Regulatory Science

Innovation Stagnation

Challenge and Opportunity on the Critical Path to New Medical Products

View from The U.S. Food and Drug Administration (FDA)
Productivity Death Spiral

Booth and Zemmel, Nature Reviews in Drug Discovery 3:451-6, 2004
Independent 501(c)3
A single MISSION:

C-Path creates innovative collaborations in research and education that enable the safe acceleration of the process for developing new medical products
C-Path & FDA MOU
Effective October 14, 2005

Memorandum of Understanding Between the United States Food and Drug Administration and the C–Path Institute

AGENCY: Food and Drug Administration, HHS.

“purpose... to establish an overarching framework for collaboration... to foster development of new evaluation tools to inform medical product development”
Thirty Employees

Offices:
Tucson, AZ: 8,000 sf
Phoenix, AZ: 3,500 sf
Rockville, MD: 3,500 sf
C-Path’s Consortia Model

- Multiple Companies
- Formal Legal Agreement
- Precompetitive Neutral ground

Involves:

- A
- B
- C
- D
- E

- FDA
- EMA
- Patients
- NIH
- Academia
C-Path’s Leveraged/Neutral Model

- FDA and AHRQ
- Foundations
- Philanthropy

Critical Path Institute

C-Path’s Programs

- Foundation for NIH
- Innovative Med. Initiative
- Regulated Industry

Research Grants
Consortia fees for Research
C-Path’s Consortia Addressing Regulatory Science

- Predictive Safety Testing Consortium (PSTC)
  **DRUG SAFETY**
- Patient-Reported Outcomes (PRO) Consortium
  **DRUG EFFICACY**
- Coalition Against Major Diseases (CAMD)
  **SHARING CLINICAL DATA (Placebo/control)**
- Critical Path to TB Drug Regimens (Gates)
  **SCIENCE TO SUPPORT DRUG COMBINATIONS**
Participants in C-Path’s Consortia

28 Major Pharmaceutical Companies
FDA and EMEA
NIA, NIAMS, NINDS, NCI, NHLBI, NIDDK
FNIH, IMI, Gates, CDC, WHO, Brookings
Seven Patient Advocacy Organizations
Over 600 Scientists
A Global Endeavor

>600 Scientists

Consortia Members and Advisor Locations

Spans 17 Time Zones!
Qualification of New Tools

A new pathway.....

Planning Phase
- Legal Agreement, Coordinating Committee, Planning etc
- Work Scope Document

Execution Phase
- Working Groups
  1. ...
  2. ...
  3. ...
  4. ...
- Methods & Results Sharing

FDA Review Phase
- FDA Submission
- FDA Review
- Qualified Methods

Greater Efficiency & Safety

Scientific Consensus
Path Submissions

**PSTC**
- 4Q09
- 1Q10
- 2Q10
- 3Q10
- 4Q10
- 1Q11
- 2Q11
- 3Q11
- 4Q11

**CAMD**
- 4Q09
- 1Q10
- 2Q10
- 3Q10
- 4Q10
- 1Q11
- 2Q11
- 3Q11
- 4Q11

**PRO**
- 4Q09
- 1Q10
- 2Q10
- 3Q10
- 4Q10
- 1Q11
- 2Q11
- 3Q11
- 4Q11

- **Briefing Package**
- **Qualification Package**
- **Scoping Summary**
- **Research Summary**
- **PRO Qualification**
Critical Path to TB drug Regimens (CPTR) Initiative

Led by

The Bill and Melinda Gates Foundation (BMGF)

A collaboration to accelerate the development of new, safe and highly effective regimens for TB by enabling early testing of drug combinations.
Objective of the CPTR Tools Consortium

Develop a scientific consensus on which methods are “qualified for use” in TB drug development

Find consensus among......

1) those who will use the methods (industry),

2) those who will accept the methods (FDA, EMEA, others).
Summary: Needed for Innovative Drug Development

- Common data elements in development
- Biomarkers “qualified for use”
- PRO instruments “qualified for use”
- Innovative tools/methods for trial design
  - Adaptive clinical trial design
  - Trial simulation using disease models
- Innovative Business Models