

C-Path's CDRC Meeting Highlights the Importance of Drug Repurposing

The Critical Path Institute's CURE Drug Repurposing Collaboratory and FDA hosted their annual meeting April 18-20 to bring together stakeholders in drug repurposing.

TUCSON, Ariz., May 4, 2023 — The CURE Drug Repurposing Collaboratory (CDRC) held a three-day annual meeting, April 18-20, in Crystal City, VA. The event, hosted for both live and virtual attendees, was tailored for drug repurposing stakeholders and featured expert presentations, panel discussions, keynote speakers and was attended by more than 100 patients, researchers, clinicians, biopharmaceutical company representatives, regulatory reviewers, and scientists.

CDRC was established through a partnership between Critical Path Institute (C-Path) and the U.S. Food and Drug Administration (FDA), in partnership with the National Center for Advancing Translational Sciences, part of the National Institutes of Health. This Collaboratory has grown to include dozens of partnerships between a variety of stakeholders in the infectious disease, rare disease, sarcoma, and academic communities. The Collaboratory serves as a centralized and standardized infrastructure to host and bring together groups interested in drug repurposing.

FDA's Jacqueline Corrigan-Curay, J.D, M.D., Principal Deputy Center Director of FDA's Center for Drug Evaluation and Research, delivered the keynote early on day two, which focused on the challenges and opportunities in automating electronic health record data extraction. "FDA can be a catalyst, but we need to promote collective efforts of academics, healthcare systems and drug developers to really realize the potential here," said Corrigan-Curray. "So, I really want to thank everyone for engaging and thank C-Path for bringing us all together in this important effort."

CDRC Advisory Committee Co-Chair David Fajgenbaum, M.D., M.Sc., MBA, co-founder of Every Cure, delivered his keynote, "From Chasing My Cure to Chasing Every Cure: Unlocking the lifesaving potential of approved medicine," which is an account of his personal experience with drug repurposing and how it saved his life. His full keynote presentation can be found here.

The keynotes were followed by panel discussions regarding the challenges and opportunities for extracting data from electronic health records (EHRs) and considerations for clinical trial innovation for repurposed drugs. The tools being developed to extract data from EHRs were further discussed in a panel session that included Laura Evans (University of Washington/Society of Critical Care Medicine), Nathalie Strub-Wourgaft (DNDi/PANTHER), Matt Robinson (Johns Hopkins) and Anup Challa (AstraZeneca). The panel on the critical considerations for clinical trials included innovations in embedding trials in clinical practice, included Trevan Locke (Duke Margolis), Jonathon Sevransky (Emory University), Stacey Coe (C-Path), Clare Thibodeaux (Cures Within Reach) and Cynthia Adinig (a Long COVID patient/ Co-Founder of BIPOC Equity Agency). Knowing that many patients are treated in community settings, a second panel discussed the

challenges for decentralized patient-centric trials and the potential for large simple pragmatic master protocols to address the diversity and inclusion challenge, as well as potentially reduce the high costs of traditional trials. Panelists included Chris Lindsell (Duke), Suanna Bruinooge (American Society of Clinical Oncology), Oved Amitay (Solve CFS), Amy Morris (IND 2 Results), Vidula Sukhatme (Global Cures), Michael Sieverts (Long COVID Patient) and Ingrid Oakley-Girvan (Medable).

The robust agenda included lightning talks to introduce international, publicly funded drug repurposing programs, real-world experience on extracting and analyzing data from EHRs, harnessing real-world data to advance repurposed drugs for rare cancers, as well as active discussions around CURE ID, data sharing and much more. In terms of rare cancers, a sarcoma project including clinicians from Memorial Sloan Kettering Cancer Center, several patient and patient advocates, data extraction partners like xCures, was the highlight on the last day with panelists outlining the unique challenges to advance treatments for these patients. The panel included contributions from Brandi Felser (Sarcoma Foundation of America), Christine Heske (National Cancer Institute), Vidula Sukhatme (GlobalCures), Andrea Gross (National Cancer Institute), Suanna Bruinooge (American Society of Clinical Oncology) and Lennie Woods (patient advocate and Executive Director and Co-Founder of Clear Cell Sarcoma Foundation).

A significant portion of the agenda featured the next steps that CDRC can take in the realm of drug repurposing and what is most impactful for patients. "One of the advantages of working with the FDA on CDRC is that they have developed the CURE ID platform to be able to capture data from clinical experiences," said CDRC Executive Director Marco Schito, Ph.D. "What we have found is that we have needed additional input into that data from patients and real-world data sources such as electronic health records, which helps drive the ability to find treatments for diseases of high unmet needs." Schito gave a presentation titled, "Paths to Maximize use of Existing Drugs," which concluded with a panel discussing challenges in validating real-world data using randomized controlled trials.

Heather Stone, Health Science Policy Analyst CDER/FDA, added, "Some of the potential advantages of combining real-world data and randomized controlled trials is, that sometimes it is the best of both worlds. You get the benefits of randomization and the benefits of more generalizable evidence."

Overall, the meeting emphasized how CDRC, and the CURE ID platform continuously integrate data and break down silos, allowing all stakeholders to have a seat at the "drug repurposing table."

A total of 55 speakers and panelists from patient organizations and regulatory, industry and academic fields participated in the meeting. A recording of the meeting presentations, panel discussions, and interviews of important stakeholders and their insight on the CDRC initiative will be available on C-Path's YouTube channel in the coming weeks.

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About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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