Critical Path Initiative: An Update

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President and CEO, Critical Path Institute

Presented at:

FIRST ANNUAL

PATIENT-REPORTED OUTCOMES (PRO) CONSORTIUM WORKSHOP

March 23, 2010 – Bethesda, MD

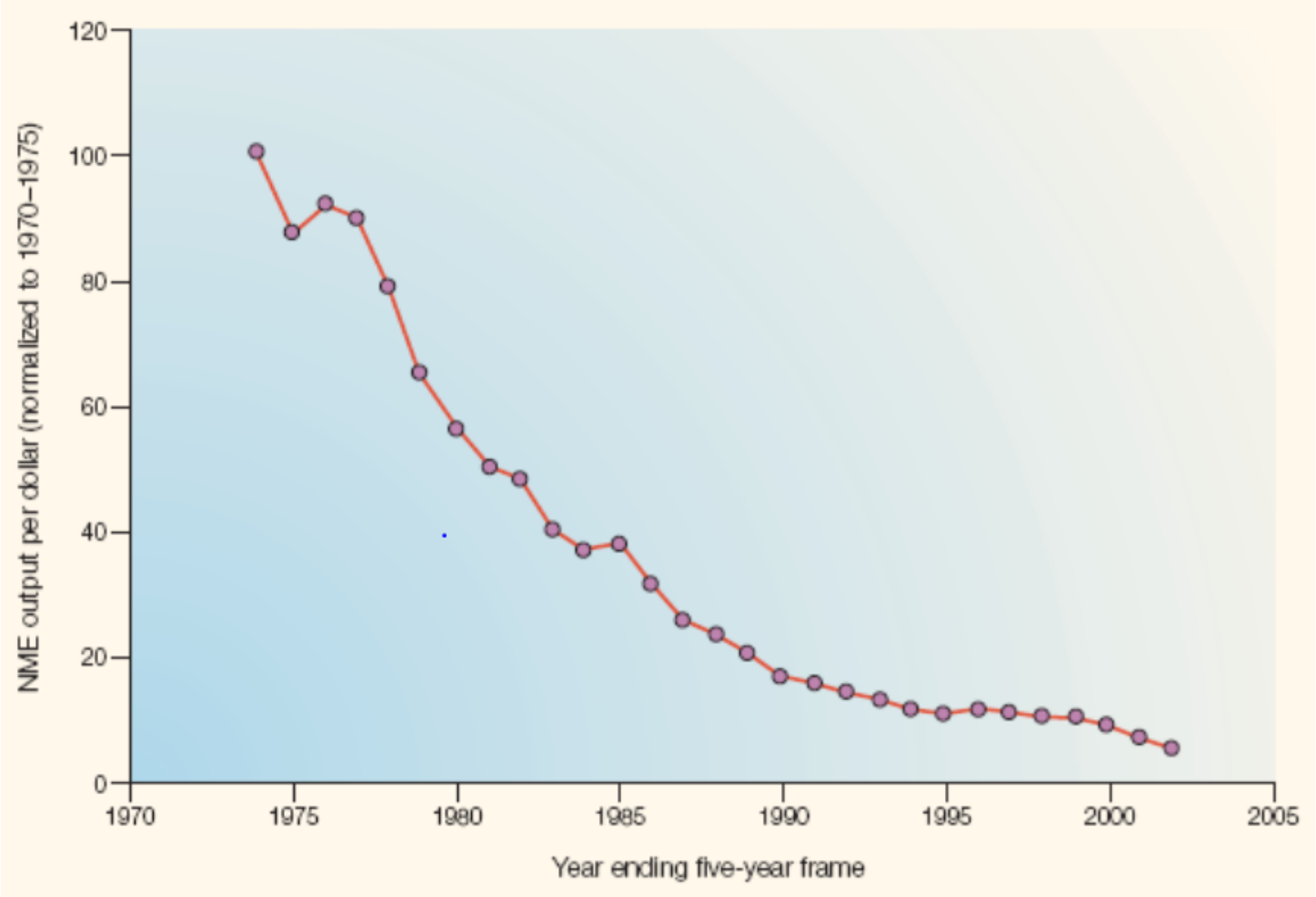
Co-sponsored by

CRITICAL PATH INSTITUTE

FDA

Regulatory Science

Productivity Death Spiral



Booth and Zemmel, Nature Reviews in Drug Discovery 3:451-6, 2004

Critical Path Institute - 2005

PRO CONSORTIUM

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

Federal Register / Vol. 70, No. 241 / Friday, December 16, 2005 / Notices 74823

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

AGENCY: Food and Drug Administration, HHS.

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[FDA 225–05–8000]

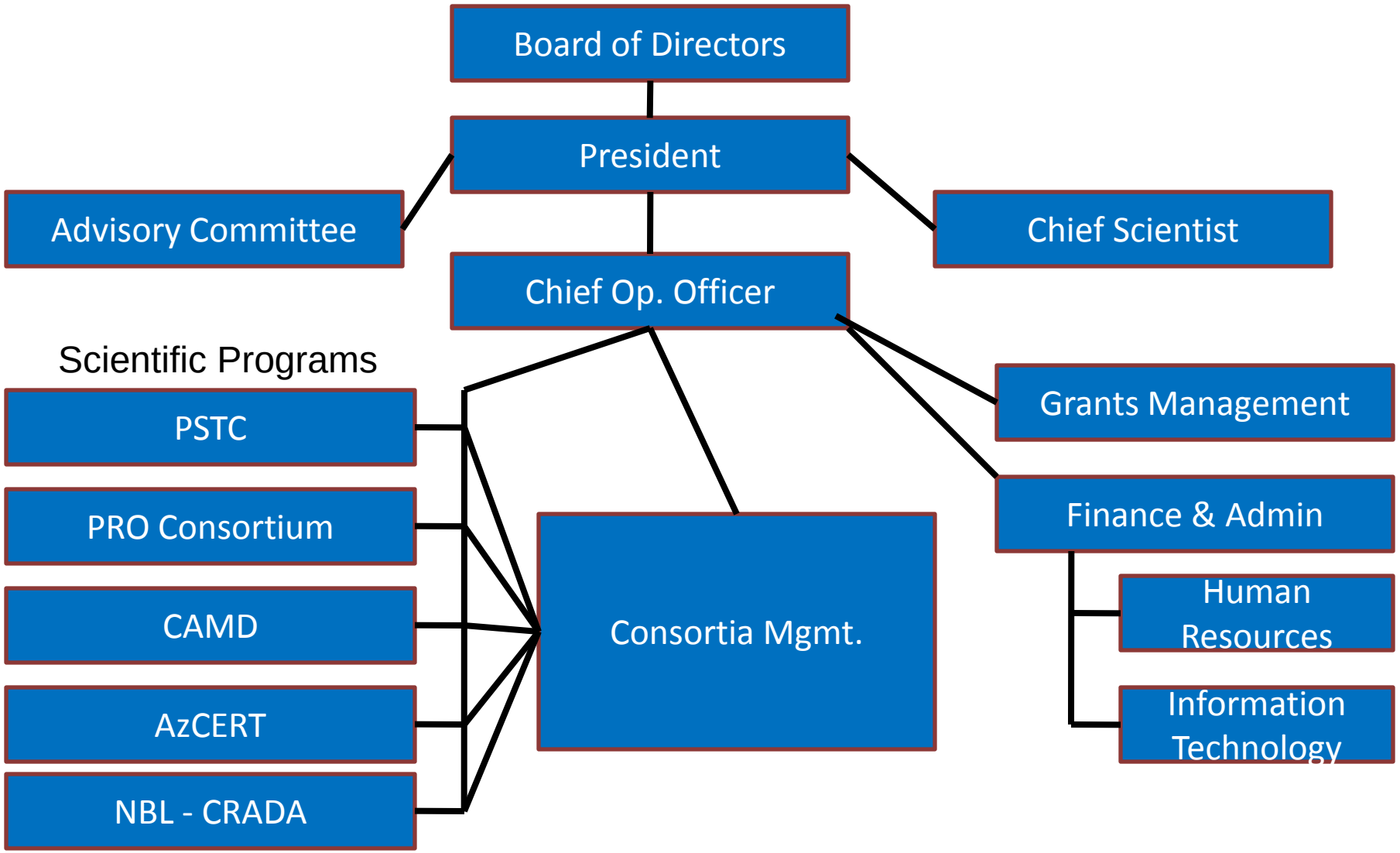
Memorandum of Understanding Between the United States Food and Drug Administration and the C–Path Institute

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

"purpose… to establish an overarching framework for collaboration… to foster development of new evaluation tools to inform medical product development"

C-Path's Structure



Critical Path Institute - 2010

Thirty Employees

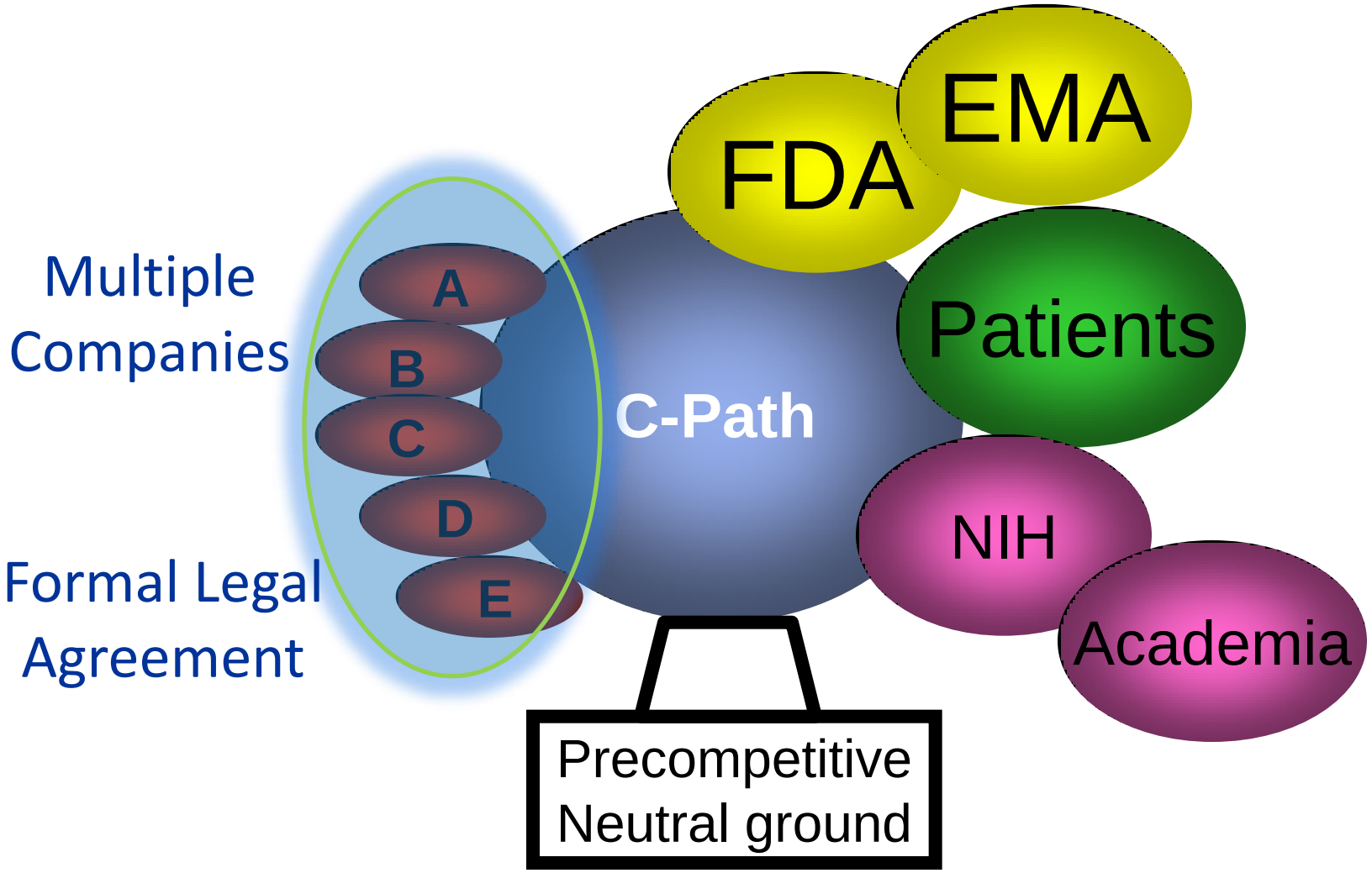
Offices:

Tucson, AZ: 8,000 sf

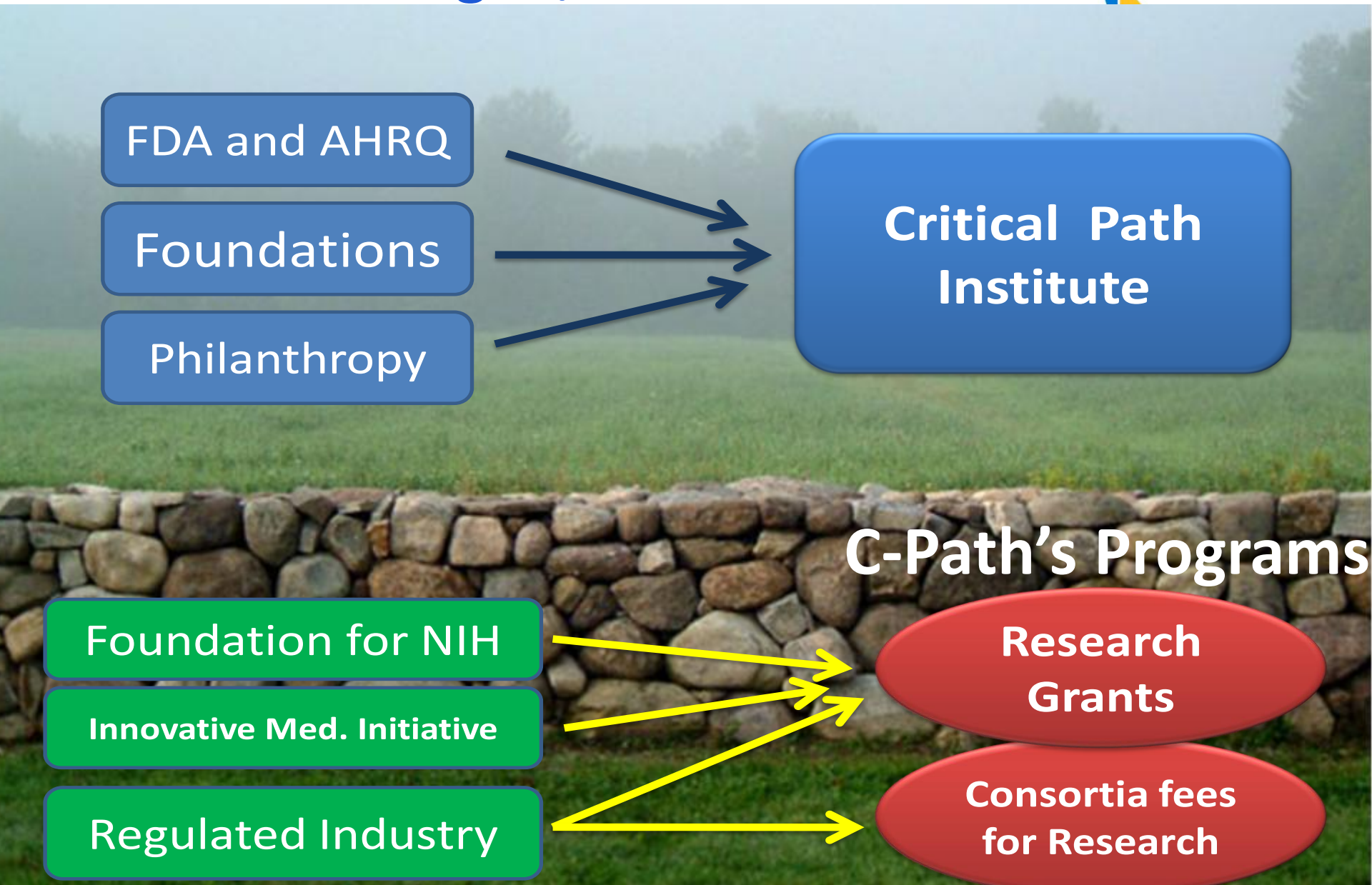
Phoenix, AZ: 3,500 sf

Rockville, MD: 3,500 sf

C-Path's Consortia Model



C-Path's Leveraged/Neutral Model



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

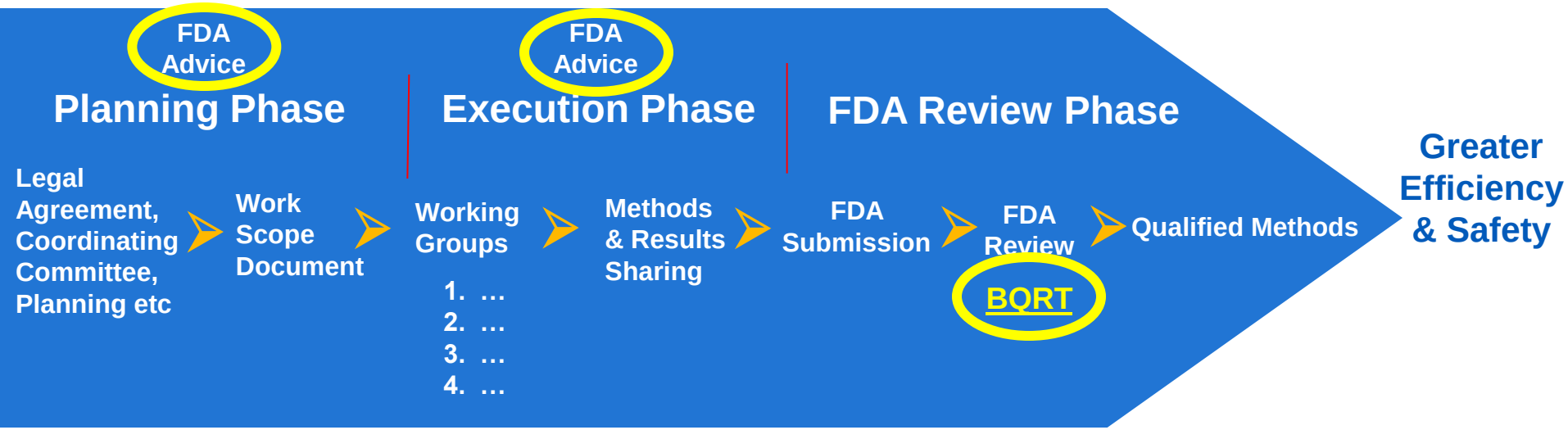
A Global Endeavor
>600 Scientists

Consortia Members and Advisor Locations

Qualification of New Tools

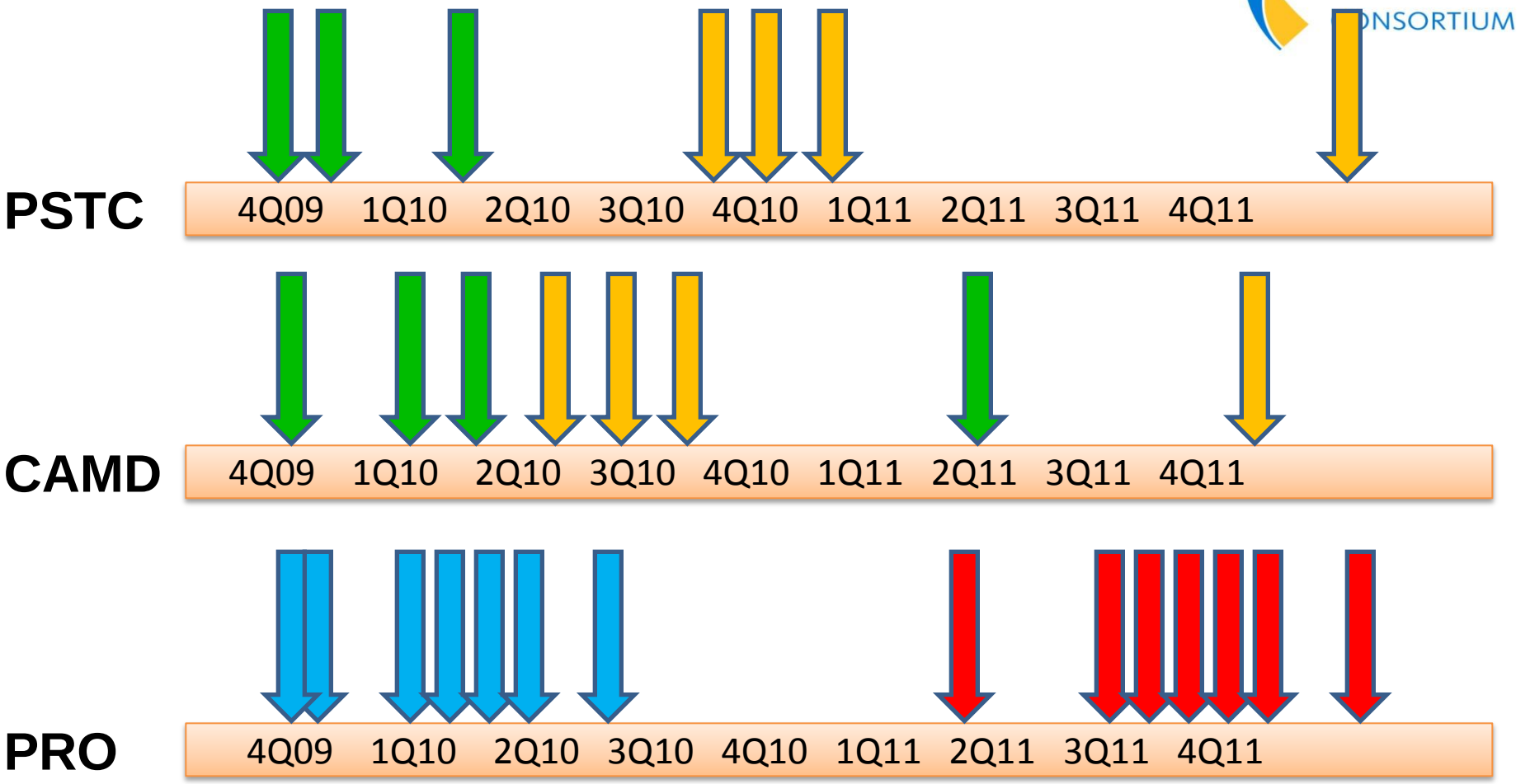
PRO CONSORTIUM

A new pathway…..



Scientific Consensus → FDA

C- Path Submissions



Critical **P**ath to **T**B drug **R**egimens (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

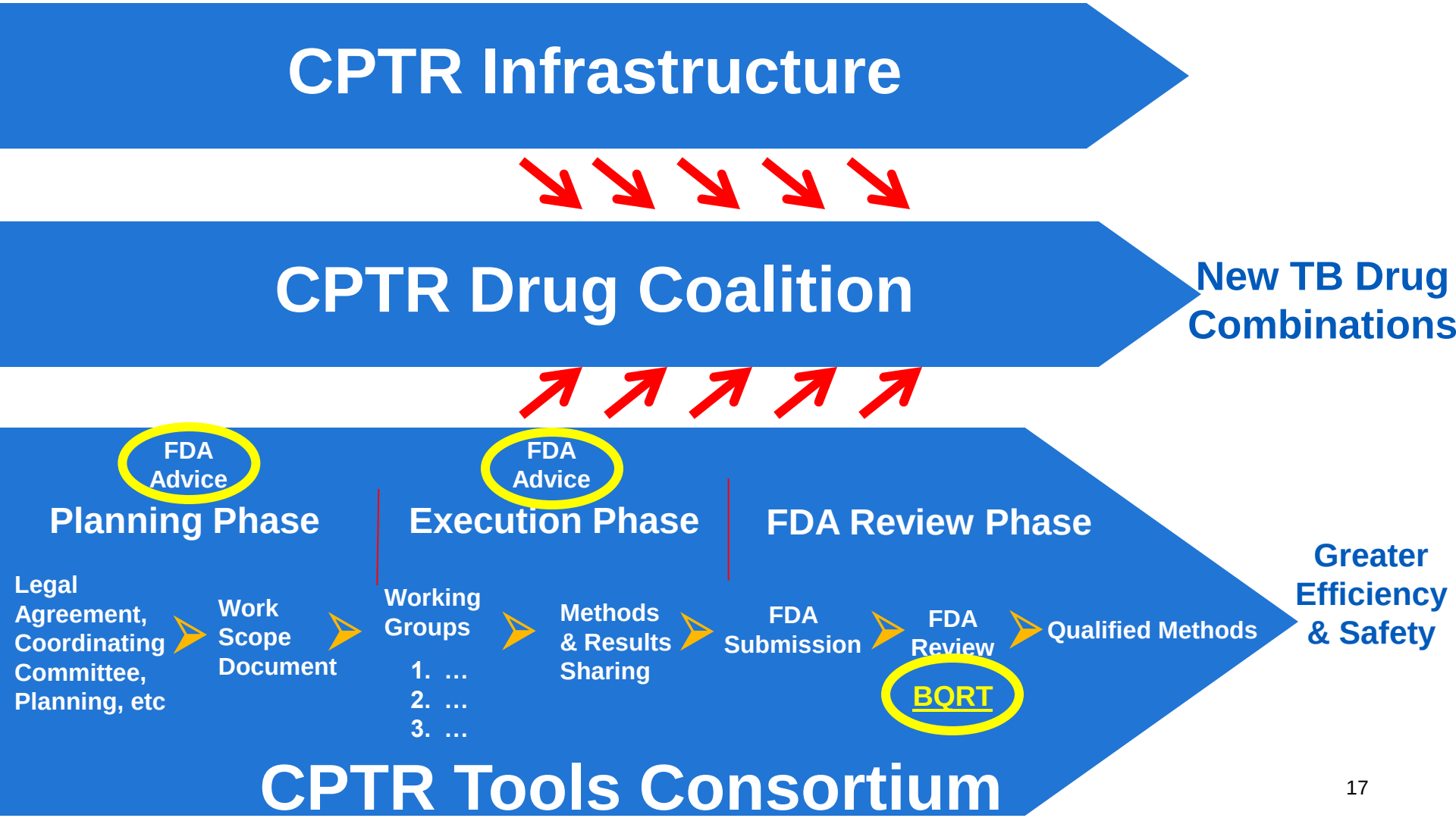
"qualified for use" in TB drug development

Find consensus among……

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

2) those who will **accept the methods** (FDA, EMEA, others).

CPTR Initiative



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