Opportunities and Challenges for adopting CDISC Standards Across Healthcare and the Life Sciences.

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Past Chairman of the Board, CDISC

9th Annual SAS Health Care & Life Sciences Executive Conference
Cary, NC, May 2012
Information from healthcare (private, aggregated) to enable research

**Healthcare**
- Quality healthcare
- Informed decisions
- Personalized medicine
- Patient safety and privacy
- Public health
- Improved therapies
- Efficiencies/reduced costs

**Research**
- Discovery of new therapies
- Understanding diseases
- Assessing efficacy
- Monitoring safety
- Public health/quality evaluations
- Understanding responses (genomics, biomarkers)
- Testing/comparing therapies (CER)
- Post-marketing surveillance

**Research findings to inform healthcare decisions**
Healthcare and Clinical Research: Parallel Universes

Patient Care World
- Multiple data sources and data types
- HL7 V2.x a pervasive standard
- Electronic medical records assembled from multiple sources
- Clinicians want to see everything they can get
- Data are organized around the patient

Clinical Research World
- Carefully controlled data
- Each trial’s data independent
- CDISC the emerging standard
- Data flows from sites to CROs to sponsors to FDA
- Bio-statisticians tightly control what is gathered
- Data are organized around a trial

Source: Landen Bain, CIO, DUMC ~ 2004
Standards, Who Needs Them?
**Why Standards?**

<table>
<thead>
<tr>
<th>Clinical Trials</th>
<th>Clinical Trials Date of Birth</th>
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<tbody>
<tr>
<td>What is the average age of patients enrolled in 11 industry trials for Alzheimer’s Disease?</td>
<td>Jan. 15, 2011</td>
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Why Standards?

- Efficient collaboration
- Better science
- Regulatory efficiency
**Gartner-PhRMA-CDISC: Business Case**

### Summary

- Using CDISC standards can save significant time and cost, especially when implemented in the early stages of the study.
- Opportunities for an additional impact on clinical research:
  - Increase data quality
  - Enable data integration; enhance re-usability
  - Facilitate data exchange with partners
  - Enable software tools
  - Improve team communication
  - Facilitate regulatory reviews and audits

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**Resource (Time and Cost) Savings**
Clinical Data Interchange Standards Consortium

The CDISC Vision: informing patient care and safety through higher quality medical research.

- Standards Developing Organization (SDO)
- Global, open, multidisciplinary, vendor-neutral, non-profit
- Founded 1997, incorporated 2000
- Member-supported (>300 organizational members, e.g. academia, biopharma, service / technology providers)
- Additional revenue streams through education, certification, grants, contributions (now a 501c3 charitable non-profit)
- Active Coordinating Committees (3C)
  - Europe, Japan, China, Korea
- > 20 User Networks around the world
- > 90 countries in participant database (~ 11,000); downloading CDISC standards from website
CDISC Strategic Goals 2012-2015

• Achieve significant progress in the use of CDISC standards to allow scientifically sound **data aggregation** and support secondary uses of research data for the purposes of scientific investigation and comparative effectiveness.

• Achieve significant progress in enabling **interoperability between clinical care and clinical research**, and explore expansion from bench to bedside (**translational research**); accelerate the cycle through which healthcare informs research and research informs clinical decisions.

• Expedite the development and rollout of new **therapeutic-area or specialty standards**, while continuing to refine, support and educate on **existing/foundational standards**, to ensure consistency in data capture and analysis related to efficacy in addition to patient safety.
CDISC Strategic Goals 2012-2015

• Develop CDISC SHARE (Shared Health And Research Electronic Library), a global, accessible, electronic library for CDISC content/semantics that will enable precise and standardized data element definitions and richer metadata that can be reused within applications and across studies to improve biomedical research and its link with healthcare.

• Leverage our global, nonprofit, vendor neutral, independent status to forge productive collaborations with and provide value to key stakeholder communities.

The CDISC mission is to develop and support global, platform-independent data standards that enable information system interoperability to improve medical research and related areas of healthcare.
Initial database required mapping to a standard (CDISC); can now be leveraged to collect data using the standard

Strategic Goal #1: Value of data aggregation made possible through CDISC standards
Conclusions from CAMD

• Data from individual clinical trials of 200-400 patients with Alzheimer’s Disease had limited power and frequently failed due to variability in outcome and small sub-groups.
• Mapping control arm data to a standard format for >6,000 patients in 20 trials **created a dataset with higher quality** (common methodology for ADAS-cog) and greater power to assess variables affecting progression
  - Severity and age at entry
  - ApoE4 genotype
• Data standards can
  1. Increase learning from clinical research study analysis
  2. Facilitate data sharing across research studies
  3. Create databases with which to design more informative and efficient research studies

**NOTE:** Data standards are most valuable (significantly reducing time and resources) when implemented from the beginning.
The CDISC vision is to inform patient care and safety through higher quality medical research.

Strategic Goal # 5: Leverage global, non-profit, vendor neutral, independent status to forge productive collaborations

Strength through Collaboration
CDISC Therapeutic Area Projects: Engagement and Initiating Organizations

“Efficacy Standards”

Through Today

- Tuberculosis (Duke, NIH)
- Acute Coronary Syndrome (Duke, NIH)
- Polycystic Kidney Disease (PKD Foundation, Tufts)
- Cardiovascular Disease (FDA, ACC)
- Alzheimer’s (C-Path, NINDS)
- Parkinson’s Disease (NINDS)
- Pain & Analgesics (FDA, U. of Rochester)
- Virology (FDA/NIH)
- Oncology (CDISC, NCI, FDA)
- Diabetes (FDA, ScenPro)

2012 and Beyond

- Expand TB (C-Path, Gates, Global TB Alliance, IMI Europe)
- Other Neurological Disorders such as TBI, MS, and other cancers
- Medical Devices and Imaging (CDISC, NCI, FDA)
- Vaccines (IMI Europe)
- Pediatrics (NICHD)
- Other FDA TA priorities (55)

Strategic Goal #3:
Therapeutic Area standards for ‘efficacy’ data to augment safety data standards
FDA’s draft Performance Goals for PDUFA V

- Clinical data standards needed for therapeutic areas
- Use of data standards indicated for future applications.

**E. Clinical Terminology Standards:** …FDA shall develop standardized clinical data terminology through open standards development organizations (i.e., ….CDISC) with the goal of completing clinical data terminology and …. implementation guides by FY 2017.

1. FDA shall develop a project plan for distinct therapeutic indications…. FDA shall publish a proposed project plan for stakeholder review and comment by June 30, 2013…..

**G. FDA shall periodically publish final guidance specifying the completed data standards, formats, and terminologies that sponsors must use to submit data in applications…..**

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<th>Tier 1</th>
<th>Pain*</th>
<th>Schizophrenia</th>
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<tr>
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<td>Schizophrenia</td>
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<td>Alzheimer’s Disease*</td>
<td>Parkinson’s Disease*</td>
<td>Solid organ transplantation</td>
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<td>Anti-diabetic agents*</td>
<td>Polycystic Kidney Disease* (added)</td>
<td>Treatment of Hepatitis C* (Virology)</td>
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<td>Tuberculosis*</td>
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<td>Cardiovascular and Cardiovascular Imaging*</td>
<td>QT Studies</td>
<td>Urinary tract infections</td>
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<tr>
<td>Oncology: time to efficacy event other than overall survival*</td>
<td>Rheumatoid arthritis</td>
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**Legend**  

<table>
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<tr>
<th>2012</th>
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* = in progress
A global, accessible electronic library, which through advanced technology, enables precise and standardised data element definitions that can be used in applications and studies to improve biomedical research and its link with healthcare.

**Key purposes:** Develop efficacy standards faster and make the CDISC standards more accessible.
A clinical research domain analysis model (DAM) in UML initiated by CDISC, BRIDGing

- Organizations (CDISC, HL7, FDA, NCI)
- Standards
- Research and Healthcare

- Now exploring how to ensure alignment with NCI Life Sciences DAM and HL7-CDISC Clinical Genomics DAM
- BRIDG is on the path to becoming an ISO/CEN standard through the JIC

Open source; Collaborative Project

- See BRIDG Model on CDISC website
  or www.bridgmodel.org
Healthcare Link Goal: Optimize the Research Process

Strategic Goal #2: CDISC Healthcare Link – interoperability between clinical research and clinical care
Patient Value: Quality of Healthcare, Safety

Research informs healthcare more effectively
Build quality into process at beginning

Care and/or Research Site
(Healthcare Location, Investigator, Site Personnel)

Study Sponsor
(e.g. ARO, CRO, Vendor, Principal Investigator, potentially AHRQ...)

Interoperability Specification

CRO or Partner
(e.g. Research Partner, Sponsor, Registry, Regulator, IRB, DSMB, Quality Measures)

Public Registries, IRB, DSMBs

Reviewers
(e.g. Research Partner, Sponsor, Registry, Regulator, IRB, DSMB, Quality Measures)

Continuity of Care Doc

EHR

Site Research Archive

Std. Common Research Dataset (+)

De-identified Data

Research Results, eSubmission Standard Formats

Scientific Publication

Interoperability Specification

CDASH Initiative

RFD*

Continuity of Care Doc

Regulatory Authority
A Learning Health System for the Nation

Pharmaceutical Firm

Beacon Community

Integrated Delivery System

State Public Health

Community Practice

Federal Agencies

Health Information Organization

Health Center Network

Governance

Patient Engagement

Trust

Analysis

Dissemination

CDISC
Summit Planning Committee
Members

- David Blumenthal, Partners HealthCare
- Adam Clark, MedTran Health Strategies
- Charles Friedman, University of Michigan (Chair)
- Claudia Grossman, Institute of Medicine
- Robert Kolodner, Open Health Tools
- Rebecca Kush, Clinical Data Interchange Standards Consortium
- Allen Lichter, American Society for Clinical Oncology
- Janet Marchibroda, Bipartisan Policy Center
- Michael McGinnis, Institute of Medicine
- Marc Overhage, Siemens Healthcare
- Frank Rockhold, GlaxoSmithKline
- Joshua Rubin, Joseph H. Kanter Family Foundation
- Jonathan Silverstein, NorthShore University HealthSystem
- Richard Tannen, University of Pennsylvania
- James Walker, Geisinger Health System
- Joseph Kanter, ex officio
Where is the Drug Safety Data?

- Clinical Trials
- Health Claims data
- Electronic Health records
  - OMOP
  - Mini Sentinel
  - IMI Protect
  - ….
Key Messages

• Research and Healthcare data are linked

• Building quality in from the beginning is ideal
  ▪ ….adding in quality at the ‘back end’ (e.g. through mapping or
    normalization) can certainly be done but only at a high cost
    (time and resources)

• Data standards improve data quality
  ▪ .....especially when implemented in the data collection steps
    at the beginning

Patients are motivated to have high quality data.
The CDISC Vision is to Inform Patient Care & Safety Through Higher Quality Medical Research

Strength through Collaboration
Backup Slides