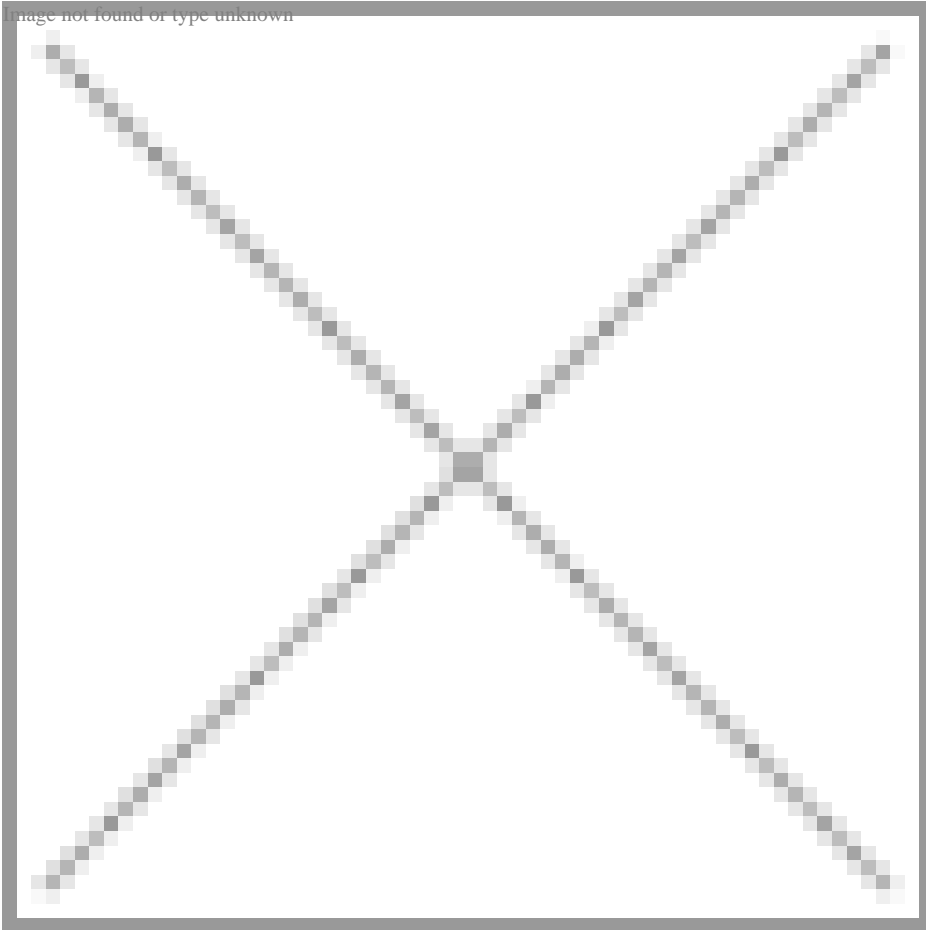


Funded by FDA, C-Path and NORD to Launch Rare Disease Data and Analytics Platform



The collaborative project between the organizations will kick off at a launch meeting in September and will aim to reduce barriers for the development of new treatments and cures for rare diseases

TUCSON, Ariz. and WASHINGTON, August 7, 2019 — The Critical Path Institute (C-Path) and the National Organization for Rare Disorders[®] (NORD) will host a meeting on Tuesday, September 17 in Bethesda, MD to formally launch development of a new rare disease data and analytics platform. Funded by a cooperative agreement through the Food and Drug Administration, [Critical Path Public-Private Partnerships Grant Number U18 FD005320 ?from the US Food and Drug Administration] the goal of the platform is to accelerate the movement of therapies from bench to bedside for rare diseases. The platform will provide the infrastructure for a sustainable, cooperative scientific approach to clinical trial readiness in rare diseases by addressing vast knowledge gaps about the natural course of disease, the clinical evaluation

of new treatments, and patients' perspective on disease and treatment.

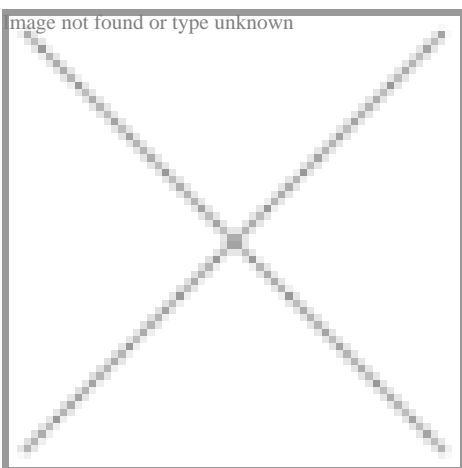
The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) will provide a centralized and standardized infrastructure to support and accelerate rare disease characterization with the goal of accelerating therapy development. The robust integrated platform will include integrated rare disease data from various sources such as clinical trials, observational studies, real world data and patient registries — including those within NORD's IAMRARE™ registry platform — and an analytics platform that will allow efficient and effective interrogation of that data to generate solutions to inform clinical trial design and regulatory review.

“For people living with rare diseases, time is of the essence,” said Joseph Scheeren, PharmD, C-Path President and Chief Executive Officer. “By leveraging the rare disease community access and data of NORD and the data curation, aggregation, governance and advanced analytics expertise of C-Path, we are poised to make a significant impact on rare disease drug development by providing quality data that will inform clinical trial design and accelerate the development of therapies.”

“Currently, more than 25 million people in the United States are affected by one or more of the over 7,000 rare diseases,” stated Peter L. Saltonstall, NORD President and Chief Executive Officer. “Drug development for these diseases is often impeded due to the low affected patient numbers and a limited understanding of how rare diseases progress or how to measure clinical improvements. FDA-approved treatments exist for only 10% of rare diseases; with this collaboration we can change that statistic for the better for our rare community.”

Meeting attendees will include representatives from across the community including regulators, patient organizations, clinicians, researchers and pharmaceutical companies interested in rare disease drug development. Attendees will have the opportunity to learn about the goals of the RDCA-DAP and how to engage in the early stages of this effort.

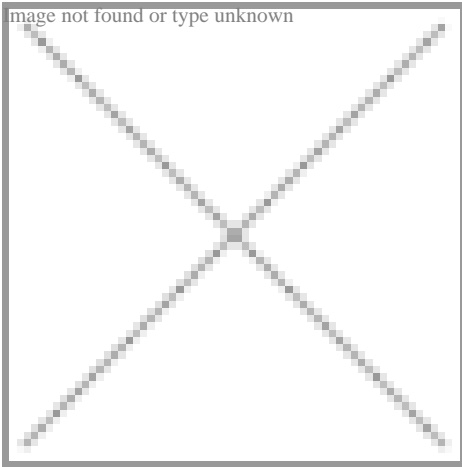
Register for the meeting here; <https://bit.ly/2YOj3jl>



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 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 c-path.org and c-path.eu.



About the National Organization for Rare Disorders (NORD)[®]

The National Organization for Rare Disorders (NORD) is the leading independent advocacy organization representing all patients and families affected by rare diseases. NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90% are still without an FDA-approved treatment or therapy. NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For more than 35 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research and providing patient and family services for those who need them most. NORD is made strong together with over 275 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease community. For more information, visit rarediseases.org.

Media Contacts:

Kissy Black
C-Path
615.310.1894
kissyblack@lotosnile.com

Laura Mullen
NORD
203.304.7258
lmullen@rarediseases.org