

Role of Patient Advocacy Groups in Accelerating Drug Development

CALBIO2012

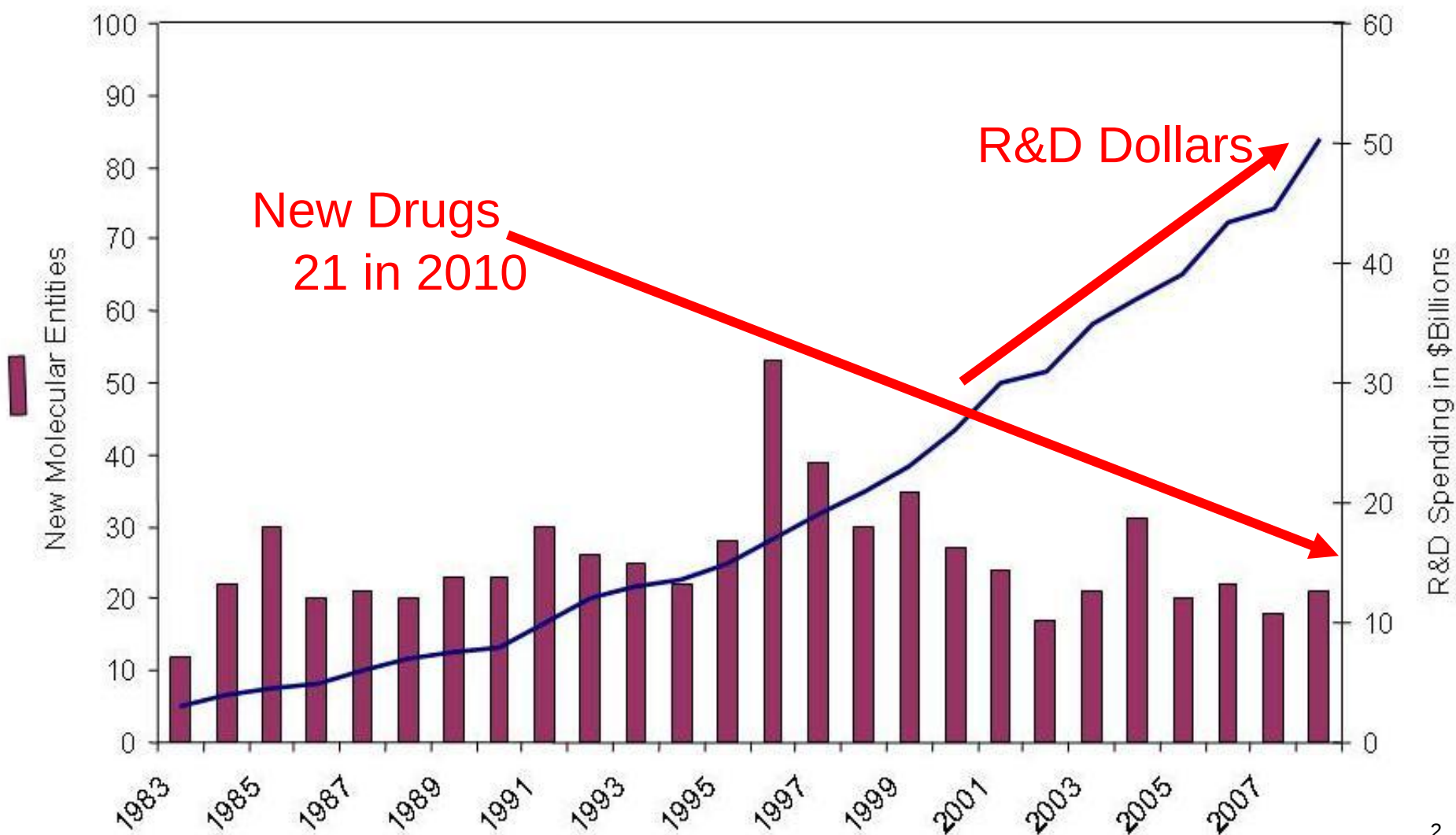
March 8, 2012

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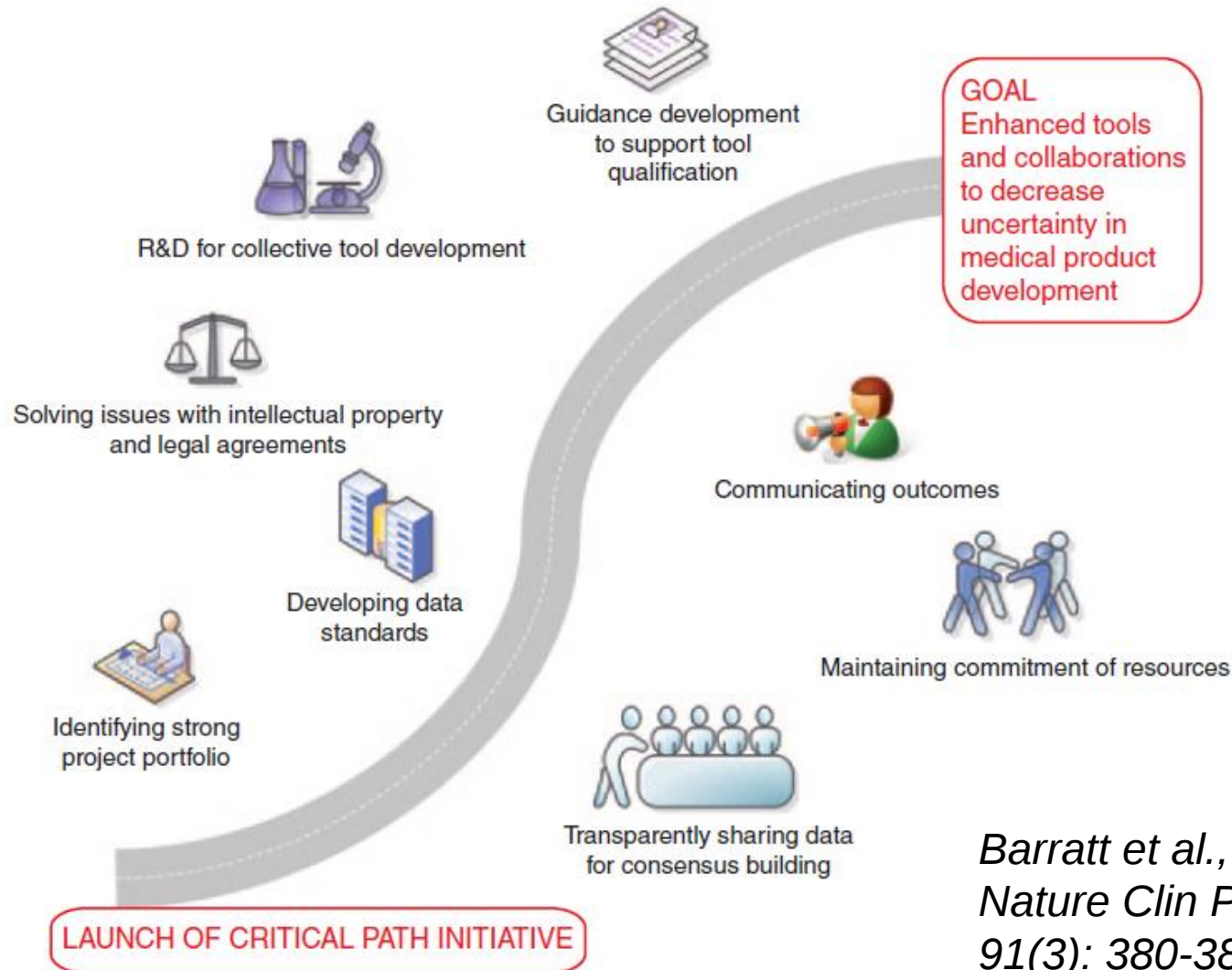
R&D Expenses Increasing New Drugs Not Increasing



FDA's 2004 Message: Find the "Critical Path"



Delivering on the FDA's Critical Path Initiative



*Barratt et al.,
Nature Clin Pharm Therap
91(3): 380-383, 2012*



***To improve health and save lives
by accelerating the development of
safe, effective medicines.***

Consortia for Creating Consensus



Predictive Safety Testing Consortium
DRUG SAFETY



Patient-Reported Outcome Consortium
DRUG EFFECTIVENESS



Coalition Against Major Diseases
**UNDERSTANDING DISEASES
OF THE BRAIN**



Polycystic Kidney Disease Consortium
NEW IMAGING TESTS



Critical Path to TB Drug Regimens
TESTING DRUG COMBINATIONS



- ☐ ***Biomarkers***
- ☐ ***Patient
Reported
Outcomes***
- ☐ ***Disease
Progression
Models***
- ☐ ***Data
Standards***



C-Path Collaborators



Industry



Partners

Patients

Research

Government

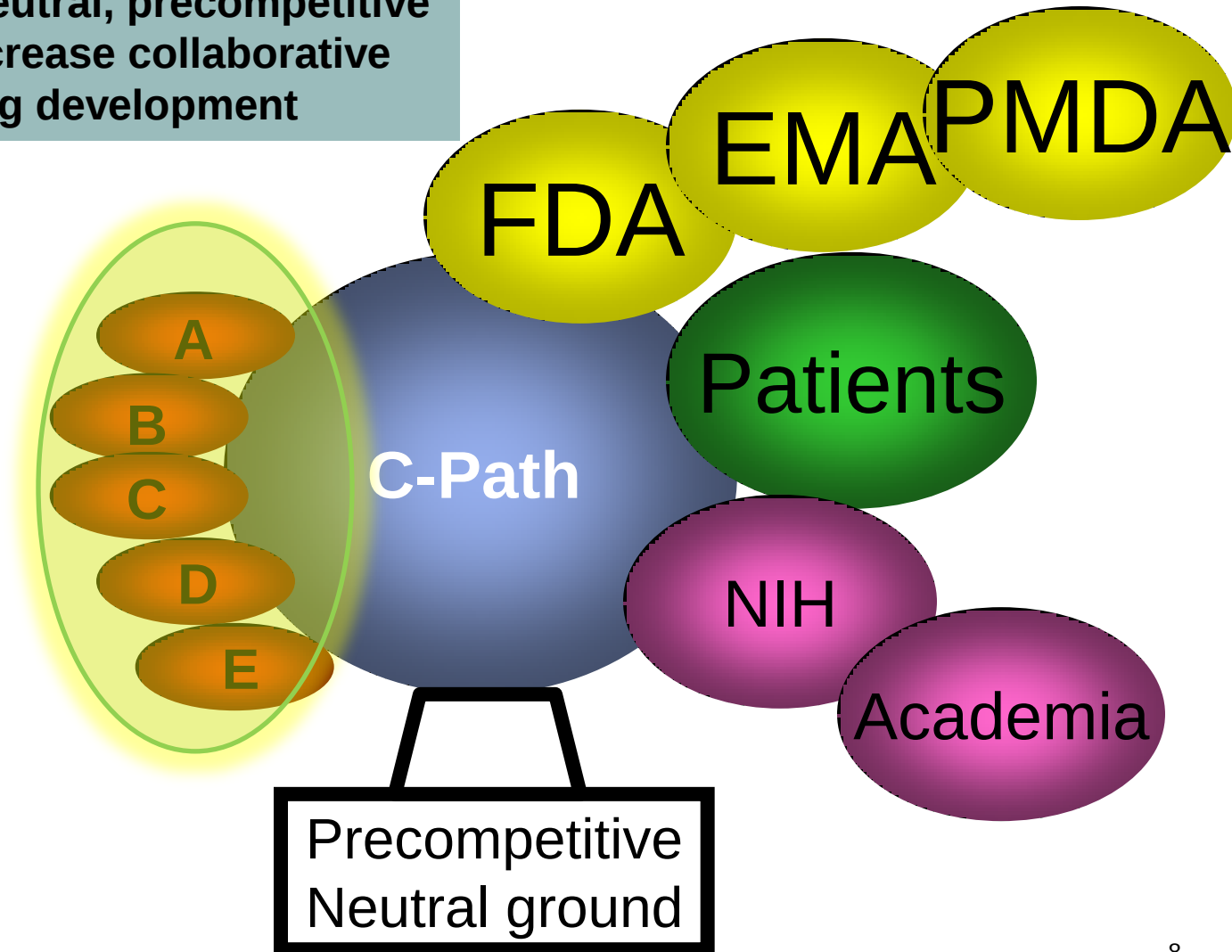


Consortia Model

Critical Path Institute (C-Path) has developed a consortium structure that provides a unique neutral, precompetitive environment to increase collaborative efforts for drug development

Multiple
Companies

Formal
Legal
Agreement



Polycystic Kidney Disease Consortium

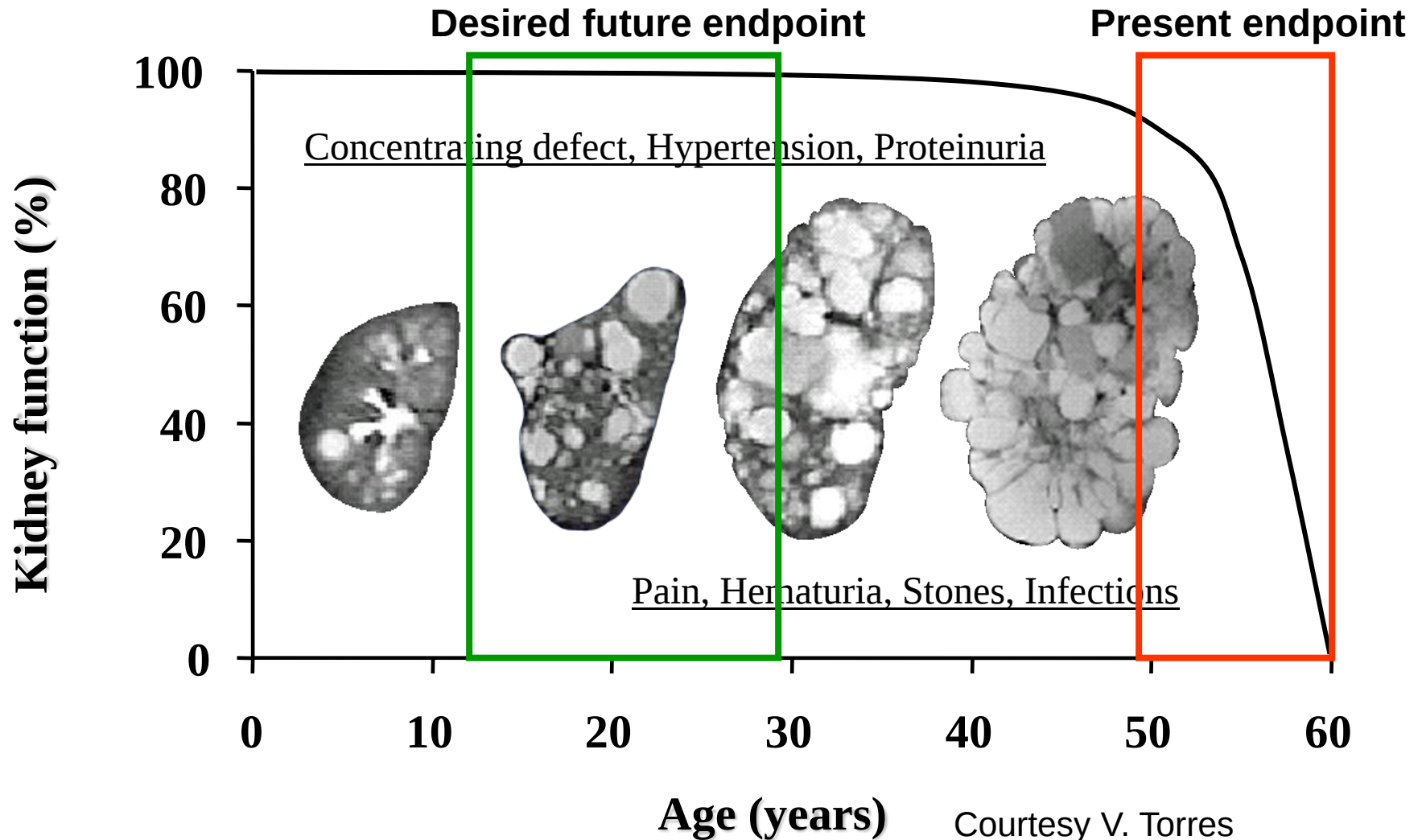


- To evaluate TKV as a biomarker to predict progression of ADPKD
- To qualify TKV as a biomarker for use as a clinical endpoint in clinical trials for adult patients with ADPKD
- To accelerate the pace of clinical research and introduction of new therapies

The Consortium is Sponsored by the PKD Foundation



Changing The Paradigm For Measuring Disease Progression



Critical Path to TB Drug Regimens Initiative



BILL & MELINDA
GATES foundation



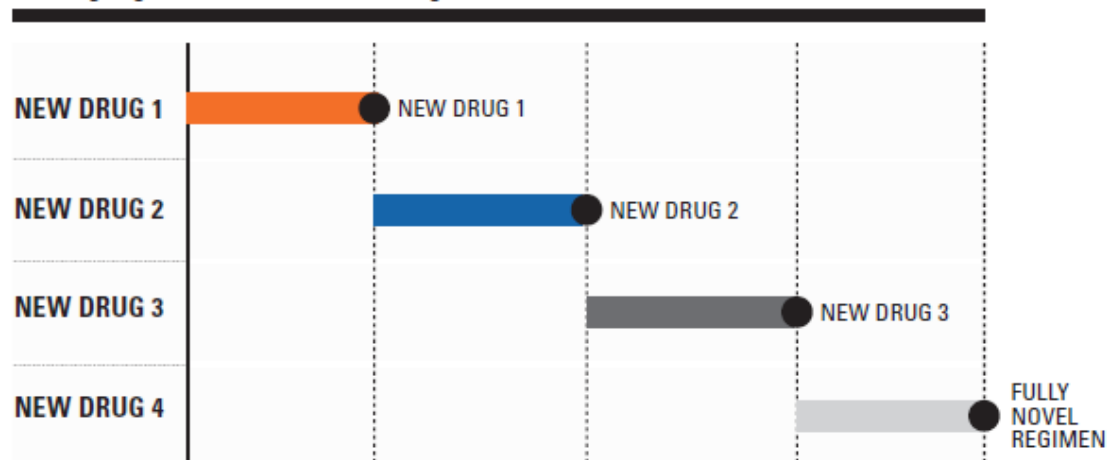
A collaboration to accelerate the development of new, safe, and highly effective regimens for TB by enabling early testing of drug combinations.



Critical Path to TB Drug Regimens Consortium

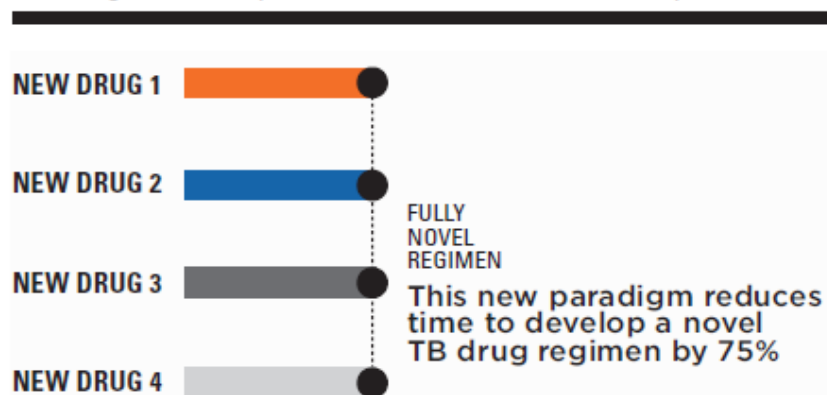
CURRENT REGIMEN DEVELOPMENT PARADIGM:

Existing regimen consists of four drugs



NOVEL COMBINATION TESTING PARADIGM:

Novel regimen development can reduce the time to develop a new TB drug regimen by 75%:



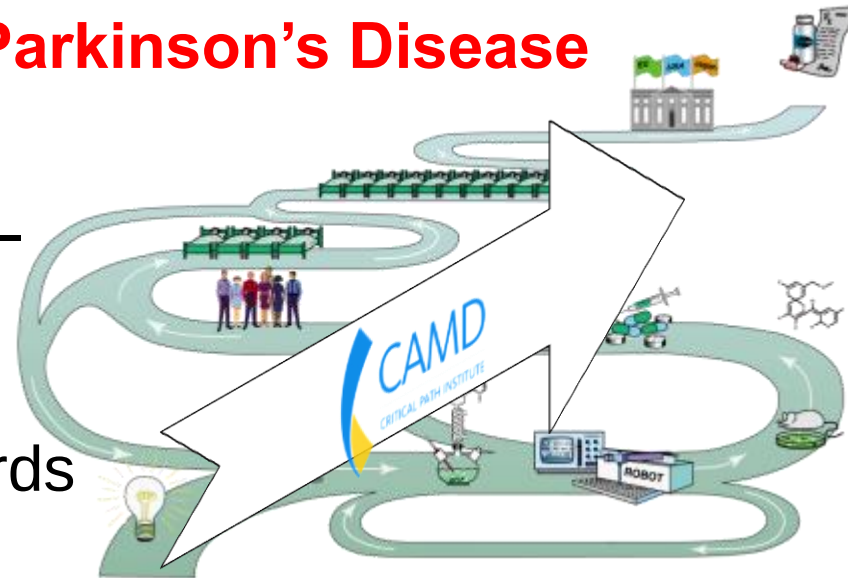
Coalition Against Major Diseases (CAMD)



Accelerate the Drug Discovery Path to Advance Effective Treatments for Alzheimer's and Parkinson's Disease

➤ Advance drug development – “Tools”

- **Develop** common data standards
- **Create** public databases of pooled clinical trial data
- **Qualify** biomarkers (FDA Draft Guidance 2010)
- **Develop** “Accepted for use” quantitative disease models



CAMD Members and Partners



Government/Regulatory participants



National Institute
on Aging ■ ♦ ★ ✱



Industry members



GE Healthcare



Non-profit research members

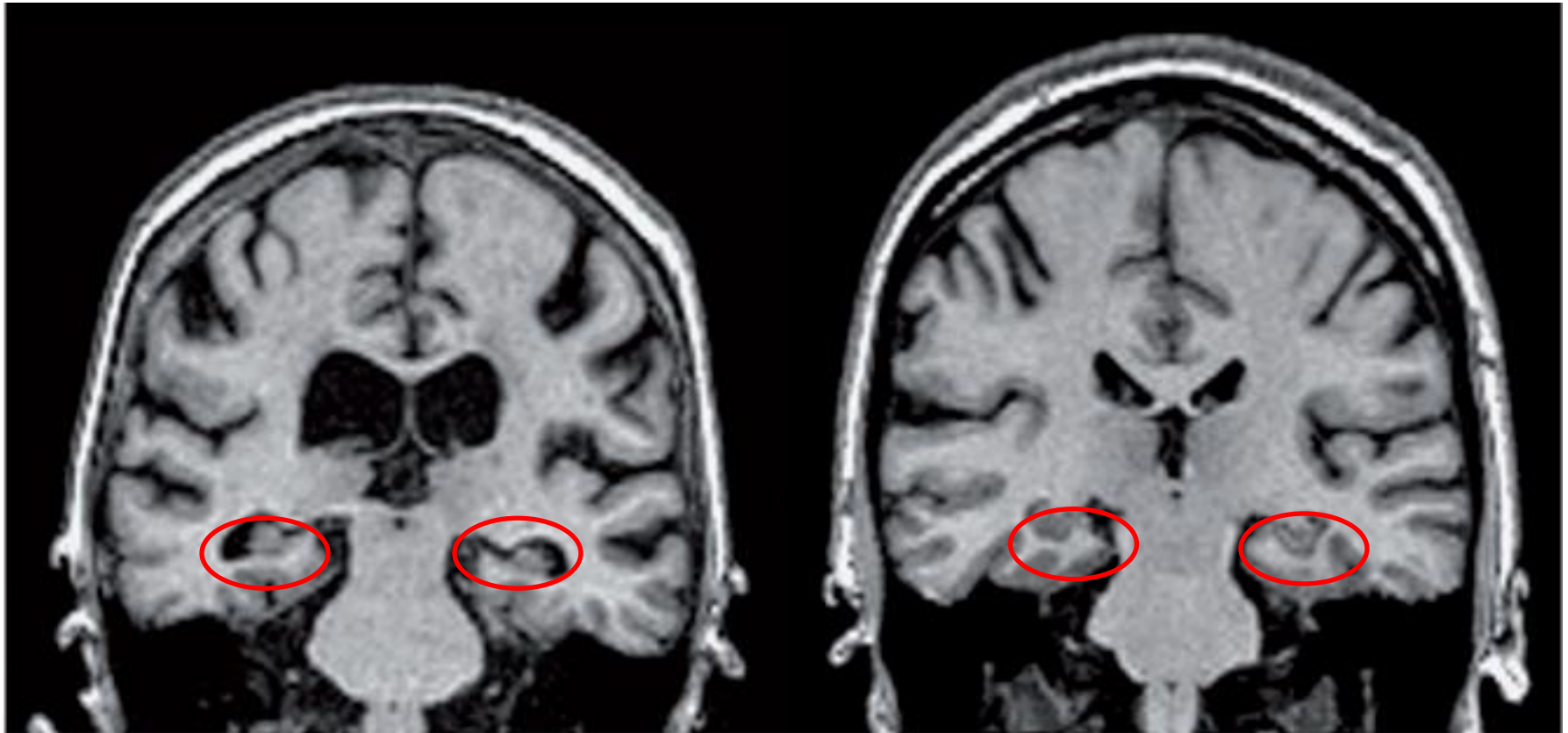


C-Path's Data Repository for Alzheimer's Disease



- **Seven companies remapped and pooled data from 21 trials for ~6000 patients: value = \$400 Million**
- **Database open to >200 qualified research teams in 35 countries**

Hippocampal Volume: Now Qualified by EMA



Mild Cognitive Impairment, progressor

Mild Cognitive Impairment, nonprogressor

From Jack et al, Brain 33:3336-48, 2010

MRI as a Drug Development Tool for Patient Enrichment



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

1 10 October 2011
2 EMA/CHMP/SAWP/809208/2011
3 Committee for Medicinal Products for Human Use (CHMP)

4 Qualification opinion of low hippocampal volume
5 (atrophy) by MRI for use in regulatory clinical trials - in
6 pre-dementia stage of Alzheimer's disease
7

Agreed by Scientific Advice Working Party	1 September 2011
Adoption by CHMP for release for consultation	22 September 2011
End of consultation (deadline for comments)	1 November 2011

Alzheimer's Disease Advocacy Groups Partner with CAMD



- Alliance for Aging Research/Accelerating Cure-Treatments for Alzheimer's
- Alzheimer's Foundation Association
- Alzheimer's Association
- National Health Council



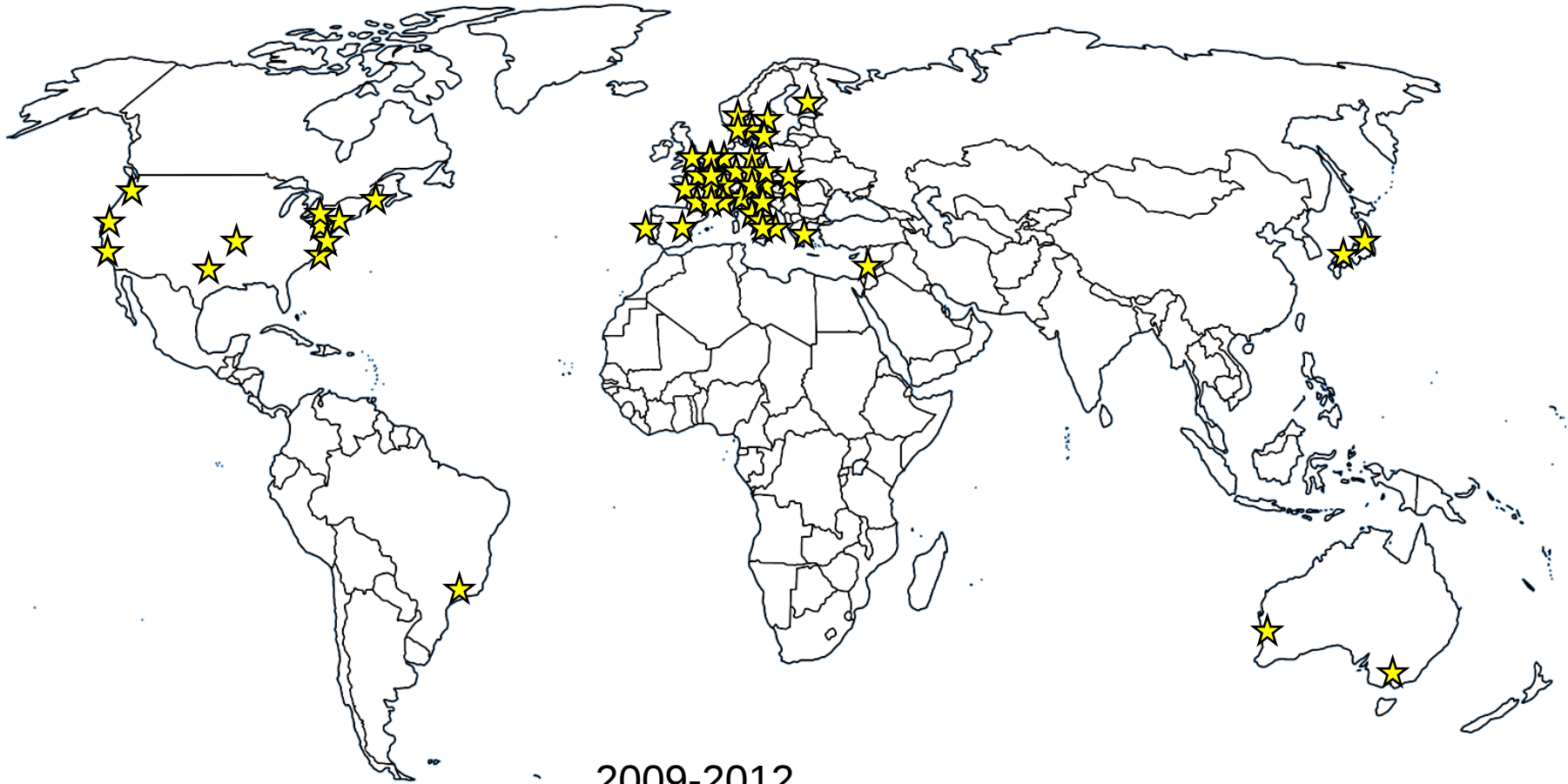
**National Alzheimer's Project Act (NAPA)
Plan Development**

alzheimer's  association®



**International Conference:
Research and
Standardization in
Alzheimer's disease
(RASAD)**

External Quality Control Program for CSF biomarkers



2009-2012

8 rounds of CSF samples (3 samples in each round)

79 laboratories

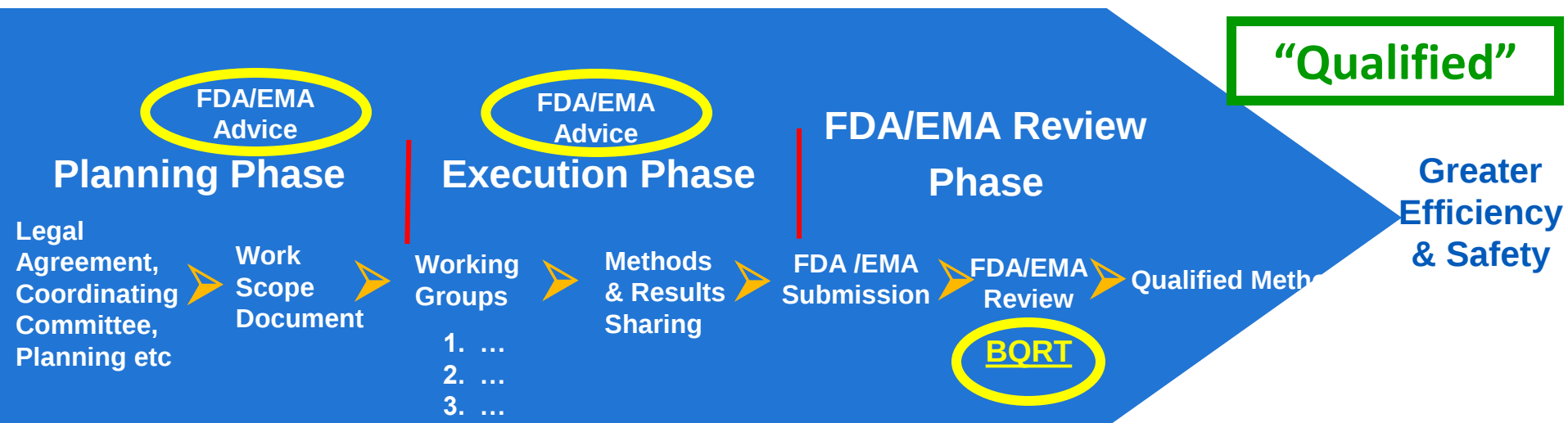
All C-Path Consortia Align with Regulatory Science



- Seven Safety biomarkers “Qualified” by FDA, EMA, and PMDA and being used by industry
- ~ 60 Biomarkers/PROs in process
- CDISC Data Standards set for Alzheimer’s disease (Parkinson’s, TB, and PKD in process)
- Database of 21 pooled industry trials opened for researchers (~6000 Alzheimer’s disease patients)
- Alzheimer’s: MRI qualified by EMA and disease progression model & CSF biomarkers in FDA review

Qualification of New Testing Methods

A new pathway.....



**Scientific
Consensus**



**FDA
EMA
PMDA**

CRITICAL PATH
INSTITUTE

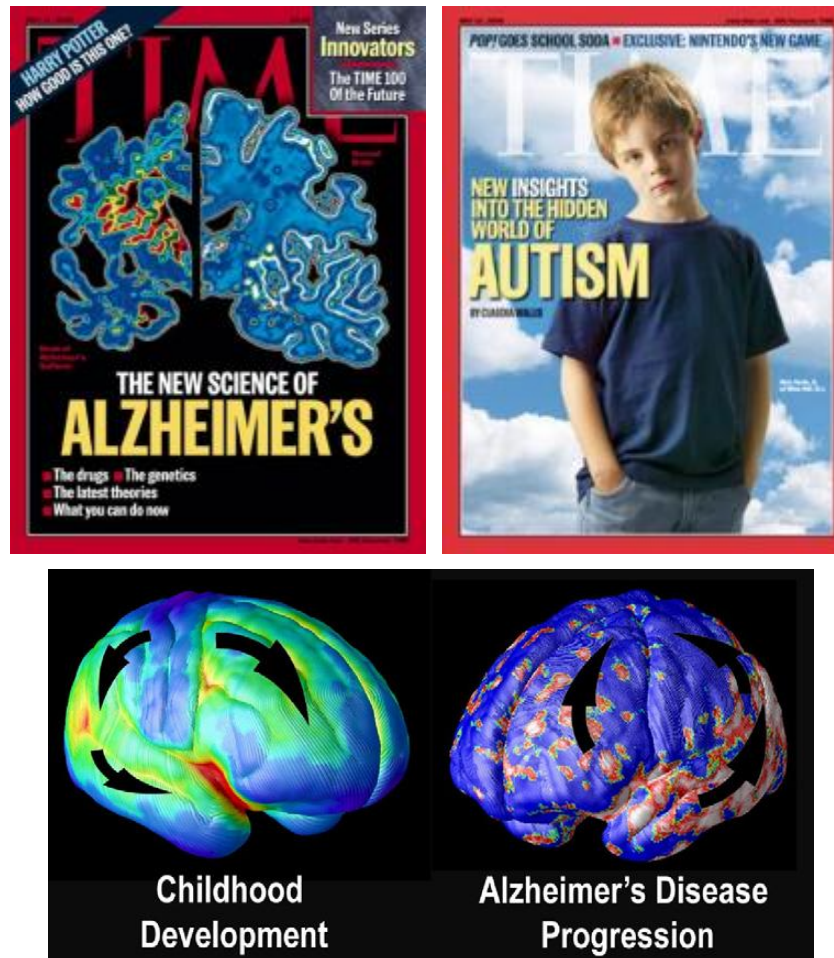
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Potential Solution: Create Unified Data Standards

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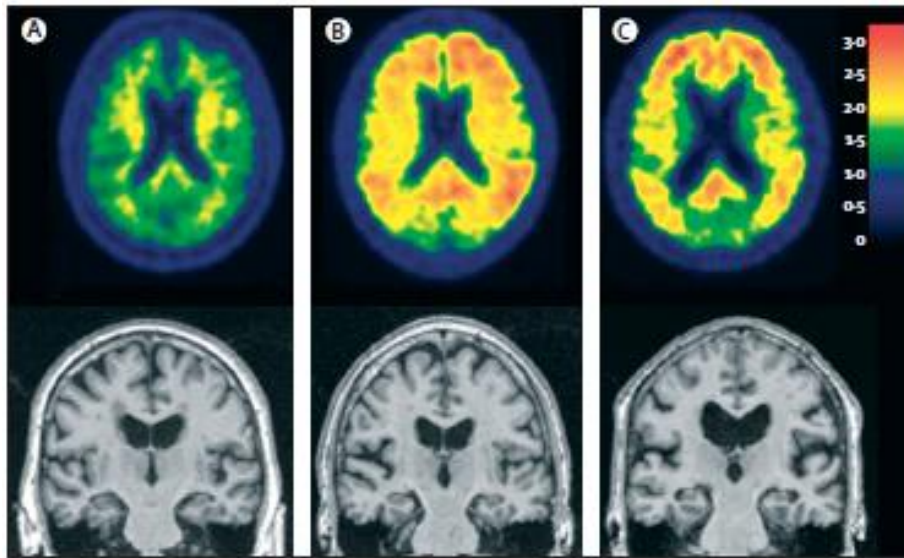
Impacting Diseases of High Unmet Medical Need

Create data standards and build database for two distinct disease conditions of high unmet need

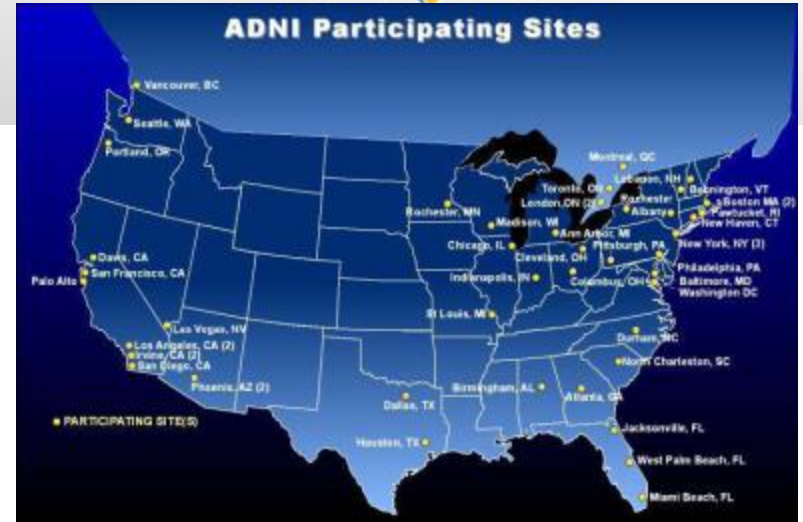


Ewers et al.,
Progress in Neurobiology
95 (2011) 525-536

The Impact of ADNI on Alzheimer's Disease



Biomarker staging of AD



- ADNI has provided seminal new information concerning the pathophysiology of Alzheimer's Disease
- Defined early detection methods for Identification of risk
- Improved treatment trials for assessing predictors and outcomes
- Accelerating a path leading to the treatment and prevention of AD

IMI Call: Translational Endpoints in Autism

AUTISMMEDS -1st & largest public-private partnership to advance autism research in Europe

Workpackages



Public Consortium

- Academia
- Small & Medium Enterprises
- Patient Organizations
- Non-profit research organizations
- Intergovernmental organizations



1 In vitro systems development

2 Animal model development

3 Translational research development

4 Clinical research development

5 Data handling, management and integration

6 Project management and communication

Industry Consortium



Focus is on enabling drug discovery

New Disease Areas Under Consideration for C-Path

- Multiple Sclerosis

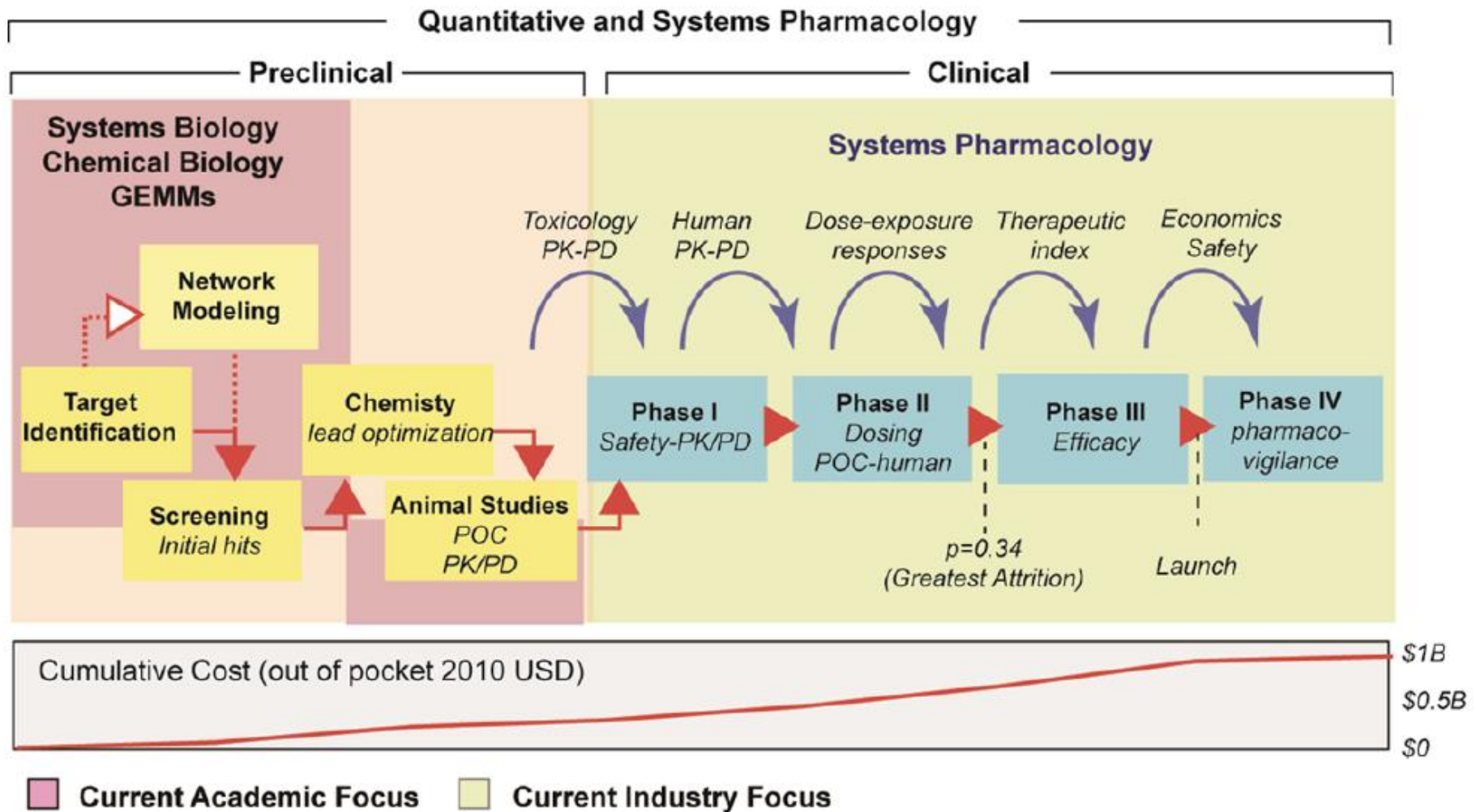


- Autism Spectrum Disorders



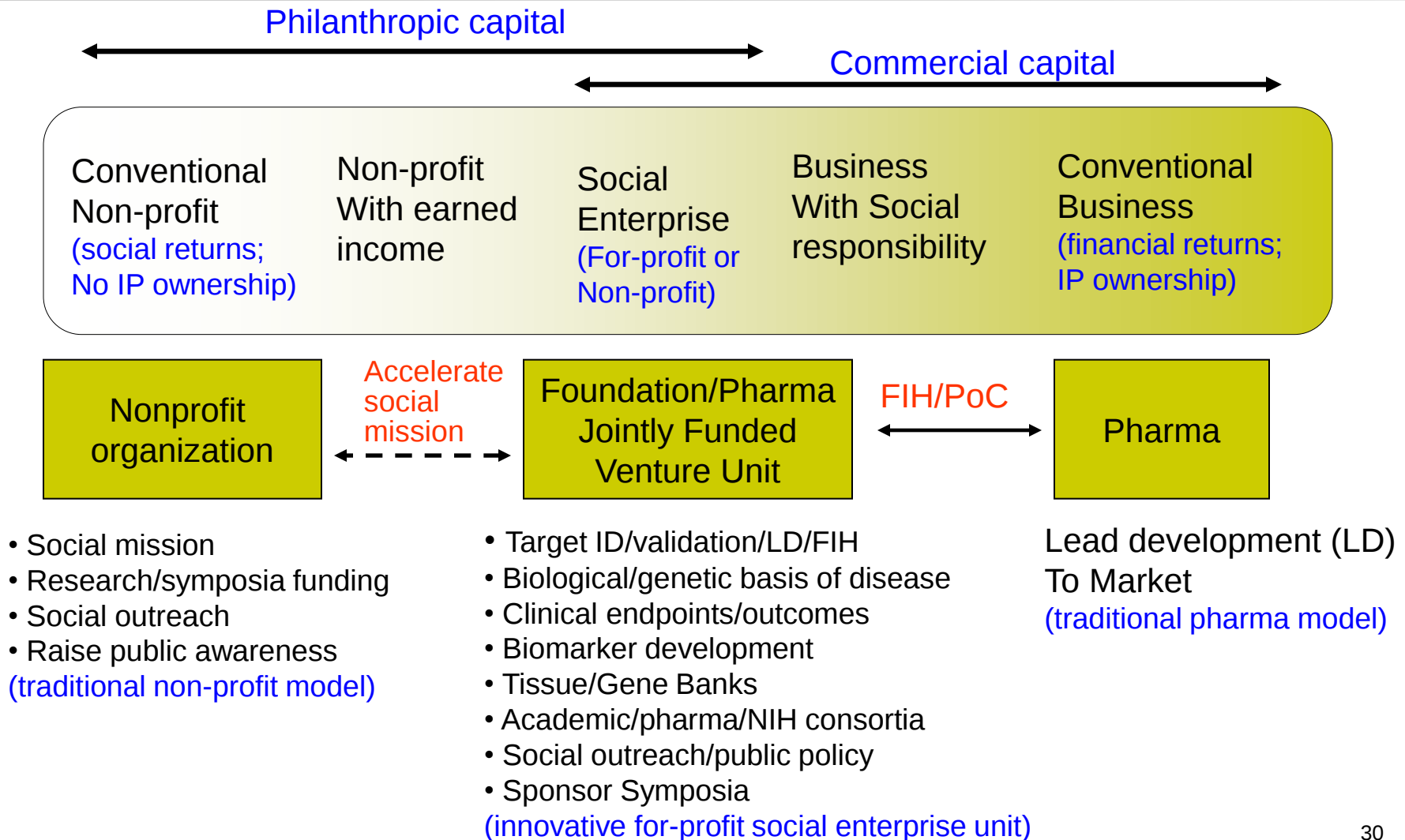
- Non AD Dementias (PSP, FTD)
- Oncology

Barriers Exist Between Academia and Industry in Drug Development



GEMM: Genetically Engineered Mouse Model; POC: Proof of concept

“Game Changing” : integrated human health social enterprise units



- Critical Path Institute has developed the infrastructure and the path to accelerating treatments for diseases of unmet need by:
 - 1) Developing disease specific data standards
 - 2) Developing unified clinical trial databases
 - 3) Advancing regulatory submissions by collaborating with the FDA to qualify drug development tools
- The combination of diverse stakeholders and close alliances with regulatory agencies is an effective strategy for sharing costs and risks.
- Patient advocacy groups catalyze progress, convey a sense of urgency contribute funds, and actively participate in consortia and regulatory science initiatives.