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## **C-Path and CHDI launch consortium to accelerate development of Huntington's disease therapies**



**Tucson, AZ, and New York, NY – March 28, 2018** – [The Critical Path Institute](#) (C-Path), together with [CHDI Foundation, Inc.](#) (CHDI), today announced the official launch of C-Path's Huntington's Disease Regulatory Science Consortium (HD-RSC). In addition to these cofounders, the consortium also includes more than 20 different member organizations, including industry partners, academic institutions, and nonprofit societies. HD-RSC members will work to advance innovation in regulatory science methods, supporting clearer development and regulatory pathways that lead to the approval of Huntington's disease (HD) therapeutics.

"C-Path has an established record of success in leading precompetitive consortia whose members collaborate to advance innovation in the regulatory science of drug development. We value this new partnership with CHDI, an organization that also embraces collaboration as a mechanism to more quickly and efficiently reach a common goal," said Martha Brumfield, PhD, President and CEO of C-Path. "We look forward to combining our strengths in this new consortium: C-Path's successful track record in overseeing global consortia and CHDI's HD therapeutic domain knowledge."

HD-RSC will provide the platform for the collaboration needed to facilitate clinical data sharing and standardization, to support the development of modeling tools, and to bring forward these tools as well as biomarkers and clinical outcome assessments for regulatory endorsement. These drug development tools will be made publicly available to help accelerate the time to drug approval and de-risk the drug-development pathway, thereby further incentivizing drug developers to enter the HD sphere. HD-RSC will work collaboratively with US Food and Drug Administration (FDA) and European Medicines Agency (EMA) staff to align on areas of high unmet need in developing therapies for this devastating disease.

"With this dedicated regulatory science consortium, stakeholders can share data and knowledge while avoiding duplication of efforts," said Robi Blumenstein, President of CHDI. "It provides a forum for everyone interested in HD therapeutics, including regulators, to participate in the development of an appropriate regulatory pathway that will deliver therapeutics to patients and families as soon as possible. This aligns perfectly with CHDI's mission to rapidly and collaboratively develop therapeutics that substantially improve the lives of individuals affected by Huntington's disease."

Charles Sabine, an HD patient-advocate, emphasizes the need to move forward with purpose in HD drug development: "We have hope," he said, and "hope can only be built on the trust that everyone is working as fast as they can in the same direction."

## About Huntington's Disease

Huntington's disease (HD) is a rare genetic neurodegenerative disorder that results in behavioral, cognitive, and motor impairments. These symptoms progressively reduce an individual's quality of life, and ultimately lead to death. HD is caused by a mutation in the huntingtin gene, which results in a toxic protein that damages neurons in the brain. Approximately one person in 10,000 carries the mutated huntingtin gene, and each child of a parent with a mutation has a 50% chance of inheriting the mutation. Current HD therapies only manage the severity of symptoms; there are no approved treatments to slow the progression of HD.

## About the organizations:



**CHDI Foundation, Inc. (CHDI)**, is a privately funded nonprofit biomedical research organization that is exclusively dedicated to rapidly developing therapies that slow the progression of Huntington's disease. As a collaborative enabler, CHDI seeks to bring the right partners together to identify and address critical scientific issues and move drug candidates to clinical evaluation as quickly as possible. CHDI scientists work closely with a network of more than 700 researchers in academic and industrial laboratories and clinical sites around the world in the pursuit of these novel therapies, providing strategic scientific direction to ensure that our common goals remain in focus. More information about CHDI can be found at [www.chdifoundation.org](http://www.chdifoundation.org).

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**Critical Path Institute (C-Path)** is an independent, nonprofit organization established in 2005 with public and private philanthropic support from the Arizona community, Science Foundation Arizona, and the US Food and Drug Administration (FDA). 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, public-private partnerships that currently include over 1,450 scientists from government and regulatory agencies, academia, patient advocacy organizations, and dozens of major pharmaceutical companies. C-Path is headquartered in Tucson, Arizona. For more information, visit [www.c-path.org](http://www.c-path.org).

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