C-Path and CDISC Announce Therapeutic Area User Guide for Duchenne Muscular Dystrophy

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TUCSON, Ariz., and AUSTIN, Texas – October 18, 2017 –Critical Path Institute (C-Path) and The Clinical Data Interchange Standards Consortium (CDISC) announce the open availability of a Duchenne Muscular Dystrophy Therapeutic Area User Guide (TAUG-DMD v1.0), which describes the most common clinical concepts relevant to Duchenne studies using the CDISC standard format. This format allows datasets from different sources to be compared or combined for data collection, sharing, and analyses.

Version 1.0 of TAUG-DMD focuses on representing data using the CDISC Study Data Tabulation Model (SDTM). Types of data covered include genetic information and imaging data, as well as results from a variety of disease measures including cardiac, musculoskeletal, and pulmonary function assessments. Data standards are also being developed for a variety of functional tests.

“We are delighted to partner with C-Path’s Duchenne Regulatory Science Consortium [D-RSC] to create a standard for Duchenne muscular dystrophy that will facilitate improved data collection, sharing, and analysis, giving patients the hope that new treatments may be discovered,” said David R. Bobbitt, MSc, MBA, President and CEO, CDISC.

The US Food and Drug Administration (FDA) Binding Guidance requires that sponsors submit data in FDA-supported CDISC formats listed in the FDA Data Standards Catalog. CDISC standards have been adopted and used in more than 90 countries, and are required by regulatory authorities in the US and Japan. To date, Therapeutic Area Standards have been developed for more than 30 different disease areas. Use of these standards from the start of clinical research programs has proven capable of saving both time and resources. Researchers in Duchenne are encouraged to implement these standards into their processes.

“I avidly follow DMD drugs in the pipeline that are soon to be evaluated by the FDA,” said Christopher (Buddy) Cassidy, patient representative for the D-RSC. “I am very enthusiastic about the role the CDISC data standard for Duchenne will play in the near future with particular regard to FDA approval. A standardized method for data collection will in turn make it easier for clinicians, researchers, drug development companies, and the FDA to share and compare data. Only through the constant exchange of like sets of data can we truly evaluate the effectiveness of new DMD treatments.” He added, “As a patient with Duchenne, I consider myself fortunate being born into the time I was. I get to witness the beginning of a promising age of genetic medicine and revolutionary new treatment.”

“We are excited to publish the new Duchenne Therapeutic Area User Guide,” said Jane Larkindale, DPhil, Executive Director of the D-RSC. “We hope the research community rapidly adopts the standard in new studies, which will allow future data to be compared, contrasted, and combined in a methodical manner, and lead to a more rapid and comprehensive understanding of disease progression and drug effects.”

TAUG-DMD v1.0 was developed under the Coalition for Accelerating Standards and Therapies (CFAST), with clinical advice and input from D-RSC members. 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 FDA, Japan’s Pharmaceutical and Medical Devices Agency, and the European Medicines Agency.

Duchenne is a rare, fatal, genetic neuromuscular disorder that is diagnosed in childhood and primarily affects boys. It causes progressive loss of muscle, resulting in loss of the ability to walk, loss of upper body strength, progressive breathing issues, cardiomyopathy, and premature death. Although there are medical treatments that may help slow its progression, there is currently no cure for Duchenne. C-Path launched the D-RSC in partnership with Parent Project Muscular Dystrophy (PPMD) to establish drug development tools that can accelerate clinical progress of new therapies for the disease.

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.

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 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 400 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 .

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