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

- Introduction and welcome address: Rosie Lovett (NHS) 8:30 – 8:35
- Paths to maximize use of existing drugs: Marco Schito (C-Path) 8:35 – 8:45

Cochairs: Marco Schito and John Liddicoat

- Lightning talks to introduce international programs
  - Rosie Lovett (NHS) 8:45 – 8:50
  - Heather Stone (FDA) 8:50 – 8:55
  - Sundeep Agrawal (FDA) 8:55 – 9:00
  - Perdita Taylor Zapata (NICHD) 9:00 – 9:05
  - Sabine Grimm (REPO4EU) 9:05 – 9:10
  - Donald Lo (REMEDI4ALL) 9:10 – 9:15
  - Charlotte Asker-Hagelberg (Sweden - MPA) 9:15 – 9:20
  - Marjon Pasmooij (Netherlands - MEB) 9:20 – 9:25
  - Questions/Answers 9:25 – 10:00

- BREAK 10:00 – 10:30

Moderators: Rosie Lovett and Donald Lo

- What projects is NHS program seeking, how do we search for them - Rosie Lovett (NHS) 10:30 – 10:35
- Design and plans for the REMEDI4ALL infrastructure and portfolio - Donald Lo (REMEDI4ALL) 10:35 – 10:40
- STAMP Perspective - Charlotte Asker-Hagelberg (Sweden - MPA) 10:40 – 10:45
- BPCA Perspective - Perdita Taylor-Zapata (NICHD) 10:45 – 10:50
- Panel 1: Candidate identification 10:50 – 12:00
  - Heather Stone (FDA)
  - Sundeep Agrawal (FDA)
  - Sabine Grimm (REPO4EU)
  - Marjon Pasmooij (Netherlands - MEB)
  - Charlotte Asker-Hagelberg (Sweden - MPA)
  - Perdita Taylor-Zapata (NICHD)
Co-chairs: Marco Schito and David Simon

- Unleashing the Potential of Financial Orphans: Blueprint for a National Initiative - **Vikas Sukhatme (Emory Morningside Center for Innovative and Affordable Medicine)**  
  1:00 – 1:20
- Changes in European IP and regulatory system for medicines: A first swing at a drug repurposing framework - **Żaneta Zemla-Pacud (Polish Academy of Sciences)**  
  1:20 – 1:40
- An Innovation Surcharge to Fund the Repurposing of Generic Drugs - **James Robinson (UC Berkeley)**  
  1:40 – 2:00
- Using Interventional Pharmacoeconomics and Advance Market Commitments to Repurpose Generic Drugs with Cost Savings - **Savva Kerdemelidis and Jason Cross (Crowd Funded Cures)**  
  2:00 – 2:20

**BREAK**  
2:20-2:50

Moderators: Marco Schito and David Simon

- Drug Reimbursement by Public Payors – **David Simon (Harvard)**  
  2:50 – 3:05
- Panel 2: Challenges in validating RWD using RCTs?  
  3:05 – 4:30
  - **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 (Emory Morningside Center for Innovative and Affordable Medicine)**
  - **Cynthia Adinig (Patient)**
- Next steps (close of open session)  
  4:30 – 4:40

Co-chairs: Rosie Lovett and Heather Stone

- Closed session with with publicly funded programs  
  4:40 – 5:30
CDRC Annual Meeting Day 2: Wednesday April 19

- **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)
  - Keynote
    - Automating Electronic Health Record Data Extraction: Challenges and Opportunities - **Jacqueline Corrigan-Curay (FDA)**  
      (8:15-8:45)

**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)**  
    (8:45-9:00)
  - Leveraging AWS Resources and Natural Language Processing to Develop an End-to-End Data and Analytics Pipeline for CURE-ID - **Wes Anderson (C-Path)**  
    (9:00-9:15)
  - Exploring the potential of causal inference modeling in CURE ID to replicate clinical trial findings - **Ruth Kurtycz (CDC)**  
    (9:15-9:30)
  - Methodology for Validating a Minimal Dataset - **Kerry Howard (Clemson)**  
    (9:30-9:45)

- **BREAK**  
  (9:45-10:00)

**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)**  
  (10:00-10:15)
- Roundtable: Real world experience on extracting and analyzing data from EHRs
  - Laura Evans (SCCM)
  - Nathalie Strub-Wourgaft (DNDi / PANTHER)
  - Matt Robinson (JHU)
  - Anup Challa (AstraZeneca)
  (10:15-11:45)

- **LUNCH**  
  (12:00-1:00)

- **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)
  - 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)**  
      (1:15-1:45)
  - Undiagnosed Diseases Network (UDN): Discovering Rare Disease Therapies Through Team Science - **Sessions Cole (WUSTL)**  
    (1:45-2:00)
Moderators: David Fajgenbaum (U Penn) and Heather Stone (FDA)

- Critical considerations for clinical trials in drug repurposing
  - Heather Stone (FDA)  
  (2:00-2:15)
- Roundtable discussions
  - Inpatient trials and innovations in embedding trials in practice  
    - Trevan Locke (Duke Margolis)
    - Jon Sevransky (Emory)
    - Stacey Coe (C-Path)
    - Clare Thibodeaux (Cures Within Reach)
    - Cynthia Adinig (Long COVID Patient)  
    (2:15-3:15)
- BREAK  
  (3:15-3:30)
- Outpatient/decentralized/patient-centric trials  
  - Chris Lindsell (Duke)
  - Nathalie Strub-Wourgaft (DNDi / PANTHER)
  - 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)
- Wrap up and next steps  
  (4:30-4:45)
- Evening: Welcome reception (optional)  
  (6:00-8:00)
CDRC Annual Meeting Day 3: Thursday April 20

• Morning: closed door meetings
  - CDRC Advisory Committee (8:30-10:00)

• BREAK (10:00-10:15)
  - Session with FDA and NIH (10:15-11:45)

• LUNCH (12:00-1:00)

• Afternoon: Rare Cancer (Sarcomas)
  - Welcome Marco Schito (C-Path) (1:00-1:15)
  - 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) (1:15-1:45)
    • The Role of Patient Advocates in Generating Real World Data in Ultra Rare Cancer - Denise Robinson (The EHE Foundation) (1:45-2:00)
    • An EHR-connected Patient-Centric Registry for Rare Cancer Research - Mark Shapiro (xCures) (2:00-2:15)
    • Opportunities to Impact Patients Through Clinical Repurposing Trials - Clare Thibodeaux (Cures Within Reach) (2:15-2:30)
    • Repurposed Drug Trials: Challenges and Opportunities - Vidula Sukhatme (GlobalCures) (2:30-2:45)

• BREAK (2:45-3:00)

  - Moderator: Bill Tap (MSKCC) and Marco Schito (C-Path)
    • Harnessing RWD to advance repurposed drugs for rare cancers (3:00-3:15)
    • Panel Discussion (3:15-4:45)
      - Brandi Felser (SFA)
      - Christine Heske (NCI)
      - Vidula Sukhatme (GlobalCures)
      - Andrea Gross (NCI)
      - Suanna Bruinooge (ASCO)
      - Lennie Woods (Clear Cell Sarcoma Foundation)
    • Next steps (4:45-5:00)

• Adjourn (5:00)
Posters

SC You, B Malik, J Kyulee, T Alkasab, P Mallol, and P Nagy. Development of the medical imaging extension. (Johns Hopkins)


S Heavner, T Llano, Z Wang, M Schito, H Stone, P Dasher, T Russel, V Kumar, B Saeks, M Cooke, R Kashyap, M Robinson, P Nagy. Lowering the OMOP ETL Barrier for Clinical Registries. (C-Path)

MG Bowring, M Cook, S Lui, K Aziz, A Nishimura, P Nagy. One institution’s approach to empowering researchers to learn and conduct observational research. (Johns Hopkins)

R Charles, B Milani, D Argaw, N Oliver, B Ali, H Stone, M Schito. Landscape Analysis to Identify Effective Drug Repurposing Candidates for the Treatment of Implantation Mycoses: Comparison of World Health Organization Survey Treatment Data and Published Case Reports on CURE ID. (ORISE/FDA)

T Farid, R Charles, K Tumas, H Stone and R Tirupathy. The Landscape of Rare Infectious Diseases – A Proof of Concept with Rare Bacterial Infections. (ORISE/FDA)

S. Strongin. Repurposing in RASopathies. (ORISE/FDA)