Getting to the Heart of the Matter: Partnerships for Understanding Cardiotoxicity

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FDA
FDA’s Mission Statement

“The FDA is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, our nation’s food supply, cosmetics, and products that emit radiation.

The FDA is also responsible for advancing the public health by helping to speed innovations that make medicines and foods more effective, safer, and more affordable; and helping the public get the accurate, science-based information they need to use medicines and foods to improve their health.”
Why Public-Private Partnerships?

- Any single party: limited resources (staff, funds, infrastructure, equipment...), time and expertise

  - Leverage resources and expertise among stakeholders to minimize costs (time and money)
  - Align missions toward mutually beneficial goals
  - Open new lines of communication among partners
  - Create value added for all stakeholders: optimizing economies of scale...advancing public health

- Whole = Greater than sum of individual parts/partners
- Implement economic law of comparative advantages
PPP Operating Principles

- Transparency: maintain public trust
- Inclusion of stakeholders
  - FDA, Industry, Academia, Professional Societies, patient advocates, other gov’t, ...
- Leveraging existing resources
  - Existing and prospective data: Data-sharing processes
  - Claims data
- Focused on Public Health and Efficient Product Development
Elements of a PPP

- Develop protocols, timelines
- Implement projects
- Determine public health questions
- Obtain stakeholder input
- Identify other efforts: join, expand
- Publish scientific articles
- Interpret scientific data
- Share resources: IP, data, time, etc.

- Develop business plan/strategy
- Create governance/Adm Oversight
- Identify & recruit partners
- Negotiate agreements, IP etc
- Administer/manage contracts
- Manage conflicts of interest
- Develop data sharing guidelines
- Develop reports/output analysis
- Secure funding
Some Elements of Business Model

• Pooling: IP, resources, expertise…
• Share Data: Proprietary, Pre-clinical, clinical, pro-competitive?
• Models: Sematech, SNP & Biomarker Consortia, C-Path Institute
• Contracts/Agreements with timelines, deliverables, specific terms and conditions? (Licenses: non-exclusive commercial, research-use?)
• Implement POC project/s
• Develop: Research/predictive tools, know-how, guidances
• Employ multiple mechanisms
General Funding Model

Whole is greater than sum of parts/partners

Private Only

Individually or in groups launch project/s

Private

Launch PPPs with combination of public/private funds with mutually beneficial goals and objectives

Public

FDA, NIH, CMS Other government

Public Only

Data, new guidances, best practices, informed clinical decisions, evidence based medicine Effective & safe medical products to patients faster, and more efficiently
Potential Sources of Funding

- State Funds
- Private Funders
- Appropriations
- Other...

PPP

Project 1
Project 2
Project 3
Basic Steps in Collaborating with FDA

(\textit{not comprehensive! not consecutive!})

- Start with the Public Health Need, SCIENCE!
- Identify Priorities for multiple stakeholders

- Identify gaps/opportunities (avoid duplication)

- Identify partners: define roles/responsibilities
- Co-develop: proposals, budgets, timelines etc.
- Leverage resources/expertise
- Implement joint \textit{Proof of Concept} projects
- Share data in public domain as quickly as appropriate: Pre & Pro-competitive tools

\textbf{Some Benefits}

- \textbf{PRIVATE PARTNERS:} Regulatory knowledge, Predictive Tools & Input in Project Selection
- \textbf{FDA:} Guidances, Standard-setting, Evaluative Tools
- \textbf{PATIENTS:} Faster, Safer and Cheaper Medical Products!!!
Data sharing

• FDA has the largest repository of clinical trial data in the world
• These data are a potential gold mine for new analyses to inform future development of medical products

, but…

• These data are not owned by FDA
• FDA is prohibited from disclosing Confidential Commercial Information
“FDA Data”

Sponsor’s Data Submitted to FDA

FDA-generated Data *

*(not to scale)
Mechanisms for sharing “FDA Data”

• Waiver from data owner

• Contractual or collaborative relationship (abides by same disclosure rules)
  – CRADA, CRA, SGE, Contract, etc.

• Publication of work performed at FDA

• Freedom of Information Act Request
The Other Way

- Sponsor’s Data
  - FDA
  - Research Access
Not a Panacea

• Data standardization is vital to subsequent pooling and analysis

• Existing studies may not have captured appropriate data to evaluate new questions

• There are no perfect clinical trials
Partnership Principles in Action

CSRC and the ECG Warehouse
Before...
ECG Warehouse: A Catalyst for the Cardiac Safety Research Consortium (CSRC)

Centralized repository for ECGs
- As flexible as data standard
- Archive in perpetuity

• Reviewer interface through browser
• Systematic collection and review of ECG waveforms as an adjunct to assessment of ECG intervals and overall interpretations
ECG Warehouse

- Powerful tool for reviewers
- Source of critical info. to inform future drug development
Collaboration Model: Cardiac Safety Research Consortium

INDUSTRY

ACADEMIA

PT. GROUPS

PROFESSIONAL ORGANIZATIONS

NON-PROFIT STATE, OTHER

FDA

Mortara/Academic

PUBLIC-PRIVATE CONSORTIUM

EXECUTIVE/STEERING COMMITTEES

WORKING GROUPS

DCRI

• NEUTRAL GROUND

• ADM. INFRASTRUCTURE

• GAP ANALYSES (WHAT’S KNOWN?)

• CO-DEVELOP PROPOSALS
  • BUDGETS
  • TIMELINES

IMPLEMENT PROJECTS