Data Standards in Clinical Trials, A Regulatory Perspective

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It’s A Difficult Road To A New Medicine

Source: PhRMA®
Clinical Trials Suffer High Failure Rates – Costly in Time and $$

Rate of Attrition for Drugs Entering Each Phase

In fiscal 2012, CDER received about 1280 study datasets per week, up to 10GB in size. 43% of effort in a review is spent on data management and primary analysis.
CDER’s Vision –

A standards based end-to-end fully electronic receipt, review, and dissemination environment that aids us in our mission to make safe and effective drugs available to the US Public
Great Baltimore Fire of 1904

Standards Are Key
How Will Clinical Content Standards Help?

- Improve efficiency of drug review
- Establish “common language” for disease and therapeutic areas through information models, concepts and controlled terminologies
- Facilitate use of sophisticated analytic tools
- Enable data sharing and data pooling
- Enhance the ability to perform complex analyses
- Build a foundation for broader benefits in clinical research, premarket analysis and safety signal detection
Example: OMOP Common Framework
Accommodating Disparate Observational Data Sources

Common Data Model

Standardized Terminologies

Drugs

Top-level concepts (Level 4)
Classifications (Level 3)
Ingredients (Level 2)
Low-level drugs (Level 1)
Source codes

Conditions

Mapping

Existing
De Novo
Derived
Example: RxNorm – Standardized Terminology Enables OMOP Research

Beyond translation to enabling structured analytics

Disparate Sources | Drug Codes | Normalized to RxNorm | Enabling Standardized Tools and Methods

Thomson | NDC | | Ingredient-based eras
GE | GPI | GPPC | Drug class definition
VA | VA Product | NDF-RT | HOI definitions
PHS | Multum | | Queries
GPRD | Multilex | FDB | Quality control

RxNorm Clinical drug Branded drug Ingredient Form Strength

Characterization tools
Database benchmarking
Indication based methods
Comparison of analytical methods
New Regulation Supports the Solution
FDA Safety and Innovation Act (FDASIA)

• Reauthorizes the fifth instance of Prescription Drug User Fee Act (PDUFA V)

• Authorizes the new Generic Drug User Fee Act and Biosimilars User Fee Act

• FDASIA Section 1136:
  – Allows FDA to require standardized fully electronic submissions related to marketing applications

• Phased-in through guidance to industry according to an agreed timetable
Clinical Terminology Standards: Using a public process that allows for stakeholder input, FDA shall develop standardized clinical data terminology through open standards development organizations with the goal of completing clinical data terminology and detailed implementation guides by FY 2017.

- FDA shall develop a project plan for distinct therapeutic indications, prioritizing clinical terminology standards development within and across review divisions. FDA shall publish a proposed project plan for stakeholder review and comment by June 30, 2013. FDA shall update and publish its project plan annually.
Many Activities In This Area
Examples of CDER Public-Private Partnerships

- Analgesic Clinical Trial Translations, Innovations, Opportunities, and Networks (ACTTION) Initiative
- Biomarker Consortium (BC)
- Cardiac Safety Research Consortium (CSRC)
- International Serious Adverse Events Consortium (iSAEC)
- Pediatric Anesthesia Safety Initiative (PASI)
- Critical Path Institute (CPath)
- Coalition Against Major Diseases Consortium (CAMD)
- Patient Reported Outcomes Consortium (PRO)
- Predictive Safety Testing Consortium (PSTC)
- Polycystic Kidney Disease Consortium (PKD)
- Critical Path to TB Drug Regimens Consortium (CPTR)
And Now CFAST

CDISC and Critical Path Institute Partnership

• Objective –
  Accelerate clinical research and medical product development by facilitating the creation and maintenance of data standards, tools, and methods for conducting research in therapeutic areas important to public health

• First Challenge –
  To progress content standards in therapeutic areas as identified by the aggressive PDUFA commitment to develop standards over the next 5 years
Closing thoughts...

• This area is rapidly evolving – data overload

• Standards are critical to facilitate both regulatory review and regulatory research

• Collaborations are critical – we can’t get there alone
Thank You