

CDRC Annual Meeting and Workshop

April 18-20, 2023

Hyatt Regency Crystal City

2799 Richmond Highway, Arlington, Virginia, United States, 22202

CDRC Annual Meeting and Workshop Day 1: Tuesday April 18

Registration and Breakfast - Continental breakfast will be provided	7:00 – 8:00 am
Introduction and welcome address: Rosie Lovett (NHS)	8:30 – 8:35 am
Paths to maximize use of existing drugs: Marco Schito (C-Path)	8:35 – 8:45 am
Cochairs: Marco Schito and John Liddicoat Lightning talks to introduce international programs <ul style="list-style-type: none"> • Rosie Lovett (NHS) • Heather Stone (FDA) • Sundee Agrawal (FDA) • Perdita Taylor Zapata (NICHD) • Sabine Grimm (REPO4EU) • Donald Lo (REMEDI4ALL) • Charlotte Asker-Hagelberg (Sweden - MPA) 	8:45 – 8:50 am 8:50 – 8:55 am 8:55 – 9:00 am 9:00 – 9:05 am 9:05 – 9:10 am 9:10 – 9:15 am 9:15 – 9:20 am
Questions/Answers	9:20 – 10:00 am
Break	10:00 – 10:30 am
Moderators: Rosie Lovett and Donald Lo What projects is NHS program seeking, how do we search for them <ul style="list-style-type: none"> - Rosie Lovett (NHS) Design and plans for the REMEDI4ALL infrastructure and portfolio <ul style="list-style-type: none"> - Donald Lo (REMEDI4ALL) STAMP Perspective <ul style="list-style-type: none"> - Charlotte Asker-Hagelberg (Sweden - MPA) BPCA Perspective <ul style="list-style-type: none"> - Perdita Taylor-Zapata (NICHD) 	10:30 – 10:35 am 10:35 – 10:40 am 10:40 – 10:45 am 10:45 – 10:50 am

<p>Panel 1: Candidate identification</p> <ul style="list-style-type: none"> • Heather Stone (FDA) • Sundee Agrawal (FDA) • Sabine Grimm (REPO4EU) • Marjon Pasmooij (Netherlands - MEB) • Charlotte Asker-Hagelberg (Sweden - MPA) • Perdita Taylor-Zapata (NICHD) 	10:50 am – 12:00 pm
Lunch	12:00 – 1:00 pm
<p>Cochairs: Marco Schito and David Simon</p> <p>Unleashing the Potential of Financial Orphans: Blueprint for a National Initiative</p> <ul style="list-style-type: none"> - Vikas Sukhatme (Emory Morningside Center for Innovative and Affordable Medicine) <p>Changes in European IP and regulatory system for medicines: A first swing at a drug repurposing framework</p> <ul style="list-style-type: none"> - Żaneta Zemła-Pacud (Polish Academy of Sciences) <p>An Innovation Surcharge to Fund the Repurposing of Generic Drugs</p> <ul style="list-style-type: none"> - James Robinson (UC Berkeley) <p>Using Interventional Pharmacoeconomics and Advance Market Commitments to Repurpose Generic Drugs with Cost Savings</p> <ul style="list-style-type: none"> - Savva Kerdelmidis (Crowd Funded Cures) 	<p>1:00 – 1:20 pm</p> <p>1:20 – 1:40 pm</p> <p>1:40 – 2:00 pm</p> <p>2:00 – 2:20 pm</p>
Break	2:20-2:50 pm
<p>Moderators: Marco Schito and David Simon</p> <p>Drug Reimbursement by Public Payors – David Simon (Harvard)</p> <p>Panel 2: Challenges in validating RWD using RCTs?</p> <ul style="list-style-type: none"> • Patricia Van damme (Anticancer fund) • Amit Aggarwal (ABPI) • James Robinson (UCB) • Żaneta Zemla-Pacud (Polish Academy of Sciences) • Clare Thibodeaux (Cures Within Reach) • Vikas Sukhatme (Morningside) • Cynthia Adinig (Patient) <p>Next steps (close of open session)</p>	<p>2:50 – 3:05 pm</p> <p>3:05 – 4:30 pm</p> <p>4:30 – 4:40 pm</p>
<p>Cochairs: Rosie Lovett and Heather Stone</p> <p>Closed session with with publicly funded programs</p>	4:40 – 5:30 pm

CDRC Annual Meeting Day 2: Wednesday April 19

Registration and Breakfast - Continental breakfast will be provided	7:00 – 8:00 am
Morning: Meeting kickoff on automating data extraction from EHRs Welcome and Overview of the topic: Marco (C-Path) and Heather Stone (FDA)	8:00-8:15 am
Keynote Automating Electronic Health Record Data Extraction: Challenges and Opportunities - Jacqueline Corrigan-Curay (FDA)	8:15-8:45 am
Cochairs: Jagdeep Podichetty (C-Path) & Matthew Robinson (JHU) Extracting data from electronic medical records Cure ID with the OHDSI Edge Tool Suite: Automating Data Extraction from Electronic Medical Records - Danielle Boyce (JHU) Leveraging AWS Resources and Natural Language Processing to Develop an End-to-End Data and Analytics Pipeline for CURE-ID - Wes Anderson (C-Path) Exploring the potential of causal inference modeling in CURE ID to replicate clinical trial findings - Ruth Kurtycz (CDC) Methodology for Validating a Minimal Dataset - Kerry Howard (Clemson)	8:45-9:00 am 9:00-9:15 am 9:15-9:30 am 9:30-9:45 am
Break	9:45-10:00 am
Moderators: Ewy Mathe (NCATS) Raghav Tirupathi (C-Path) COVID-19 in the Real World: Preliminary Results from the Edge Tool Suite - Smitty Heavner (C-Path) Roundtable Discussions: Real world experience on extracting and analyzing data from EHRs <ul style="list-style-type: none">○ Laura Evans (SCCM)○ Nathalie Strub-Wourgaft (DNDi)○ Matt Robinson (JHU)○ Anup Challa (AstraZeneca) Wrap up and next steps	10:00-10:15 am 10:15-11:45 am 11:45 am -12:00 pm
Lunch on your own	12:00-1:00 pm
Afternoon: Pragmatic adaptive platform trials Cochairs: Marco Schito (C-Path) and Leonard Sacks (FDA) Welcome: Marco (C-Path) and Heather Stone (FDA)	1:00-1:15 pm

<p>Keynote From Chasing My Cure to Chasing Every Cure: Unlocking the lifesaving potential of approved medicine - David Fajgenbaum (Every Cure and CDRC AC Co-Chair)</p>	1:15-1:45 pm
<p>Undiagnosed Diseases Network (UDN): Discovering Rare Disease Therapies Through Team Science - Sessions Cole (WUSTL)</p>	1:45-2:00 pm
<p>Moderators: David Fajgenbaum (U Penn) and Heather Stone (FDA) Critical considerations for clinical trials in DR - Heather Stone (FDA) Roundtable discussions: Inpatient trials and innovations in embedding trials in practice</p> <ul style="list-style-type: none"> ○ Trevan Locke (Duke Margolis) ○ Jon Sevransky (Emory) ○ Stacey Coe (C-Path) ○ Clare Thibodeaux (Cures Within Reach) ○ Cynthia Adinig (Long COVID Patient) 	2:00-2:15 pm 2:15-3:15 pm
Break	3:15-3:30 pm
<p>Outpatient/decentralized/patient-centric trials</p> <ul style="list-style-type: none"> ○ Chris Lindsell (Duke) ○ Nathalie Strub-Wourgaft (DNDi) ○ Suanna Bruinooge (ASCO) ○ Oved Amitay (Solve CFS) ○ Amy Morris (IND 2 Results) ○ Vidula Sukhatme (GlobalCures) ○ Michael Sieverts (Long-COVID Patient) ○ Ingrid Oakley-Girvan (Medable) 	3:30 - 4:30 pm
Wrap up and next steps	4:30-4:45 pm
Evening: Welcome reception (optional)	6:00-8:00 pm

CDRC Annual Meeting Day 3: Thursday April 20

Registration and Breakfast - Continental breakfast will be provided	7:00 – 8:00 am
Morning: closed door meetings CDRC Advisory Committee	8:30-10:00 am
Break	10:00-10:15 am
Session with FDA and NIH	10:15-11:45 am
Lunch on your own	12:00-1:00 pm
Afternoon: Rare Cancer (Sarcomas) Welcome Marco Schito (C-Path)	1:00-1:15 pm
Cochair: Marco Schito (C-Path) and Bill Tap (MSKCC) Sarcoma: The Evolution of Care in Rare Cancer: Establishing a Comprehensive Unified Approach - Bill Tap (MSKCC) The Role of Patient Advocates in Generating Real World Data in Ultra Rare Cancer - Denise Robinson (The EHE Foundation) An EHR-connected Patient-Centric Registry for Rare Cancer Research - Mark Shapiro (xCures) Opportunities to Impact Patients Through Clinical Repurposing Trials - Clare Thibodeaux (Cures Within Reach) Repurposed Drug Trials: Challenges and Opportunities - Vidula Sukhatme (GlobalCures)	1:15-1:45 pm 1:45-2:00 pm 2:00-2:15 pm 2:15-2:30 pm 2:30-2:45 pm
Break	2:45-3:00 pm
Moderator: Bill Tap (MSKCC) and Marco Schito (C-Path) Harnessing RWD to advance repurposed drugs for rare cancers. Panel Discussion <ul style="list-style-type: none"> • Brandi Felser (SFA) • Christine Heske (NCI) • Vidula Sukhatme (GlobalCures) • Andrea Gross (NCI) • Suanna Bruinooge (ASCO) • Lennie Woods (Clear Cell Sarcoma Foundation) Next steps	3:00-3:15 pm 3:15-4:45 pm 4:45-5:00 pm
Adjourn	5:00 pm