Public-Private Partnerships to Support Pediatric Trials

Pediatric Trials Consortium

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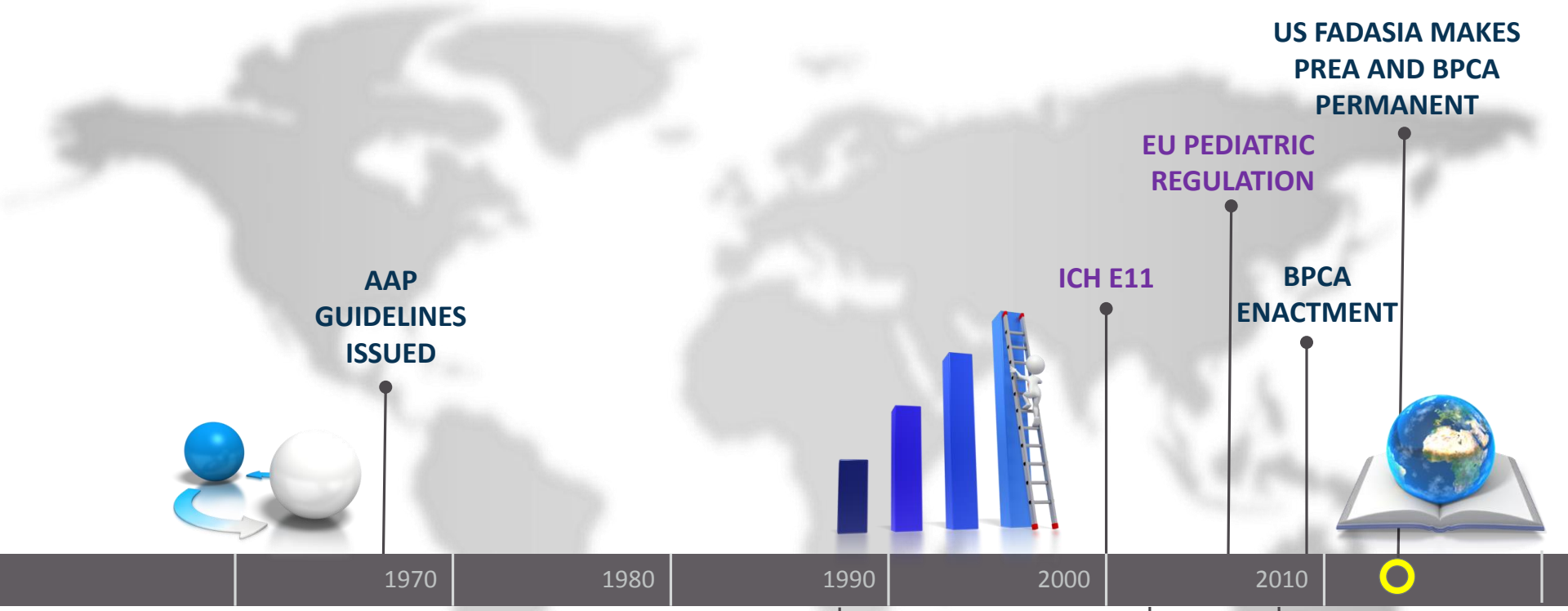
PEDIATRIC TRIALS CONSORTIUM
CRITICAL PATH INSTITUTE

IMPRESSIVE HISTORY OF PEDIATRIC LEGISLATION



608 NEW PEDIATRIC LABELS UNDER PREA AND BPCA

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SIGNIFICANT GAP REMAINS IN PEDIATRICS

- More than **50%** of all drugs used in children do not have adequate information in their labels
- More than **90%** have not been studied in newborns

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Few Patients
Per Site

Multiple
Countries
More Sites

Multiple Age Based Population

Pediatric Clinical Trials

Awareness And Commitment

More Time
Start-Up
Higher Costs

Design
Validated Endpoints
Relevance

Tools
Best Practices
Staff

Sustainable Infrastructure

Child Friendly Formulations

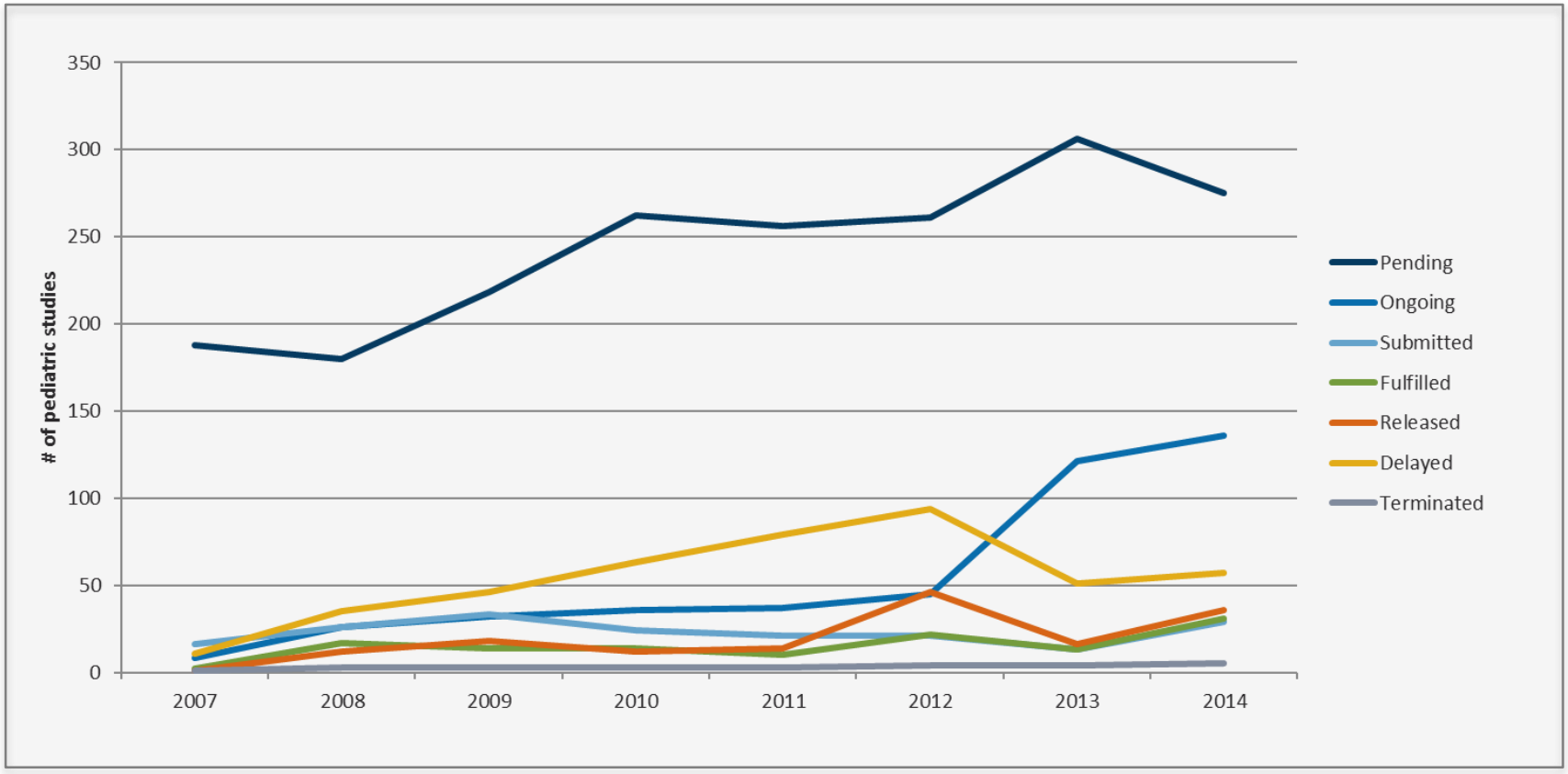
Stakeholder
Planning and
Strategy

Recent Progress But Still Struggling

Annual Snap Shot at Each Year End
Recent shifts are promising

United States

FDA Data Base Accessed March 11.2016 http://www.fda.gov/downloads/scienceresearch/specialtopics/pediatrictherapeuticsresearch/ucm308465.pdf



Next Major Step After Landmark AAP Forum

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AAP, NIH, FDA, EMA, PhRMA, BIO, Academia, Patient Advocates and Industry

Special article: 2014 AAP Stakeholder's Forum Planning Committee

Clifford Bogue, Linda A. DiMeglio, Samuel Maldonado, Ronald J Portman, P. Brian Smith, Janice E. Sullivan, Charles Thompson, Heide Woo and Susan Flinn; on behalf of The Pediatric Clinical Trials Stakeholder Forum Planning Committee. Pediatr Res doi:10.1038/pr.2015.255

Next Major Step After Landmark AAP Forum

*Addressing these challenges requires all children's health stakeholders to move beyond the current fragmented and insular approach, create a **shared new vision** for pediatric clinical trials, and then **collaborate to implement** that vision.*

*The attendees at the AAP Stakeholder Forum Planning Committee resolved to establish **a global pediatric clinical trials network** and are committed to engage in the work to create and sustain it.*

Adapted from: Clifford Bogue, Linda A. DiMeglio, Samuel Maldonado, et al. *Pediatr Res*
doi:10.1038/pr.2015.255

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Next Major Step After Landmark AAP Forum

Critical Path Institute established the Pediatric Trials Consortium to catalyze this initiative…..

*"Health care providers must have high-quality, reliable data to inform treatment decisions for their pediatric patients. C-Path has leveraged its expertise and stakeholders to support the development of a first-of-its-kind pediatric clinical trial network. **The [Pediatric Trials] Consortium signals an important step toward efficient and sustainable pediatric data collection and development of better treatments for children.** I strongly support this effort and look forward to working with this global consortium."*

Dr. Janet Woodcock, Director of the U.S. Food and Drug Administration's (FDA) Center for Drug Evaluation and Research (CDER)

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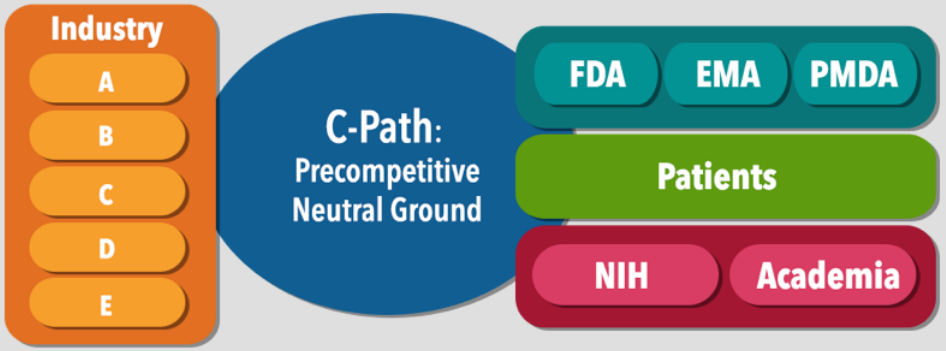
C-Path: A Public-Private Partnership

In response to Critical Path Initiative, the Critical Path Institute was formed as an independent 501(c)3 founded in 2005 "… to foster development of new evaluation tools to inform medical product development"

- Act as a trusted, neutral third party
- Convene scientific consortia of industry, academia, and government for sharing of data/expertise
  - ✓ The best science
  - ✓ The broadest experience
  - ✓ Active consensus building
  - ✓ Shared risk and costs



- Enable iterative EMA/FDA/PMDA participation in developing new methods to assess the safety and efficacy of medical products
- Official regulatory endorsement of novel methodologies and drug development tools

C-Path Consortia – December 2015

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Twelve global consortia collaborating with 1,300+ scientists and 61 companies

CAMD — **Coalition Against Major Diseases**
Focusing on diseases of the brain

CFAST — **Coalition For Accelerating Standards and Therapies**
Data standards

CPP — **Critical Path for Parkinson's Consortium**
Enabling clinical trials in Parkinson's Disease

CPTR — **Critical Path to TB Drug Regimens**
Accelerating the development of TB drug regimens and diagnostics

D-RSC — **The Duchenne Regulatory Science Consortium**
Duchenne Muscular Dystrophy

INC — **International Neonatal Consortium**
Neonatal clinical trials

MS — **Multiple Sclerosis Outcome Assessments Consortium**
Measuring drug effectiveness in MS

PKD — **Polycystic Kidney Disease Outcomes Consortium**
New imaging biomarkers

PRO — **Patient-Reported Outcome Consortium**
Assessing treatment benefit

ePRO — **Electronic Patient-Reported Outcome Consortium**
Electronic capture of treatment benefit

PSTC — **Predictive Safety Testing Consortium**
Drug safety

PTC — **Pediatric Trials Consortium**
Developing effective therapies for children

- ✓ Biomarkers
- ✓ Clinical outcome assessment instruments
- ✓ Clinical trial simulation tools
- ✓ Data standards
- ✓ In vitro tools

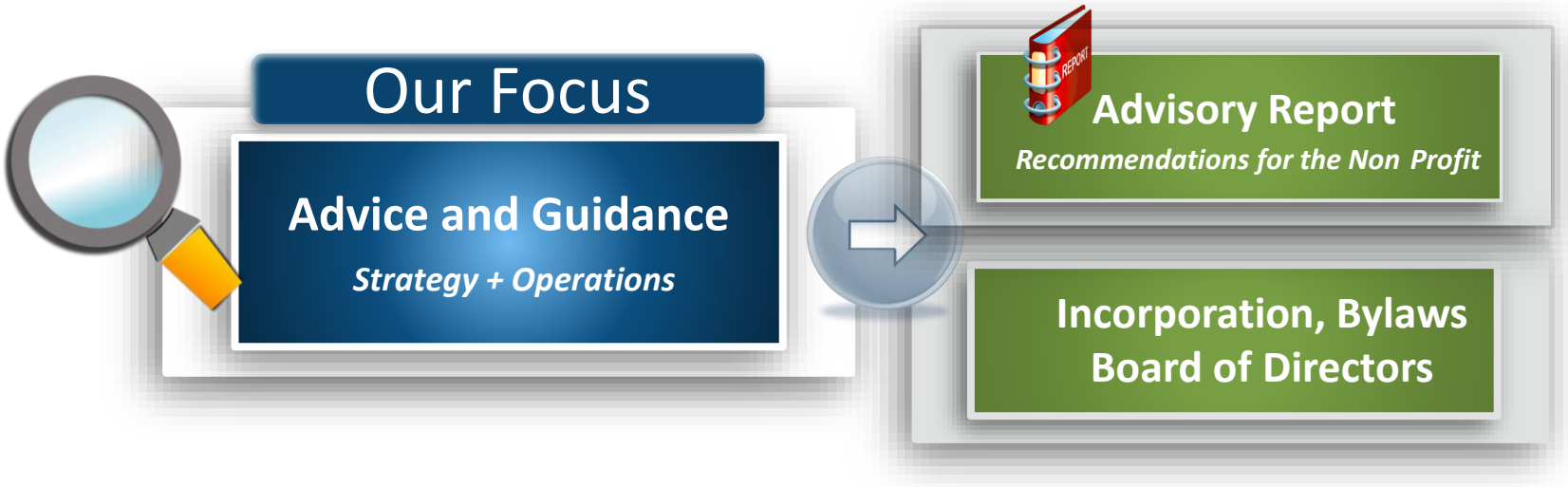
About the Pediatric Trials Consortium

PTC is committed to enabling the creation of a **sustainable solution** that assures the timely and efficient evaluation of ***innovative drugs, biologics and devices for children*** by delivering the ***regulatory-quality data*** needed for product labeling.

It will provide the support and guidance necessary for **C-Path to create a new freestanding non-profit organization** ("the Non-Profit") that will work independently, but with input across relevant sectors.

About the Pediatric Trials Consortium

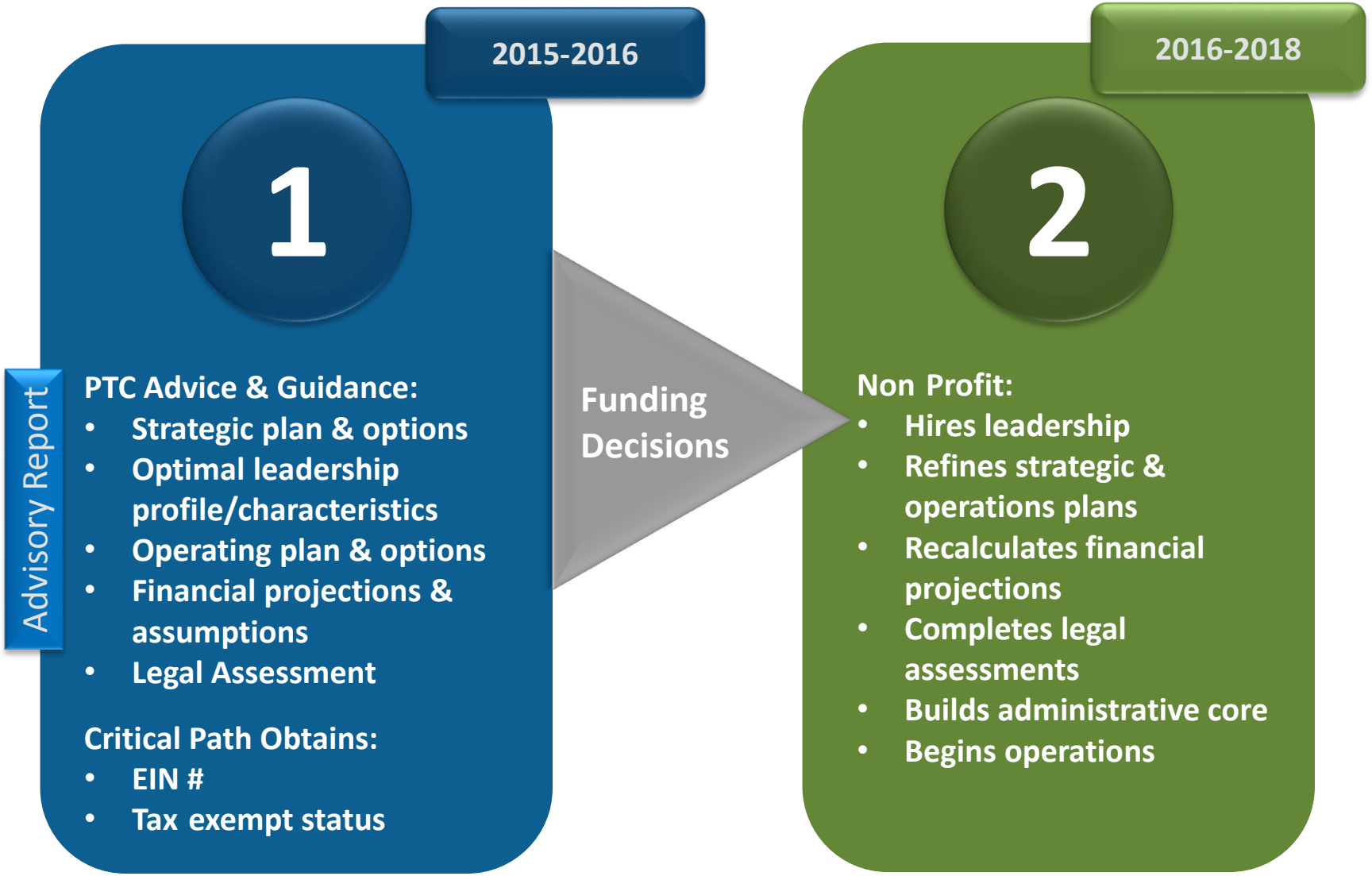
- Involves 32 diverse global stakeholders organizations
- Focused on pediatric product development & clinical trials
- Neutral and collaborative independent forum
- Launched October 2015
- Overseen by Coordinating Committee (with 3 Subcommittees) with 5 Work Streams focused on key areas
- Slated to complete work by the end of 2016



PTC: Current Participating Organizations

- American Academy of Pediatrics
- Arkansas Children's Hospital
- Children's Hospital Argentina
- Children's National Health System
- Clinical Research Alliance, LLC
- Connecticut Children's Medical Center
- Critical Path Institute
- Drug Information Association (DIA)
- Duke University
- Eli Lilly & Company
- University of Utah
- Food & Drug Administration (FDA)
- Independent Patient Advocates
- Innovative Medicines Initiative (IMI)
- International Children's Advisory Network (iCAN)
- Istituto G. Gaslini (Italy)
- Johnson & Johnson
- National Center for Child Health and Development (Japan)
- National Institute for Child Heath and Human Development (NICHD)
- Nationwide Children's Hospital
- Novartis Pharmaceuticals Corporation
- Paul Hastings, LLC
- Pfizer
- Pharmaceutical Research and Manufacturers of America (PhRMA)
- Rady Children's Hospital - San Diego
- Rusing, Lopez & Lizardi, PLLC
- Sczudlo Advisors, LLC
- Tufts University
- University of British Columbia
- University of Liverpool
- University of Michigan - Mott Children's Hospital

PTC Deliverables



A non-profit research, scientific, educational organization to enable and advance pediatric clinical research

Vision

*Facilitate the development of **innovative drugs, biologics and devices** according to the highest ethical and scientific standards to help extend and enhance the lives of children.*

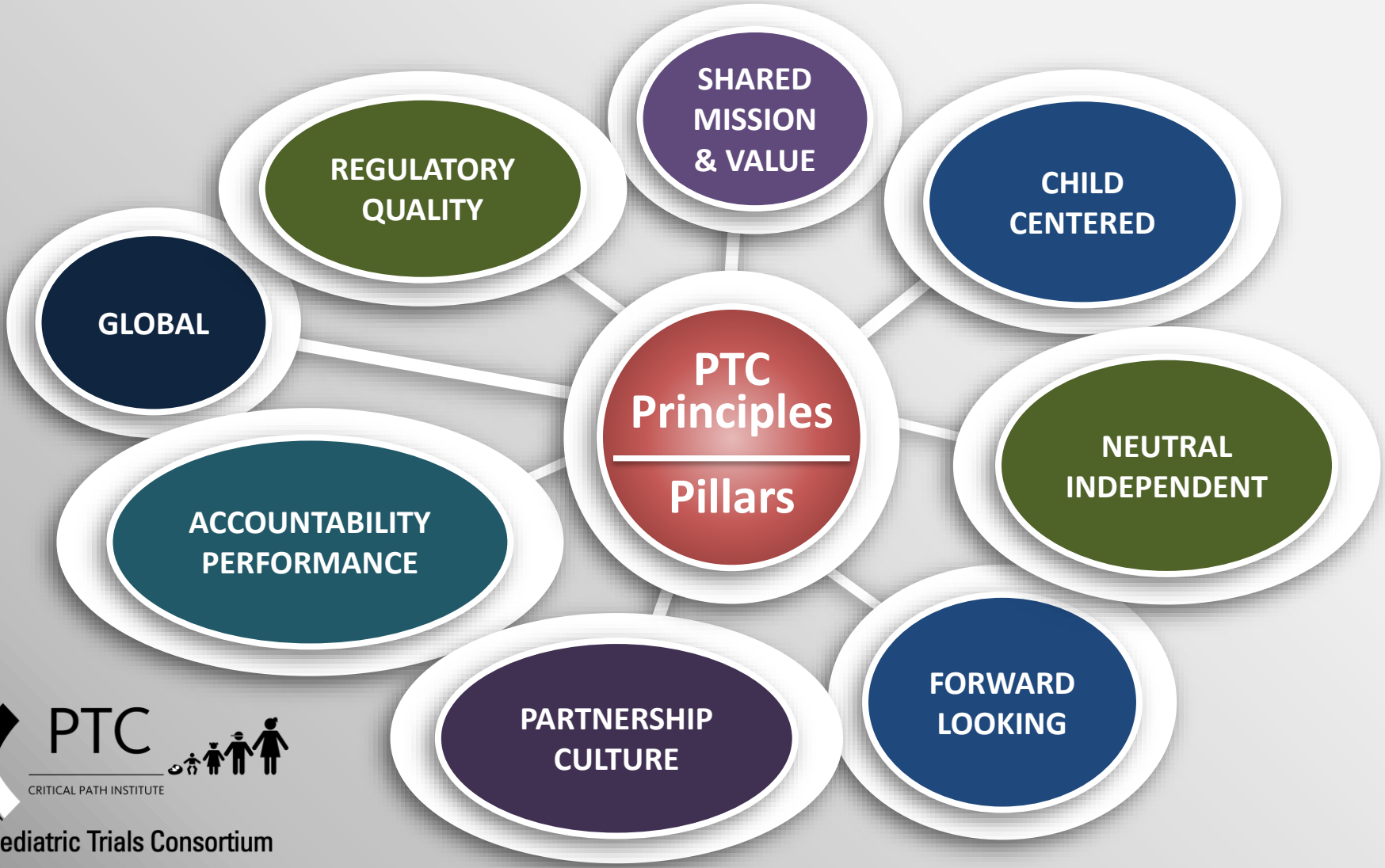
Mission

*Create and maintain a **sustainable infrastructure** to plan, start up, conduct and close out pediatric clinical studies that will enable the attainment of the vision. Partner to build new or connect with existing organizations/networks to enable the global execution of high-quality clinical trials which adhere to regulatory standards.*

Envisioned New Independent Non-Profit Organization

Public-Private Partnership

PTC KEY PRINCIPLES IN FORMING NEW NON-PROFIT



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Non-Profit Proposed Scope and Focus

PRODUCT DEVELOPMENT STRATEGY AND PLANNING

Getting it "right" the first time…
Early input from stakeholders
Design, Feasibility, Input

CAPABILITIES, TOOLS, EDUCATION AND BEST PRACTICES

Institutionalizing expertise…
Common processes and materials

INFRASTRUCTURE AND TRIALS EXECUTION

Sustainable Centers of Excellence in Product Development

ACCOUNTABILITY and PERFORMANCE METRICS
REGULATORY QUALITY DATA
MULTI-SPECIALITY
SYNERGISTIC WITH EXISITING RESOURCES

Capabilities & Trial Execution

Status of PTC as of March 2016

- More than 45 senior international leaders are actively engaged in PTC's deliverables.
- Advisory Report is on track for completion
- Critical Path Institute has completed work on Certificate of Incorporation and Bylaws for the new non-profit

NEXT 3-6 Months

- Complete Advisory Report
- Legal review/assessment of Advisory Report
- Critical Path filing of corporate documents for new non-profit
- File IRS application for Tax Exempt Status

Launch the new independent non-profit public-private partnership in early 2017

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For More Information: www.c-path.org/programs/ptc

Or contact Ed Connor econnor@c-path.org.