A Brave New World:

CDISC’s New Therapeutic Area Standards for Clinical Research

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Rhonda Facile, Sr. Director, TA Standards Development, CDISC
Agenda

- What is CDISC?
- Foundational Standards
- Therapeutic Area Standards
- Why Does it Matter?

www.cdisc.org
Clinical Data Interchange Standards Consortium

- Non-profit 501(c)3 organization
- Provides infrastructure and leadership
- Most work done by volunteers

Consensus-based standards development

- Public reviews; open to all

Now supported by >300 member organizations

- Biopharma; CROs/service providers; tech providers, academic institutions, government agencies, non-profits

CDISC Groups

- U.S., Japan, Europe, China, South Korea
- Growing in India, Australia and other parts of the world
CDISC Audience

Clinical Investigators

Clinical Research Associates (Monitors)

Study Coordinators

Clinical Data Managers / CRF Designers

Clinical Data and Statistical Programmers

Biostatisticians

Drug Safety

Protocol & Study Report Authors

Interventional & Non-interventional

Clinical, Pre-Clinical, Non-Clinical

FDA Regulated and Unregulated

Regulatory Agencies, Industry, Academia, Healthcare
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Clinical, Pre-Clinical, Non-Clinical
FDA Regulated and Unregulated
Regulatory Agencies, Industry, Academia, Healthcare

Everyone involved in designing, capturing or using study-related data
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www.cdisc.org
CDISC Standards

Foundational Standards
- PLANNING
  - Protocol
  - Study Design
- DATA COLLECTION
  - CDASH
  - Lab
- DATA TABULATIONS
  - SDTM
  - SEND
- STATISTICAL ANALYSIS
  - ADaM

XML Data Exchange
- SDM-XML
- ODM
- Define-XML

Semantics
- Glossary
- BRIDG
- SHARE
  - Controlled Terminology

Implementations
- Therapeutic Areas
- Questionnaires
- Healthcare Link

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Moleded Core Domains

General Observation Classes

Interventions
- Concomitant Medications
- Exposure
- Substance Use

Events
- Adverse Events
- Disposition
- Medical History
- Deviations
- Clinical Events

Findings
- ECG
- Inclusion/Exclusion Criteria Not Met
- Labs
- Physical Exam
- Questionnaire
- Subject Characteristics
- Vital Signs
- Drug Accountability

Special Purpose
- Demographics
- Comments
- Subject Elements
- Subject Visits

Relationships
- SUPP--
- RELREC

Trial Design
- Trial Elements
- Trial Arms
- Trial Visits
- Trial Inclusion/Exclusion
- Trial Summary

2 characters in red underline indicate domain prefix
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www.cdisc.org
### Goal

Standardize efficacy data elements in 57 therapeutic areas by 2017, including clinical data terminology and implementation guides

- FDA intends to require use of these standards by 2017
- PDUFA V was approved
- PDUFA V Key Performance Goals; section XII E. ‘Clinical Terminology Standards’
- Enforceable guidance requiring CDISC for submissions is through public review

*PDUFA* = *Prescription Drug User Fee Act*

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#### Priority Disease/Domain Areas for Data Standardization

<table>
<thead>
<tr>
<th>Tier 1</th>
<th>Tier 2</th>
<th>Tier 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acne</td>
<td>Gastroesophageal reflux disease</td>
<td>Actinic keratoses</td>
</tr>
<tr>
<td>Alzheimer’s Disease*</td>
<td>Influenza</td>
<td>Decompensated CHF</td>
</tr>
<tr>
<td>Anti-diabetic agents*</td>
<td>Irritable bowel syndrome</td>
<td>Tinea pedis</td>
</tr>
<tr>
<td>Crohn’s Disease</td>
<td>Lipid-altering drug groups</td>
<td>Traumatic brain injury</td>
</tr>
<tr>
<td>Infections of skin and/or subcutaneous tissue</td>
<td>Major depressive disorder</td>
<td>General Anxiety Disorder</td>
</tr>
<tr>
<td>Oncology: time to efficacy event other than overall survival*</td>
<td>Objective tumor response*</td>
<td>Treatment of cough</td>
</tr>
<tr>
<td></td>
<td>Rheumatoid arthritis</td>
<td>Helicobacter pylori ulcer disease</td>
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<tr>
<td></td>
<td></td>
<td>Infectious diseases of the abdomen</td>
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<tr>
<td></td>
<td></td>
<td>Treatment of erectile dysfunction</td>
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<tr>
<td></td>
<td></td>
<td>Treatment of hepatitis B</td>
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</tbody>
</table>

*CDISC*
Core Philosophy

You decide what to collect/how to analyze based on science, regulation and business requirements
# Therapy Area User Guide Content

<table>
<thead>
<tr>
<th>Concept maps of key disease area clinical concepts</th>
</tr>
</thead>
<tbody>
<tr>
<td>Essential core data elements with definitions, data types, code lists and CDISC mappings</td>
</tr>
<tr>
<td>Implementation Guides with permissions statements</td>
</tr>
<tr>
<td>Data domains and examples</td>
</tr>
<tr>
<td>Standard CRFs with annotations, as appropriate</td>
</tr>
<tr>
<td>Analysis plans (to be incorporated later)</td>
</tr>
</tbody>
</table>
Concept Mapping

Sudy Subject
  has
  is enrolled in
  is a
  is participated in by
  includes
  has
  number

ID
  has
  Study
  Subject
  Visit

Temperature test
  results in
  Temperature measurement
    may have
    Normal range

Assessor
  reports

Clinical significance assessment
  reports
  results in
  Clinical significance result

Toxicity grade assessment
  reports
  results in
  Toxicity grade result

Clinical normality assessment
  reports
  results in
  Normal/abnormal result
# CDISC Therapeutic Area Standards

<table>
<thead>
<tr>
<th>Published Standards</th>
<th>Progress</th>
<th>Planned</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alzheimer’s Disease v1</td>
<td>Multiple Sclerosis v1</td>
<td>COPD v1</td>
</tr>
<tr>
<td>Parkinson’s Disease v1</td>
<td>Cardiovascular Endpoints v1</td>
<td>Cardiovascular Imaging v1</td>
</tr>
<tr>
<td>Polycystic Kidney Disease v1</td>
<td>Diabetes v1</td>
<td>Rheumatoid Arthritis v1</td>
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<tr>
<td>Tuberculosis v1</td>
<td>QT Studies v1</td>
<td>Schizophrenia v1</td>
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<tr>
<td>Virology v1</td>
<td>Hepatitis C v1</td>
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<tr>
<td>Asthma v1</td>
<td>Traumatic Brain Injury v1</td>
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<tr>
<td>Alzheimer’s Disease v2</td>
<td>Breast Cancer v1</td>
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<tr>
<td></td>
<td>Influenza v1</td>
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<tr>
<td></td>
<td>Dyslipidemia v1</td>
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</tbody>
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For more information on the CFAST Program see http://www.cdisc.org/therapeutic
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CDISC Harmonized Standards

<table>
<thead>
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<th>Objective</th>
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<tbody>
<tr>
<td>Aggregate sufficient data across partners to enable trustworthy research</td>
</tr>
<tr>
<td>analyses, including comparative effectiveness</td>
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<tr>
<td>Identify new biomarkers and link them to population characteristics and</td>
</tr>
<tr>
<td>outcomes</td>
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<tr>
<td>Reduce the ~17 year lag for research information to inform healthcare</td>
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<tr>
<td>decisions</td>
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<tr>
<td>Meet regulatory requirements</td>
</tr>
</tbody>
</table>

**IMPROVE PUBLIC HEALTH**
For More Information

Standards, Implementation Guides and TAUGs
www.cdisc.org

CDISC Education
Online: http:\\cdisc.trainingcampus.net
Classroom: www.cdisc.org/education
Thank you!