

Cure Drug Repurposing Collaboratory

Annual Meeting

November 16-18, 2021

Final Agenda

Day 1: Session 1 and 2 – Rare diseases

- Success stories and lessons learned...using old drugs in novel new ways.
 - Stakeholder perspectives and viewpoints.
 - Opportunities for repurposing drugs in rare diseases and rare cancers.
 - Using real-world data, what process should be used to determine whether the signal should be confirmed in a randomized clinical trial.
-

Day 1: session 1

November 16, 2021 – (9:00 am to 11:00 am Eastern Time)

Topic: Rare Diseases

Moderators: Matt Might (UAB) + Shira Strongin (FDA)

Panelists:

1. Andy Crouse (UAB)
2. Ethan Perlstein (Perlara)
3. Eva Morava-Kozicz (Mayo)
4. Lisa Schill (RASopathies Network)
5. Omid Karkouti (Rarebase)
6. Clare Thibodeaux (Cures Within Reach)
7. Sandra Sermone (ADNP Kids Research Foundation)

Discussion Topics: TBA

Presenter	Affiliation	Presentation title
Matthew Might	UAB	<i>Repurposing Case Study</i>
Shira Strongin	FDA	<i>Disease of Interest Summary</i>
Sandra Sermone	ADNP Kids Research Foundation	AI tool predicted ketamine may help ADNP-driven autism
Ethan Perlstein	Perlara PBC	Repurposed drug to successfully treat PMM2-CDG with epalrestat – Industry perspective
Eva Morava -Kozicz	Mayo	Repurposed drug to successfully treat PMM2-CDG with epalrestat – Clinician perspective

Day 1: session 2

November 16, 2021 – (3:00 pm to 5:00 pm Eastern Time)

Topic: Rare Oncology

Moderators: Leslie Doros (FDA) and Sonia Singh (FDA)

Panelists:

1. Bill Tap (MSKCC)
2. Denise Reinke (U Michigan)
3. Kris Ann Schultz (Children's MN)
4. Pan Pantziarka (Anticancer Fund)
5. Breelyn Wilky (U Colorado)

Discussion Topics: TBA

Presenter	Affiliation	Presentation title
Leslie Doros	FDA	Introduction and background for sarcomas
Bill Tap	MSKCC	The use of repurposed drugs in sarcoma and current examples
Denise Reinke	U Michigan	Patient perspective on drug repurposing for sarcoma
Kris Ann Schultz	Children's MN	Registries for rare tumors and CRFs
Pan Pantziarka	Anticancer Fund	Propranolol in Angiosarcoma - the story so far

Day 2: Session 3 and 4 – Special Populations and Regulatory Challenges

- Use of real-world data when clinical trials are not feasible due to loss of equipoise.
 - Real-world data sources other than medical records, registries, and observational studies.
 - Programs initiated by regulatory authorities to advance drug repurposing.
 - What other opportunities such as financial incentives, patent law, and legislative action should be considered to help advance drug repurposing and potential unintended consequences.
-

Day 2: session 3

November 17, 2021 – (9:00 am to 11:00 am Eastern Time)

Topic: Special Populations

Moderators: Matt Laughon (UNC) + Kate Borkowski (FDA)

Panelists:

1. Anup Challa (Vanderbilt)
2. Rachel Greenberg (Duke)
3. Mili Duggal (FDA)
4. Matt Robinson (Hopkins)
5. Khyzer Aziz (Hopkins)
6. Perdita Taylor-Zapata (NICHD)
7. Kanwaljit Singh (INC)
8. Prabha Viswanathan (FDA)
9. Genny Taylor (UNC)
10. David Kimberlin (UAB)
11. Jason Lang (Duke)
12. Barbara Goodman (Cures Within Reach)

Discussion Topics: TBA

Presenters	Affiliation	Presentation title
Kate Borkowski	FDA	Ranking diseases in pregnancy for drug repurposing
David Kimberlin	UAB	Congenital CMV treatment options and existing guidelines/ Standing up a trial for valganciclovir, and FDA & BPCA
Jason Lang	Duke	Efficacy of montelukast in NICU for bronchopulmonary dysplasia
Mili Duggal	FDA	The need to develop survey tools
Rachel Greenberg	Duke	Accomplishments and challenges in pediatric drug development: The Pediatric Trials Network experience
Genny Taylor	UNC	Global Rank Score for use in real-world data

Day 2: session 4

November 17, 2021 – (3:00 pm to 5:00 pm Eastern Time)

Topic: Regulatory

Moderators: David Simon (Harvard) + Marco Schito (C-Path)

Panelists:

1. Jonathan Darrow (Harvard)
2. John Liddicoat (Cambridge)
3. Sundeep Agrawal (FDA)
4. Daniel O'Connor (MHRA)
5. Lydie Meheus (Anticancer Fund)
6. Heather Stone (FDA)
7. Perdita Taylor-Zapata (NICHD)
8. Nitin Bagul (TGA)
9. César Hernandez Garcia (AEMPS Spain)
10. Momir Radulović (Slovenian Medicines Agency)
11. Agnes Klein (Health Canada)

Discussion Topic: TBA

Presenters	Affiliation	Presentation title
Marco Schito	C-Path	Introduction and Workgroup Goals
Jonathan Darrow	Harvard	Issues regarding drug repurposing
John Liddicoat	Cambridge	Closing the repositioning gap with a resource hidden in plain site
Sundeep Agrawal	FDA	Project Renewal
Daniel O'Connor	MHRA	ILAP initiative
Lydie Meheus	Anticancer fund	Role of non-profits in advancing drug repurposing

Day 3: Session 5 – Extracting real-world data from electronic health records

- Lessons learned from the COVID-19 VIRUS disease registry.
 - Challenges in automating data extraction from electronic health records from US and non-US sites.
 - Opportunities and challenges in creating knowledgebases using automated data extraction from electronic health record data to identify how existing drugs can be optimized in different health care settings.
 - Despite recent advances, what barriers exist that prevent optimizing data sharing and what are the prospects for innovation.
-

Day 3: session 5

November 18, 2021 – (9:00 am to 12:00 pm Eastern Time)

Topic: Electronic Health Records

Panel 1

Moderators: Smith Heavner (C-Path) + Aysun Tekin (Mayo Clinic)

Panelists:

1. Rahul Kashyap (VIRUS Registry)
2. Matt Robinson (JHU)
3. Paul Nagy (JHU)
4. Matt Roe (Verana Health)
5. Laura Merson (Oxford)

Discussion Topic: “Lessons Learned in Registry Development”

- Selection of variables, data automation, site recruitment and retention, interpandemic period, challenges and regulatory barriers

Panel 2

Moderator: Laura Merson (Oxford) + Smith Heavner (C-Path)

Panelist:

1. Will Stevens (RECOVERY Trial, University of Oxford)
2. Ann-Marie Mallon (NHS Digital)
3. Kalynn Kennon (IDDO)
4. Miguel Pedrera Jimenez (Hospital 12 de Octubre, Madrid, Spain)

Discussion Topic: “Perspectives from Across the Pond”

- Mapping EHR data, data integration, use of EHR RWD for clinical trials and observational research, limitations of EHR for research questions

Presenters	Affiliation	Presentation title
Laura Merson	Oxford (UK)	International Perspective Barriers to Automating ISARIC/RECOVER
Miguel Pedrera Jimenez	Hospital Universitario, Madrid, Spain	Mapping EHR data to inform clinical characterization
Will Stevens	RECOVERY	Combining RWD and case report forms to deliver the RECOVERY trial
Paul Nagy, Matt Robinson	JHU	The Edge Tool
Rahul Kashyap, Smith Heavner	VIRUS	Automating the VIRUS COVID-19 Registry
