
FDA Awards C-Path Grant to Use Real-World Data to Generate Real-World Evidence in Neonates



Pilot project will include the development of a Real-World Data and Analytics Platform

TUCSON, Ariz., October 27, 2020 — Arizona-based Critical Path Institute (C-Path) is pleased to announce it has been awarded a multi-year grant by the U.S. Food and Drug Administration (FDA) to advance standards and methodologies designed to generate real-world evidence (RWE) from real-world data (RWD) through a neonatal pilot project through the International Neonatal Consortium (INC).

This grant will support the collection of neonatal intensive care unit (NICU) data from many key stakeholders worldwide which will be deposited into a Real-World Data and Analytics Platform (RW-DAP). Data will be used to define actionable reference ranges of commonly used laboratory values in neonates and the creation of a natural history model of bronchopulmonary dysplasia (a chronic lung disease common in preterm neonates).



Jonathan Davis, M.D.

“Each year, 10 percent of births are preterm in the United States and there is an urgent need to improve survival and clinical outcomes in this population,” said Jonathan Davis, M.D., Vice-Chair of Pediatrics and Chief of Newborn Medicine at Tufts Children’s Hospital, and Co-Principal Investigator on the grant. “Unfortunately, new drug development in neonates has been extremely limited. Many comprehensive datasets exist in NICUs, but a lack of systematic integration, data sharing, and data standards has greatly limited drug development for this vulnerable population.”

Recognizing this critical need, the FDA collaborated with C-Path in 2015 to create the INC with the goal of sharing and standardizing data and methods to advance actionable medical product development tools for endorsement by FDA and other global regulatory agencies. INC and its expert stakeholders (e.g. academic investigators, industry, nurses, regulators, and families) have developed a number of influential statements about aspects of neonatal drug development, covering the design and conduct of clinical pharmacology studies, safety assessment, seizures, long-term follow-up, as well as lung, eye and intestinal conditions unique to children born prematurely. INC and its members will partner with C-Path's Quantitative Medicine Program (QuantMed) and Data Collaboration Center (DCC) to execute this project.



Klaus Romero, M.D., M.S., F.C.P.

“C-Path’s DCC has substantial experience enabling the sharing and integration of clinical and non-clinical data to extrapolate new knowledge from existing data,” C-Path’s Chief Science Officer and project Co-Principal Investigator Klaus Romero, M.D., M.S., F.C.P. said. “DCC’s diverse team of data managers and scientists work to implement innovative data solutions to enable advances in regulatory and data science. This endeavor requires a significant amount of data, analytics and regulatory science expertise to fully optimize the use of RWD to extract actionable RWE. Through the work of C-Path’s QuantMed Program, DCC and INC, we plan to generate tangible solutions to accelerate clinical trials for therapies in neonates.”

“This project is likely to be transformative for the field of neonatology. This is something neonatologists have always wanted to do, but could not due to lack of resources,” Davis said. “FDA’s generous support for addressing the unmet drug development needs in neonates is greatly appreciated.”



Kanwaljit Singh, M.D., M.P.H.

“Neonates are therapeutic orphans and drug development efforts specific to the neonatal population have been sparse in the last several decades,” said INC Executive Director and project Co-Investigator Kanwaljit

Singh, M.D., M.P.H. “The electronic medical records data collected in this project will facilitate the design and conduct of clinical trials in this population. This collaborative effort with C-Path and INC partners will go a long way in addressing some of the reasons why neonates are still therapeutic orphans.”

INC will host its annual workshop, including a Real-World Data for Real-World Evidence in Neonatology session, October 28-29. Register at no cost: <https://bit.ly/INC2020Workshop>

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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 dozens of pharmaceutical and biotech companies. C-Path US is headquartered in Tucson, Arizona and C-Path, Ltd. EU is headquartered in Dublin, Ireland, with additional staff in multiple other locations. For more information, visit www.c-path.org and c-path.eu.

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