C-Path, CHDI Foundation, and CDISC Announce Public Review Period for Huntington's Disease Therapeutic Area User Guide

September 11, 2017

TUCSON, AZ, NEW YORK, NY, and AUSTIN, TX – September 11, 2017 – Critical Path Institute (C-Path), CHDI Foundation, Inc. (CHDI), and The Clinical Data Interchange Standards Consortium (CDISC) announce the availability of a draft Huntington's disease (HD) Therapeutic Area User Guide (TAUG-HD v1.0) for public review. The TAUG-HD v1.0 describes how HD clinical data should be recorded in a standardized database to establish common best practices across the healthcare industry for the recording, reporting, and sharing of clinically relevant disease-specific metadata, research data, and patient information. Use of the standard will allow the HD research community to compare and contrast data from different studies more easily and with more scientific rigor, and will make it easier for researchers to understand natural history, biomarker, and trial data in the future. It will also facilitate regulatory submissions for novel therapeutics.

Researchers and clinicians are encouraged to review TAUG-HD v1.0 and offer feedback. This input will be essential to ensure that neutral, consensus-based data standards are developed and adopted by a diverse global community that is committed to improving research processes and quality for the benefit of all.

The public review period begins on September 11, 2017, and extends through November 10, 2017. On September 12, CDISC will host a public webinar detailing the public review process; this webinar will be available online throughout the review period.

Huntington's disease is a genetic, progressive neurodegenerative disease that causes a range of physical, mental, and emotional disabilities, including involuntary movements, cognitive decline, and behavioral changes. The disease is caused by an expansion mutation in the CAG repeat region of the *huntingtin* gene, resulting in a toxic protein that leads to neurodegeneration. Around one person in 10,000 carries the mutated *huntingtin* gene, and each child of a parent with the mutation has a 50% chance of inheriting the disease. Current HD therapies only manage the severity of a subset of symptoms; there are no approved treatments to slow the disease's progression.

"Huntington's research is moving forward rapidly, with greater industry focus seeking to develop novel therapies," said Martha Brumfield, PhD, C-Path President and CEO. "As we move forward with the aggregation of clinical data sets, it is our hope to create tools that will inform innovative clinical trial designs,

which can help expedite development programs. It is an ideal moment for the development of a CDISC TAUG that will standardize nomenclature, formats, collection, and reporting of these new forms of data. C-Path's collaboration with CHDI and CDISC on this user guide is both valuable and timely."

"The HD research field is now at the threshold of important clinical developments that will help us move some of the promising therapeutic candidates in the pipeline to clinical trial," said Cristina Sampaio, MD, PhD, Chief Clinical Officer at CHDI Management, Inc. "This collaboration to develop universal data standards is another example of how the highly collaborative nature of the HD research community can accelerate therapeutic development for the benefit of patients and their families."

To review the therapeutic area user guide, please visit: https://www.cdisc.org/public-review/huntingtons-disease-therapeutic-area-user-guide-v10-available-public-review

TAUG-HD v1.0 describes the biomedical concepts relevant to HD clinical studies and the necessary metadata to represent such data consistently, allowing datasets from multiple sources to be compared or combined for analysis. TAUG-HD v1.0 focuses on the representation of data using the CDISC Study Data Tabulation Model (SDTM). Types of data covered include family history, genetics, and biomarkers to assess disease onset and progression. Specific biomarkers included in this user guide include neuroimaging assessments (MRI and PET) and biofluid biomarkers, with a focus on cerebrospinal fluid analytes.

TAUG-HD v1.0 was developed through the Coalition for Accelerating Standards and Therapies (CFAST) consortium, with clinical advice and input from CHDI, C-Path, CDISC, and other knowledge domain experts. CFAST, a joint initiative of CDISC and C-Path, was formed to accelerate clinical research and medical product development by creating and maintaining data standards, tools, and methods for conducting research in therapeutic areas important to public health, with invaluable support and advice from such organizations as the National Cancer Institute, Innovative Medicines Initiative, TransCelerate, and regulatory agencies including the FDA, Japan's Pharmaceutical and Medical Devices Agency, and the European Medicines Agency.

CDISC standards have been adopted and used in more than 90 countries, and are required by regulatory authorities in the U.S. and Japan. To date, therapeutic area standards have been developed for more than 30 different disease areas, with most being developed under the CFAST program. Use of these standards from the start of clinical research programs has proven capable of saving both time and resources. HD researchers are encouraged to implement these standards into their processes.

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About the organizations:

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 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.

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 around the world in the pursuit of these novel therapies, providing strategic scientific direction to ensure that these common goals remain in focus. More information about CHDI can be found at www.chdifoundation.org.

CDISC is a 501(c)(3) global nonprofit organization that develops data standards to foster smarter research and enable connections to healthcare. CDISC standards allow data to *speak the same language*, by providing common formats for data collection, data sharing and data analyses to make the most of the valuable information offered by patients participating in research studies around the globe, enabling researchers to discover new treatments, find breakthroughs, and unlock cures. CDISC standards are required for regulatory submissions to the US FDA and Japan's PMDA, are endorsed by the China CFDA, and are requested for use by the European Innovative Medicines Initiative (IMI). The suite of CDISC standards is freely available on the CDISC website.

CDISC is funded through the generous support of over 420 member organizations from pharmaceutical, biotech, clinical research organizations, regulatory agencies, academia, and healthcare, as well as through grants, authorized CDISC Education courses, events, and charitable contributions. To find out more about how to support CDISC and get involved, please visit www.cdisc.org.