How Can the Regulator and Regulated Work Together?

Martha A. Brumfield, Ph.D.
Director of International Programs
Critical Path Institute
Areas For Focus

Transparency

Global alignment

Engagement in trying new paradigms
Objective of Critical Path

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

1) those who will **use the methods** (industry, academics),
   **AND**

2) those who will **accept the methods** (FDA, EMA, PMDA).
The Innovative Medicines Initiative: the Largest PPP in Life Sciences R&D

Focused, government endorsed approach to address needs in innovation
C-Path’s Consortia Model

Multiple Companies

Formal Legal Agreement

Precompetitive Neutral ground

C-Path

EMA

PMDA

FDA

Patients

NIH

Academia
C-Path’s Consortia Model

30 Major Pharmaceutical Companies

FDA, EMA (EU), PMDA (Japan)

NIA, NINDS, NCI, NHLBI, NIDDK

Eight Patient Advocacy Organizations
A Global Endeavor
>750 Scientists

Consortia Members and Advisor Locations

Spans 17 Time Zones!
C-Path’s Key Principles

- Trusted and neutral third party
- Consortia model facilitates collaboration on applied and regulatory science
- Use of legal agreements
- Focused on “qualification” process and not product
- Sharing information – success and failures
- Public-Private Partnerships
C-Path’s Vision

Trusted Third Party

- Public & foundation funding of infrastructure
  - No funding from regulated companies
- State and Federal Grants
- Philanthropy
- Transparency
- FDA, EMA and PMDA participation
What Can Be Shared

- Work to Define:
  - Common Data Elements
  - Performance Standards

- Methods
  - Safety Testing; Efficacy Testing; Other?

- Knowledge of Diseases
- Applied Science Research
- Regulatory Science Research
C-Path’s Consortia Addressing Regulatory Science

- Predictive Safety Testing Consortium (PSTC)(15) 
  **DRUG SAFETY**

- Patient-Reported Outcomes (PRO)(26) 
  **DRUG EFFICACY**

- Coalition Against Major Diseases (CAMD)(13) 
  **SHARING CLINICAL DATA (Placebo/control)**

- Critical Path to TB Drug Regimens (Gates)(14) 
  **SCIENCE TO SUPPORT DRUG COMBINATIONS**
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.
C-Path Accomplishments

- **Regulatory engagement**
  - Ability to discuss overall program goals with regulators

- **Cross border collaboration – IMI’s and C-Path**
  - Discussions ongoing for enhanced cross border collaboration on specific projects

- **Consortia Model Demonstrated Successful**
  - Provides mechanism for scientific experts to exchange data and information
Utility of Model

- Have biomarker qualifications positively impacted a new medicine’s development?
  - Anecdotal evidence is positive
  - C-Path survey suggests use by companies

- Do regulators see this as value added?
  - FDA funding continues & feedback is positive
  - EMA granted fee reduction due to “address imperative reasons of public health”

- How do we measure impact on accelerated development?
  - Time will tell
Lessons Learned

- Must have buy in on methods, data, tools from broad segment of stakeholders – regulators, regulated, academics, patient groups
  - Consortia model fosters dialogue

- Success depends on resource availability
  - Industry appears committed
  - Regulators seem resource constrained

- C-Path staff provide neutral leadership and project management & maintain momentum
Thank You