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CDISC and TransCelerate BioPharma Inc. Announce Landmark Asthma Data Standard Released for Public Review

AUSTIN, Texas, Sept. 18, 2013 /PRNewswire/ -- The Clinical Data Interchange Standards Consortium (CDISC) and TransCelerate BioPharma Inc. ("TransCelerate"), are pleased to announce that version 1.0 of the Asthma Therapeutic Area (TA) Data Standard is now available for public review on the CDISC website.

The Asthma TA standard is the first standard to have been developed with TransCelerate's active participation in the Coalition for Accelerating Standards and Therapies (CFAST) initiative. CFAST, a joint initiative of CDISC and the Critical Path Institute (C-Path), was launched to accelerate clinical research and medical product development by facilitating the establishment and maintenance of data standards, tools and methods for conducting research in therapeutic areas important to public health. CFAST collaborators include the U.S. Food and Drug Administration (FDA), TransCelerate, the National Cancer Institute Enterprise Vocabulary Services (NCI-EVS), with participation and input from many other organizations.

The TA data standards augment an existing suite of CDISC global standards for clinical research, which streamline the entire clinical research process, saving time and money while improving data quality and enabling development of scientific insights for the benefit of patients. CDISC TA standards developed to date, including those for Alzheimer's and Parkinson's diseases, have led to a faster, scalable standards development process that is now being implemented for Asthma. It is anticipated that the process followed for this standard will serve as a model for the development of many more therapeutic area data standards through the CFAST Initiative. The Asthma TA data standard specifically includes variables being collected in clinical research studies in support of therapies for asthma in adults. To access and comment on the Asthma TA draft standard now available for public review, please visit the CDISC website at http://www.cdisc.org/therapeutic.

"The CDISC Asthma Therapeutic Area Standard is a significant achievement for CDISC, and marks a major milestone in our collaborative efforts with TransCelerate, which has contributed significant resources to the CFAST Initiative," stated Rhonda Facile, CDISC Senior Director of Standards & Development, who is leading the Asthma project. "We would also like to thank the CDISC Technical Teams (BRIDG, CDASH, SDS, Terminology and ADaM), as well as the NCI, NIH Heart Lung and Blood Institute (NHLBI), FDA, and those on the CFAST TA Standards Steering Committee for their efforts in developing this landmark standard."

"TransCelerate is happy to participate in the development of therapeutic area data standards via the CFAST initiative," said Dalvir Gill, PhD, Chief Executive Officer of TransCelerate. "The efficient, consensus-based development of such standards is aligned with our goal of collaborating to improve data flow, quality and interoperability in and across clinical trials – ultimately bringing new therapeutics to patients."

ABOUT CDISC

CDISC is a 501(c)(3) global non-profit charitable organization, with over 300 supporting member organizations from across the clinical research and healthcare arenas. Through the efforts of volunteers around the globe, CDISC catalyzes productive collaboration to develop industry-wide data standards enabling the harmonization of clinical data and streamlining research processes from protocol through analysis and reporting, including the use of electronic health records to facilitate the collection of high quality research data. The CDISC standards and innovations can significantly decrease the time and cost of medical research and improve quality, thus contributing to the faster development of safer and more effective medical products and a learning healthcare system. The CDISC Vision is *to inform patient care and safety through higher quality medical research.*

ABOUT TransCelerate BioPharma Inc.

TransCelerate BioPharma Inc. was formed in 2012 and is a non-profit organization focused on advancing innovation in research and development (R&D), identifying and solving common R&D challenges and further improving patient safety, with the goal of delivering more high quality medicines to patients. TransCelerate evolved from discussions at various forums for executive R&D leadership to discuss relevant issues facing the industry and solutions for addressing common challenges. Founding members include AbbVie, AstraZeneca, Boehringer Ingelheim, Bristol-Myers Squibb, Eli Lilly and Company, GlaxoSmithKline, Johnson & Johnson, Pfizer, the Roche Group, and Sanofi, and all have representation on the Board of Directors. Additional members include Astellas Pharma Inc., Biogen Idec, Braeburn Pharmaceuticals, Cubist Pharmaceuticals, EMD Serono, Inc. (a subsidiary of Merck

KGaA, Darmstadt, Germany), Forest Research Institute (a subsidiary of Forest Laboratories, Inc.) and Onyx Pharmaceuticals.

Membership in TransCelerate is open to all pharmaceutical and biotechnology companies who can contribute to and benefit from these shared solutions. Executive offices are located in Philadelphia, PA. For more information, please visit http://www.transceleratebiopharmainc.com/.

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SOURCE The Clinical Data Interchange Standards Consortium; TransCelerate BioPharma Inc.

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http://www.transceleratebiopharmainc.com
http://www.cdisc.org

Program Overview – November 2013

Approved Therapeutic Area Standards Projects

Therapeutic Area	Coordinating Organization(s) / Project Manager	Start Date	Stage 0 / Scoping & Input	Stage 1 / Concept Modeling	Stage 2 / Standards Development	Stage 3a / Internal Review	Stage 3b / Public Review	Stage 3c / Publication	Notes
Alzheimer's Disease v2	CPATH/CDISC Jon Neville	Jan 13	Jan	Mar	Jun	Sep	Oct	*Q413*	
Asthma v1	CDISC Rhonda Facile	Nov 12	Jan	Mar	Jun	Jul	Oct	*Q413*	
Cardiovascular Endpoints v1	CDISC/DCRI Amy Palmer	Jun 13	Jul	Sep	Nov	*Dec*		*Q214*	Dependent on new Clinical Decisions (CD) domains
Multiple Sclerosis v1	CPATH/CDISC Bess Leroy	Mar 13	May	*Oct*	Nov	*Dec*		*Q114*	Parallel development of Stage 1 & 2.
Diabetes v1	TCB/CDISC Rachael Zirkle	Apr 13	May	Aug	Nov	*Dec*		*Q114*	
QT Studies v1	TCB/CDISC John Owen	Aug 13	Oct	*Nov*	*Jan*			*Q214*	
Traumatic Brain Injury v1	CDISC Rhonda Facile	*Oct 13*	Nov	*Dec*				*2014*	
Hepatitis C v1	TCB/CDISC John Owen	Nov 13	*Jan*					*2014*	
Schizophrenia v1	CDISC/DCRI Amy Palmer	*Oct 13*	Nov	*Dec*				*2014*	
Breast Cancer v1	TCB/CDISC/UCSF Sarah Davis	Q1 14						*2014*	
Influenza	TBD	Jan 14						*2015*	
COPD v1	TBD								

Key: | Stage completed | Stage ongoing | *Italics*=Projected | Months reflect when stage **completed**

Approved CFAST Project Summaries

Alzheimer's Disease

CFAST is currently engaged in creating v1.1 of the Alzheimer's Disease (AD) Therapeutic-area Data Standard. The goal of this project is to expand upon the work done in v1.0, increasing its utility for clinical trials and observational studies, with an emphasis on early AD and Mild Cognitive Impairment (MCI). The standardization effort focuses on three major areas of content: clinical scales of cognition and function, cerebrospinal fluid (CSF) sampling and biomarkers, and imaging biomarkers. The workgroup has identified ~10 clinical scales which are in the process for developing controlled terminology and supplemental guide documentation. Through ongoing webinars with imaging subject-matter experts, we have nearly completed elucidating the inter-related concepts of image acquisition, analysis, and derivation of both functional and morphological metrics of the brain that are relevant to this disease. Similarly, we have almost finished a parallel task to understand the relationships between the quantitative CSF protein biomarker measurements of interest in AD, and the various procedures of lumbar puncture, CSF sampling, handling, processing and storage of the specimen that help put those quantitative results in context for the researcher. We are on track to finish the concept maps this month, and to begin vetting these within the CDISC user community so we can begin drafting the user guide documentation.

Asthma

CFAST is proposing the development of the CDISC Asthma User Guide v1.0 (TAUG - Asthma) Therapeutic-area Data Standard. Descriptions addressed in this TAUG-Asthma v 1.0 will include the clinical situations from which the data arise, and the reasons these data are relevant for asthma. This standard would build on the existing CDISC Virology TA standards to facilitate the collection and use of data relevant to Hepatitis C clinical trials. The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to Asthma: pulmonary function tests, exacerbations of asthma, biomarkers, symptom assessment, QoL measures and composite outcomes, medical history, health care resource utilization, AEs of special interest and CMs of special interest.

Breast Cancer
Approved Nov. 14, 2013

CFAST is proposing development of v1.0 of the CDISC Breast Cancer (BC) Therapeutic-area Data Standard. This standard would build on the existing SDTM standards and related CDASH standards to facilitate the collection and use of data relevant to BC clinical trials.

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology.

The standardization effort is expected to focus on the following areas of specific interest to BC: Data to substantiate diagnosis, including H&E, ER/PR/Her2 status, as well as other key biomarkers (e.g., Ki-67, luminal A, luminal B, Oncotype DX or other gene profile assays); Medical and relevant family history (oncologic, gynecologic, and general); RECIST 1.1 tumor lesion burden and response measurements; Bone lesion assessments; Imaging modality; Key time to event analysis endpoints, including overall, progression, and disease free survival; Treatment history type (systemic, radiological, surgical) and intent (neo/adjuvant, curative, palliative); Post-study treatment therapies type (systemic, radiological, surgical) and intent (curative, palliative); Historical/preexisting and treatment emergent adverse events using CTCAE/MedDRA terms and severity criteria; Treatment and study disposition, including reasons for discontinuation of study treatment and study participation; Cardiac function assessment; Concomitant medications; Health care resource utilization (related to both disease and supportive care) including hospitalization, intensive care, emergency visits, and hospice care.

COPD
CFAST is proposing development of v1.0 of the CDISC COPD Therapeutic-area Data Standard. This standard would build on the existing SDTM Asthma TA standards, Cardiovascular TA standards, and related CDASH standards to facilitate the collection and use of data relevant to COPD clinical trials.

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to COPD: data to substantiate diagnosis, medical history of special interest, pulmonary function tests, symptom assessment, COPD exacerbations, AEs of special interest (e.g. MACE), CMs of special interest (e.g. rescue medication), patient reported outcomes (e.g. Saint George's Respiratory Questionnaire (SGRQ), Transition Dyspnea Index (TDI)), and health care resource utilization including hospitalization, intensive care, and emergency visits.

CV Endpoints

Version 1.0 of the Cardiovascular Therapeutic-area Data Standard and User Guide (CV-UG) will incorporate cardiovascular endpoints developed by subject matter experts at FDA and Duke Clinical Research Institute (DCRI) with grant support from FDA. Cardiovascular endpoints are defined as an objective morbid condition or cause of death linked to cardiovascular disease, and is of particular interest for multiple clinical trials. Obesity and diabetes mellitus are often linked to cardiovascular disease as are a history of chronic kidney disease and hypercholesterolemia.

The standardization of the cardiovascular endpoints is focused on the following: percutaneous coronary intervention (PCI), peripheral vascular intervention (PVI), heart failure, myocardial infarction (MI), stent thrombosis, transient ischemic attack (TIA), stroke, hospitalization for unstable angina, and cardiovascular death and un-determined cause of death. Additional data elements are included for CV anatomical locations, severity and symptoms of the events, as well as complications of the interventions. There are roughly 110 data elements currently under development for cardiovascular and stroke endpoints. This UG will also incorporate additional CDEs relevant to acute coronary care which may be commonly collected during cardiovascular trials. Future versions of the CV-UG will include additional CV data standards, such as CV Imaging, when developed. The target completion date for the v1 CV data standard and UG is the end of the first quarter 2014.

Diabetes

CFAST is proposing development of v1.0 of the CDISC Diabetes Therapeutic-area Data Standard. This standard would build on the existing CDISC domains standards to facilitate the collection and use of data relevant to diabetes clinical trials.

The workgroup proposes developing a CDISC therapeutic area User Guide that includes key lab assessments (including, but not limited to A1c, fasting glucose, C-peptide), patient self-monitoring blood glucose (SMBG) profiles, antihyperglycemic agents, hypoglycemia (additional module included AE domain), CV events/outcomes, diabetes history, complication history, patient reported outcomes (questionnaires or patient completed scales) that are used in many diabetes studies. Second priority endpoints will include glucose tolerance testing and dietary information (e.g. daily caloric intake, carbohydrate intake). Project deliverables will include concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on areas of specific interest to diabetes: medical history, health care resource utilization, patient reported outcomes, AEs of special interest and CMs of special interest.

Hepatitis C
CFAST is proposing development of v1.0 of the CDISC Hepatitis C Therapeutic-area Data Standard. This standard would build on the existing CDISC Virology TA standards to facilitate the collection and use of data relevant to Hepatitis C clinical trials.
The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to Hepatitis C: medical history, health care resource utilization, patient reported outcomes, cirrhosis, progression of liver disease, AEs of special interest, CMs of special interest and HCV viral load testing.
For more information on Hep-C, see:
http://www.who.int/mediacentre/factsheets/fs164/en/

Major Depressive Disorder (MDD)
CFAST is proposing development of v1.0 of the CDISC MDD (Adult and Pediatric) Therapeutic-area Data Standard. This standard will build on the existing SDTM and related CDASH standards to facilitate the collection and use of data relevant to MDD clinical trials.

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples, and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to MDD: data to substantiate diagnosis, medical history (psychiatric and neurologic, including course of illness), symptom rating scales, patient reported outcomes, suicidality, treatment history (drug and non-drug), medication side effects, and health care resource utilization, including psychiatric hospitalizations and emergency visits.

Multiple Sclerosis
The Multiple Sclerosis Outcomes Assessment Consortium (MSOAC) was created in December 2012 with a CDISC Therapeutic Area User Guide for MS being a key deliverable. This User Guide will be based on Common Data Elements (CDEs) developed by the National Institute of Neurological Disorders and Stroke (NINDS). The MSOAC will hold its first annual meeting on April 1st and 2nd of 2013 where the newly formed data work group will begin to assess which CDEs would be appropriate to include in a version 1.0 of the standard. Project deliverables will include: concept maps as needed, an SDTM v1.0 TA User Guide for Multiple Sclerosis, new SDTM Domains as needed, and QS implementation supplements with statements of copyright permission for the clinical scales modeled in SDTM.

QT Studies

CFAST is proposing development of a CDISC QTc Therapeutic-area Data Standard. This standard would build on the existing CDISC ECG [EG] standards, and related CDASH standards, to facilitate the collection and use of data specifically expected to be collected during so called 'thorough QTc studies [Guidance for Industry E14 Clinical Evaluation of QT/QTc Interval Prolongation and Proarrhythmic Potential for Non-Antiarrhythmic Drugs].

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to QTc studies: ECG-related variables [e.g., morphology, intervals] whether from Standard 12-Lead ECGs or Ambulatory ECG Monitoring, AEs, dosing, pharmacokinetics & relevant pharmacogenomics data.

Note: in particular, this project is not intended to cover cardiovascular endpoints, cardiovascular imaging which are being covered via other data standards development projects, but will be developed in a manner consistent with the forthcoming CDISC CV [cardiovascular] Therapeutic Area data standard.

Rheumatoid Arthritis (RA)

CFAST is proposing development of the CDISC RA Therapeutic-area Data Standard. This standard will build on the existing SDTM and related CDASH standards to facilitate the collection and use of data specifically expected to be collected during adult and juvenile RA studies.

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps, metadata, examples and controlled terminology. The standardization effort is expected to focus on the following areas of specific interest to rheumatoid arthritis (RA): medical history, duration of RA disease, past and baseline medication use for RA, screening procedures (such as screening for tuberculosis), clinical assessments (such as joint assessments), patient reported outcomes, radiographic assessments, health care resource utilization (such as ER visits, in-home health care visits), concomitant medication for RA, surgical procedures for RA, AEs of special interest (such as infections, including serious and opportunistic infections and tuberculosis, malignancies, including lymphoproliferative disorders, injection site reactions, systemic hypersensitivity reactions, including anaphylaxis, drug-induced liver injury), laboratory tests of special interest, device malfunction (for drug-device combination products).

Schizophrenia

CFAST is proposing development of v1.0 of the CDISC Schizophrenia Therapeutic-area Data Standard, building on existing SDTM and related CDASH standards to facilitate the collection and use of data relevant to this disease area.

Schizophrenia is a mental disorder characterized by delusions, hallucinations, and disorganized speech, with the onset of symptoms usually appearing in young adulthood. The Clinical Data Elements (CDEs) to be used in developing v1.0 of the standard are being defined and developed by Duke Clinical Research Institute (DCRI) through HL7 with input from subject matter experts in government, academia, and industry under grant support from the FDA.

The Schizophrenia CDEs focus on the following data categories or domains areas: diagnosis, course of illness, family psychiatric history, psychiatric hospitalizations, AEs of special interest (e.g., tardive dyskinesia, akathisia, and hyperprolactinemia), and rating scales/questionnaires measuring multiple areas, including psychotic symptoms, functionality, neurocognition, and quality of life. This User Guide (UG) will be based on approximately 75 CDEs and will also include examples from several of the 135 applicable questionnaires.

Traumatic Brain Injury
Approved Oct 31, 2013

CFAST is proposing development of v1.0 of the CDISC Traumatic Brain Injury (TBI) Therapeutic-area Data Standard.

Traumatic Brain Injury (TBI) is a form of acquired brain injury, which occurs when a sudden external force causes damage to the brain. TBI can be classified based on severity, mechanism (closed or penetrating head injury) or other features. TBI affects an estimated 10 million people worldwide and more than 3.4 million in the U.S. every year. TBI is a silent epidemic--its symptoms are frequently invisible, thus difficult to diagnose and treat. TBI can lead to motor, cognitive, and social impairments that interfere with an individual's ability to be productive.

In collaboration with One Mind for Research, the workgroup proposes developing a therapeutic area User Guide to support clinical research and enable medical product development through the establishment and maintenance of data standards, tools and methods for conducting research in brain injury on a global scale. This project will build from the established NINDS CDEs for TBI to support SDTM (Study Data Tabulation Model) data models supplemented with guidelines to support CRFs consistent with CDASH (Clinical Data Acquisition Standards Harmonization).

The workgroup proposes developing a CDISC therapeutic area User Guide, including concept maps to show critical information elements and their relationships, domain mappings, rules, controlled terminologies and examples for representing data using SDTM and CDASH.

The standardization effort is expected to focus on the following areas of specific interest to TBI:

- Medical history of special interest
- Key biomarkers
- Physical and neurological assessments (functional assessments) of interest,
- Clinical observations based on medical imaging
- Patient reported outcomes and scales.