REAL (WORLD EVIDENCE): APPLICATIONS FROM THE ONCOLOGY COMMUNITY

Tuesday, September 22, 2020
10:00am - Noon (Eastern)

Public Session	10:00am-10:15am	Chief Provocateur	Jonathan Hirsch, Founder and President, Syapse
	10:15am-11:15am	Panel Response and Discussion	Gideon Blumenthal, VP, Global Regulatory Affairs, Merck Christopher Kim, Director, Observational Research (Epidemiology), Amgen Alicyn Campbell, Head of Digital Health Oncology R&D, AstraZeneca Jeff Allen, President and CEO, Friends of Cancer Research
	11:15am	Public Session Wrap-Up and Adjourn	
Closed Session	11:30am-Noon	Small Group Digital Whiteboarding: Action Items and Next Steps	Moderator: Brian Alexander, Chief Medical Officer, Foundation Medicine; President and co-founder of the Global Coalition for Adaptive Research
	Noon	Full Adjourn	

SESSION 4 | FROM BROOKLYN TO BEIJING: GLOBAL RARE DISEASE REGISTRIES AS GLOBAL LEARNING PLATFORMS

Tuesday, September 29, 2020
10:00am - Noon (Eastern)

Public Session	10:00am-10:30am	Fireside Chat	Amy Abernethy, Principal Deputy Commissioner, U.S. FDA Steve Usdin, Editor, BioCentury
	10:30am-10:45am	Moderated Audience Q&A	Amy Abernethy, Principal Deputy Commissioner, U.S. FDA Steve Usdin, Editor, BioCentury
	10:45am-11:15am	Moderated Discussion	"Are We a Small Group of Thoughtful Committed Citizens?" Moderator: Steve Usdin, Editor, BioCentury Elizabeth Powers, Vice President and Category Lead, Safety Evidence for Regulators, Real World Solutions, IQVIA Jill Weimer, Senior VP or Discovery Science at Amicus Therapeutics & Senior Director of Therapeutic Development at Sanford Researh Glen de Vries, co-CEO and co-Founder, Medidata Sean Khozin, Global Head of Data Strategy, Janssen
	11:15am- 11:30am	Concluding Mission Moment and Series Adjourn	Eric Marsh, Clinical Director, Orphan Disease Center