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TUCSON, Ariz., October 31, 2016 — Critical Path Institute (C-Path) and CHDI Foundation, Inc., are collaborating to set up a broad-based consortium that will more clearly define regulatory pathways leading to the approval of Huntington's disease (HD) therapeutics. The HD Regulatory Science Consortium (HD-RSC) will bring together participants from within and without the HD community to advance drug development tools (DDTs, such as biomarkers and clinical outcome assessments) for regulatory endorsement and to facilitate clinical data standardization and collaboration. These DDTs will be made publicly available to help expedite and de-risk the drug-development pathway, speed the time to drug approval, and offer further incentive to drug developers to enter the HD sphere.

This regulatory focus is in response to the US Food and Drug Administration's (FDA) *Guidance for Industry and FDA Staff – Qualification Process for Drug Development Tools*. Such tools would potentially accelerate the drug approval process. Among the consortium's specific projects will be the regulatory qualification of biomarkers and outcome measures, and the development of a clinical trial simulator. The HD-RSC will also work in partnership with the Clinical Data Interchange Standards Consortium (CDISC) to develop therapeutic area data standards, and then centrally warehouse shared data as the foundation for these and other activities.

C-Path will manage the day-to-day activities of the HD-RSC, which is expected to first meet in Q2 2017. CHDI has provided planning and start-up funds.

“We support and share C-Path’s model of collaborative initiatives to accelerate drug development,” says Robi Blumenstein, President of CHDI Management, Inc. “The need for C-Path’s expertise in consortium building and regulatory science has become increasingly apparent as our community sees a number of high-quality therapeutic programs approach the clinic.”

Through the pre-competitive consortium approach, CHDI, C-Path, and other stakeholders can share data and knowledge, avoid duplication of effort, and collaborate in innovative ways so that scientific advances can be translated into therapeutics as efficiently as possible.

“One of the fundamental challenges to drug development for a genetically predetermined disease such as HD is shifting the paradigm for drug approval, so that we can intervene as early as possible; ideally, this would eventually be before any overt symptom onset,” says Martha Brumfield, PhD, C-Path president and CEO. “For quantitative analytical approaches to address this problem, data sharing, standardization, and aggregation through a rigorously defined procedure are paramount. In this regard, C-Path’s core competencies in clinical data standards development, as well as secure data management, standardization, and curation, will be tapped. These skills, combined with C-Path’s track record of success in consortium building and regulatory science, and CHDI’s extensive data resources and research network, provide a solid foundation for the HD-RSC’s success.”

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., 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.

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 12 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.

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