Data Aggregation Across Diseases and Between Stakeholders

- **Enrique Avilés, Chief Technology Officer**

CRITICAL PATH INSTITUTE
a decade of excellence

10 YEARS

Goals, structure, governance and expertise enable effective collaboration – C-Path consortia – 4Q 2015

CRITICAL PATH INSTITUTE

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 PD

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

Data Sharing – Key Success Factors

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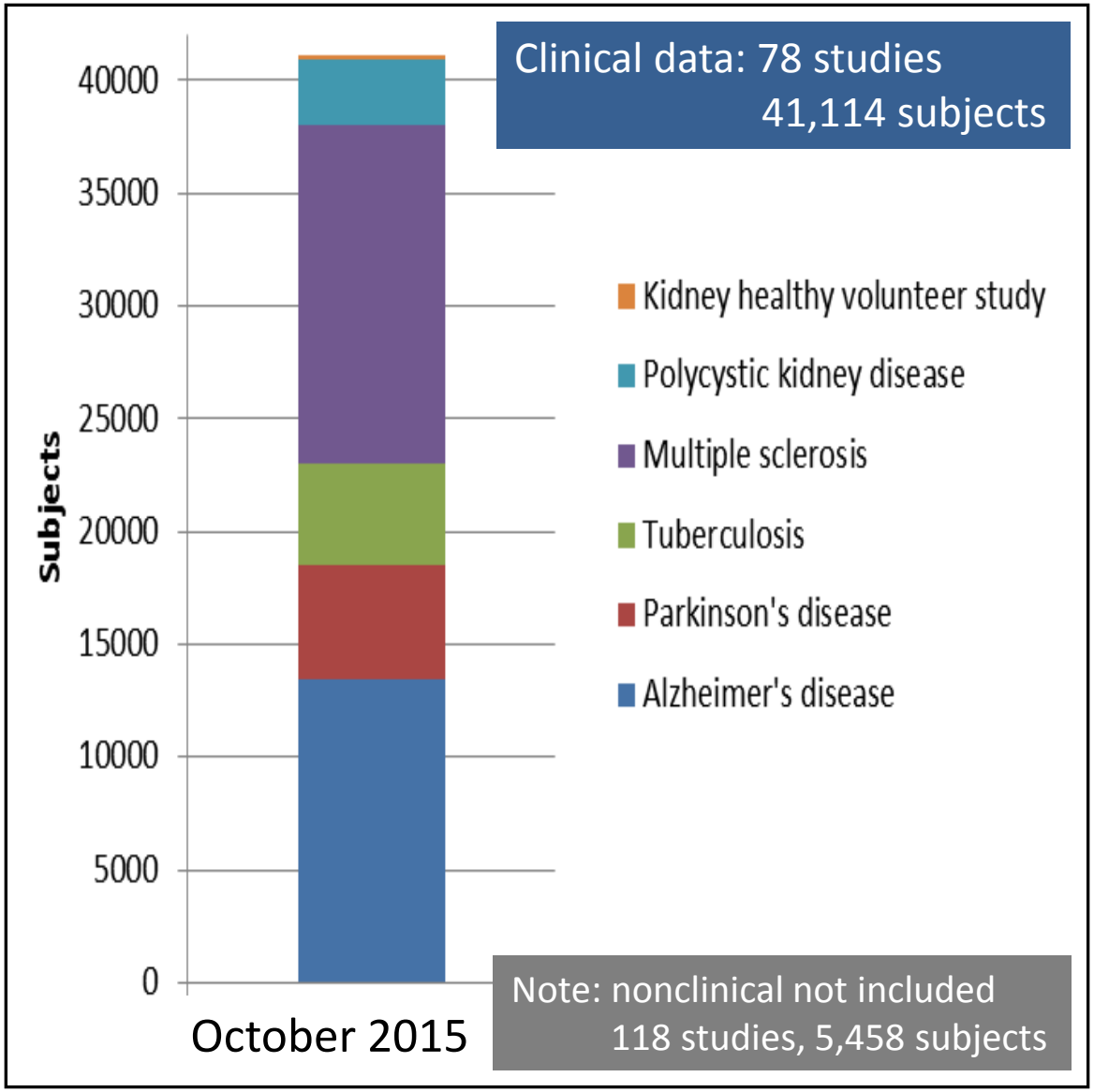
Factor	Key characteristics for success
Collaboration	• Specific goals and buy-in to value proposition • Structure • Governance

Expertise	• Scientific and medical subject matter experts
	• Full spectrum of supporting disciplines
	  • Data privacy preservation
	  • Data management and analysis
	  • Information technology
	  • Project management

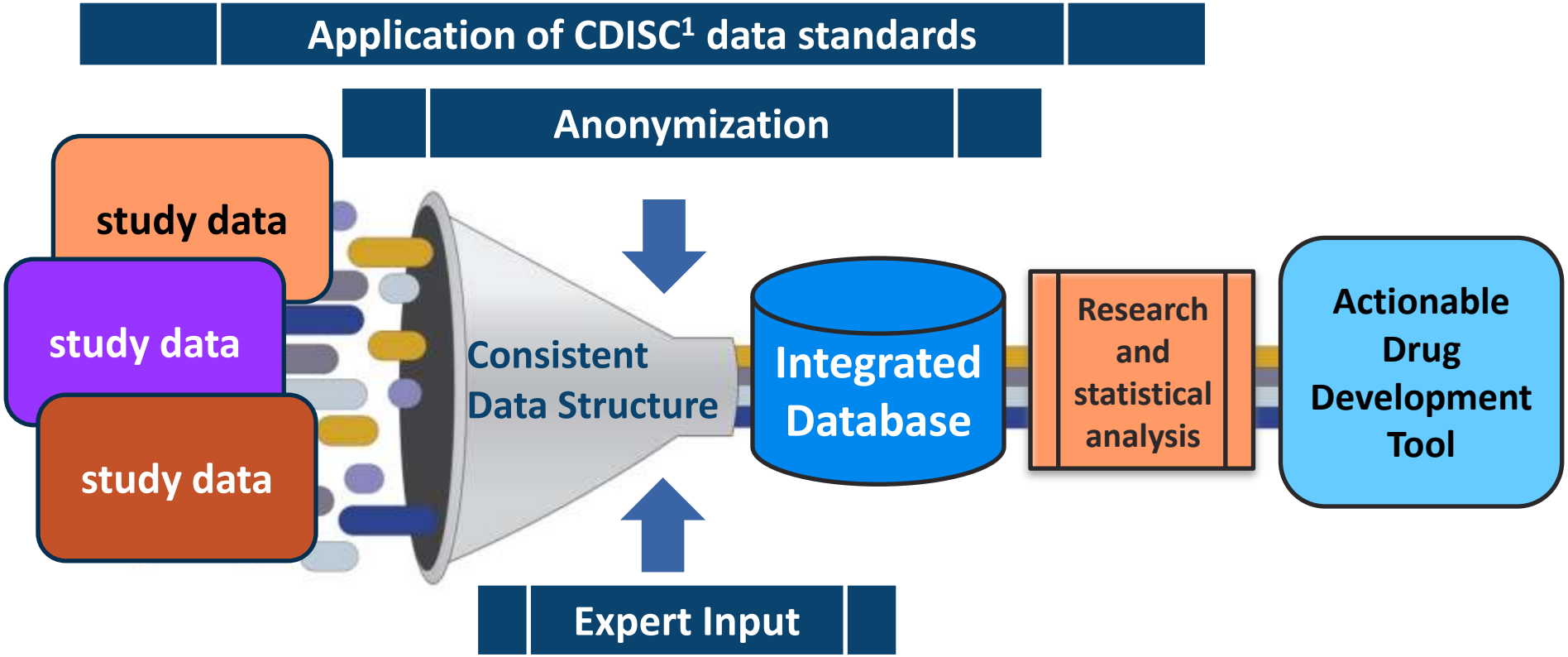
Data	• Data acquisition process = many conversations • Data use / data sharing agreements • Consistent data structure • Scientific validation of integration methods • Defined approach to optimize signal to noise ratio

Clinical data sharing - key considerations

- **Data contribution agreements implemented per dataset**
- **Level of allowable sharing defined in advance**
- **Data privacy assessment and anonymization must conform to all applicable regulations**



C-Path Data Aggregation Approach



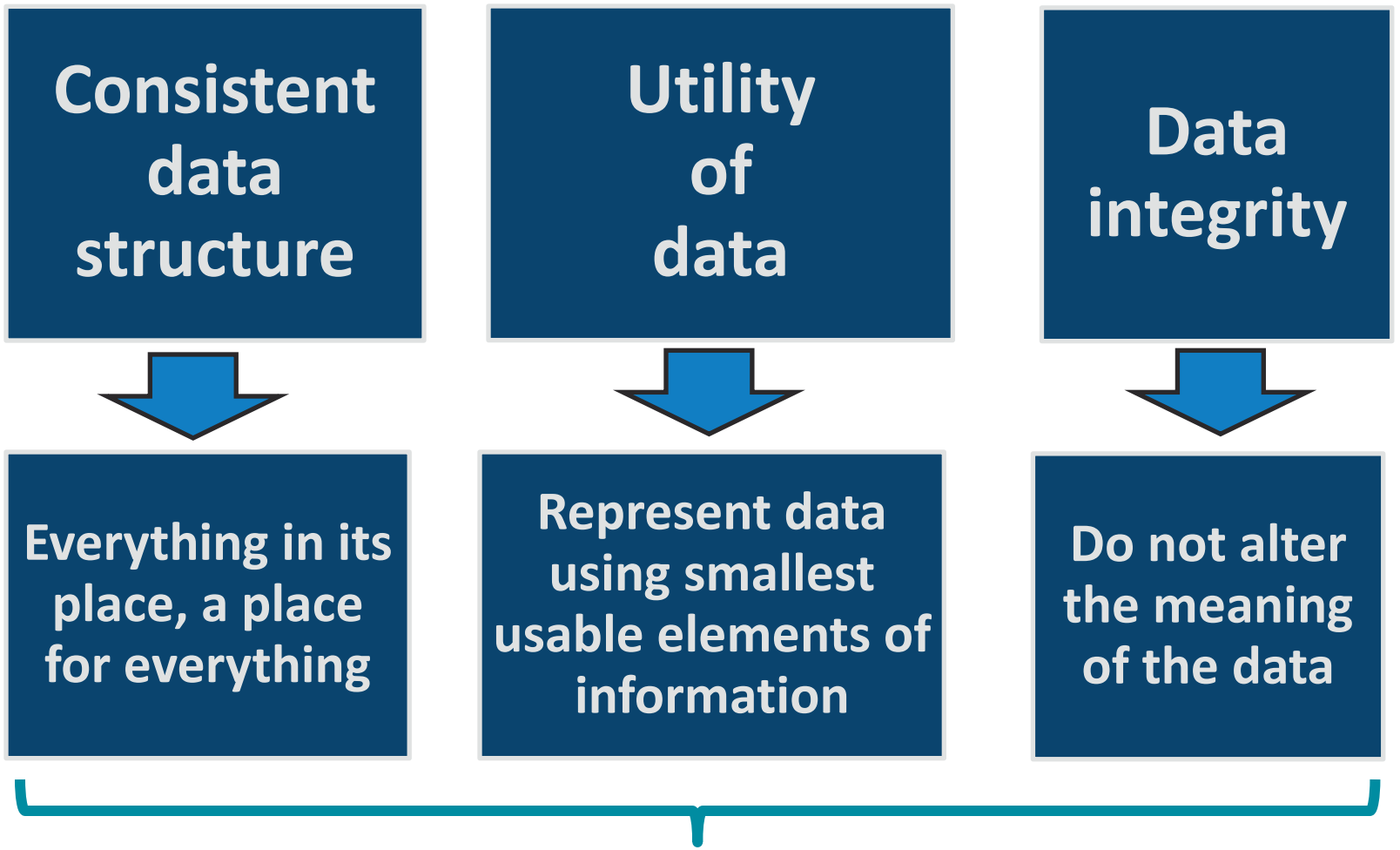
Data as contributed

Master standardized datasets

Analysis datasets

[1] CDISC: Clinical Data Interchange Standards Consortium, www.cdisc.org

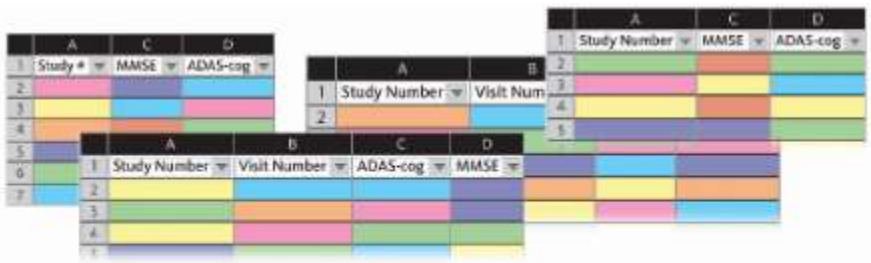
Data Aggregation – Key Objectives



CDISC[1] clinical data standards enabled this approach

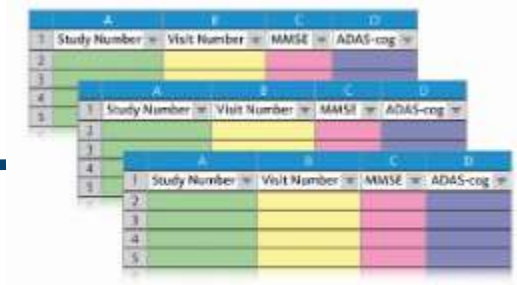
[1] CDISC: Clinical Data Interchange Standards Consortium, www.cdisc.org

Example: C-Path CAMD Alzheimer's Disease Modeling & Simulation Tool



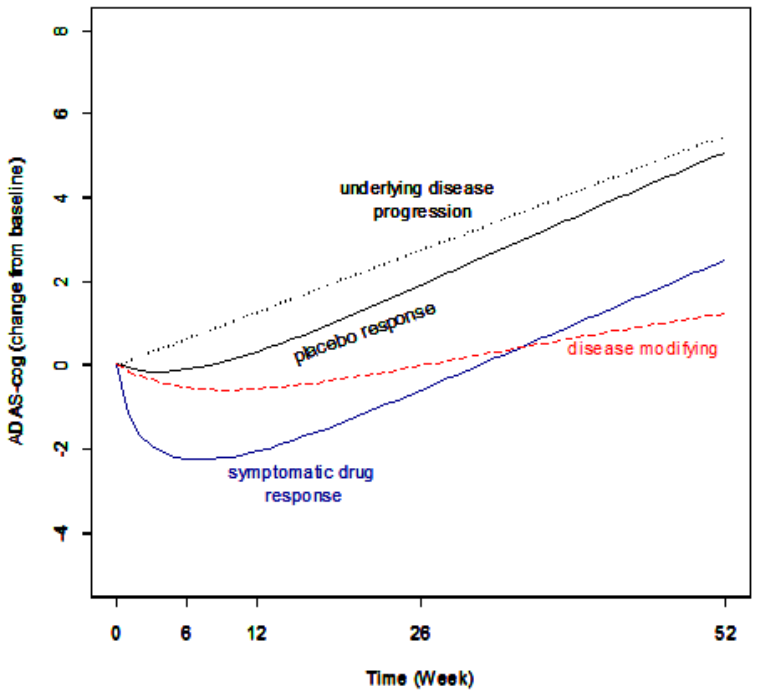
Mixed Legacy Data

Data Standards



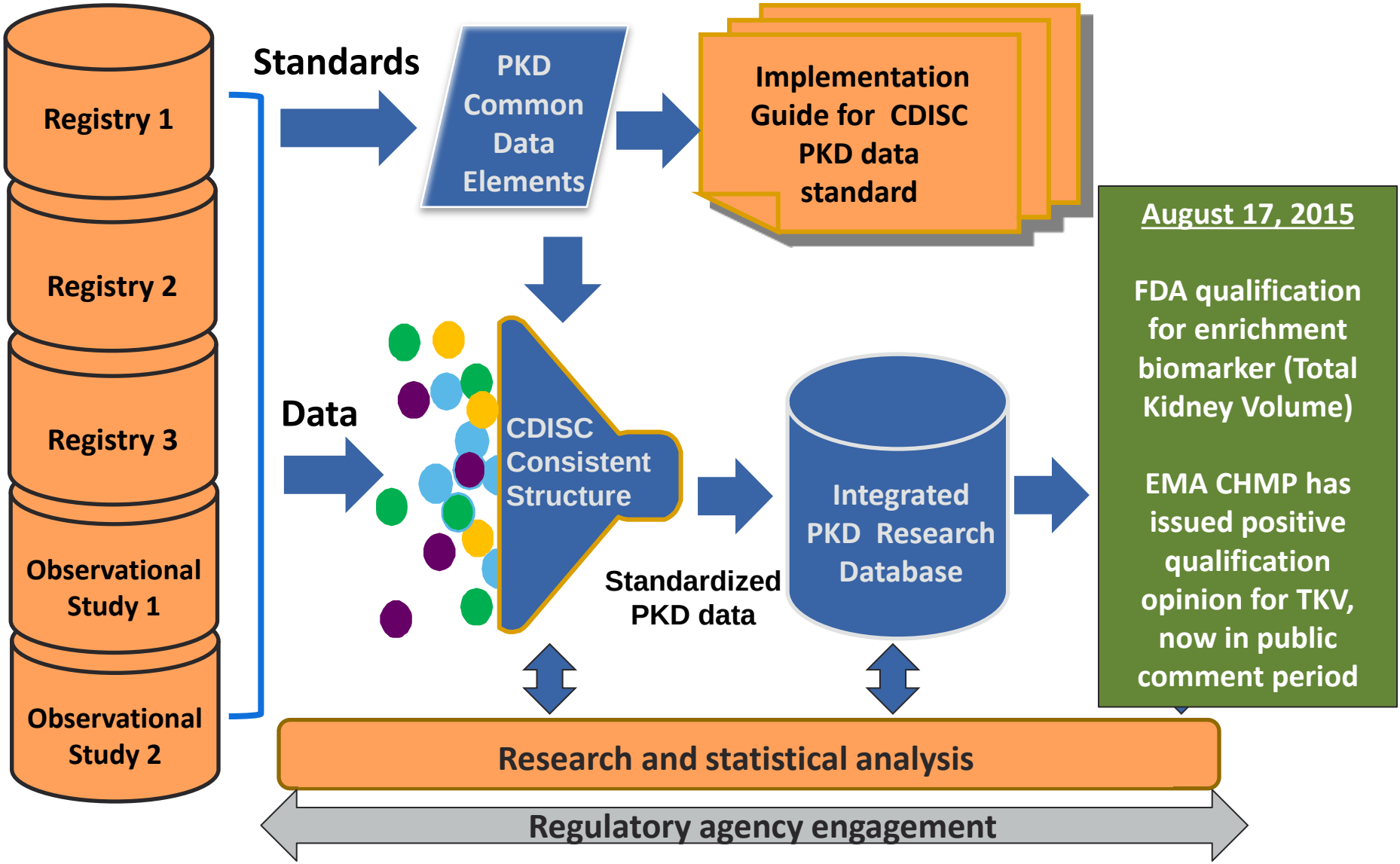
Integrated Data

Deterioration

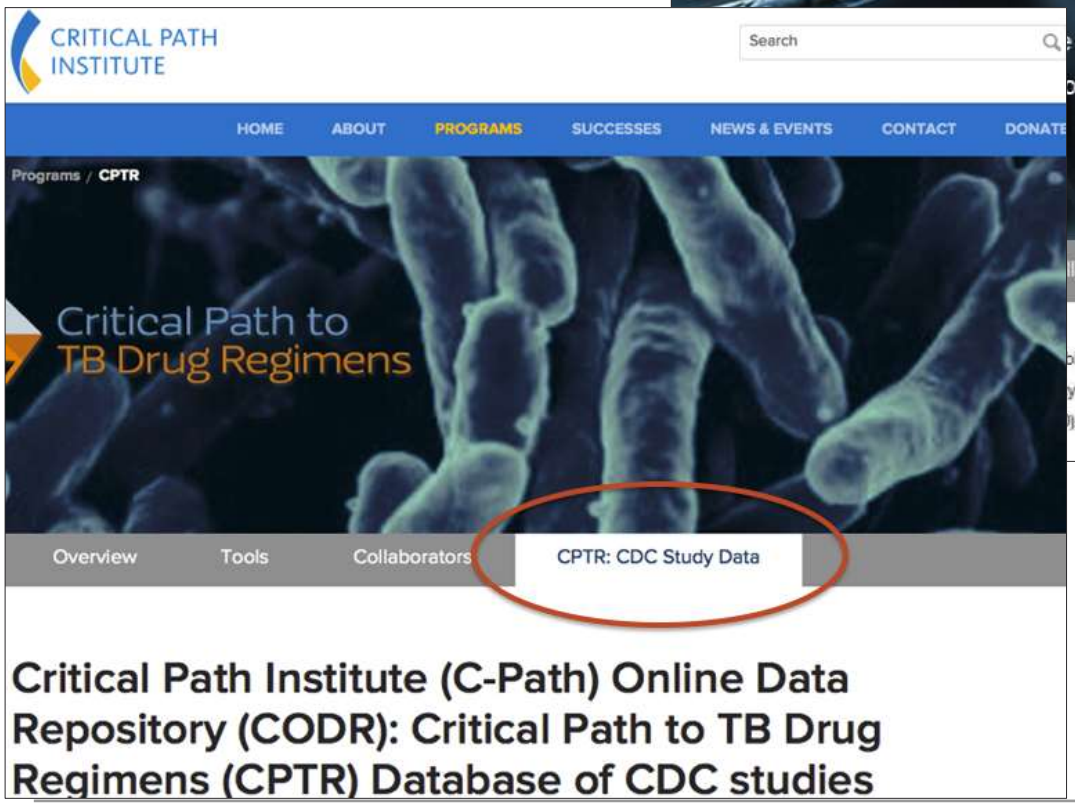


Rogers et al., J Pharmacokinetics Pharmacodyn. 2012 Oct;39(5):479-98

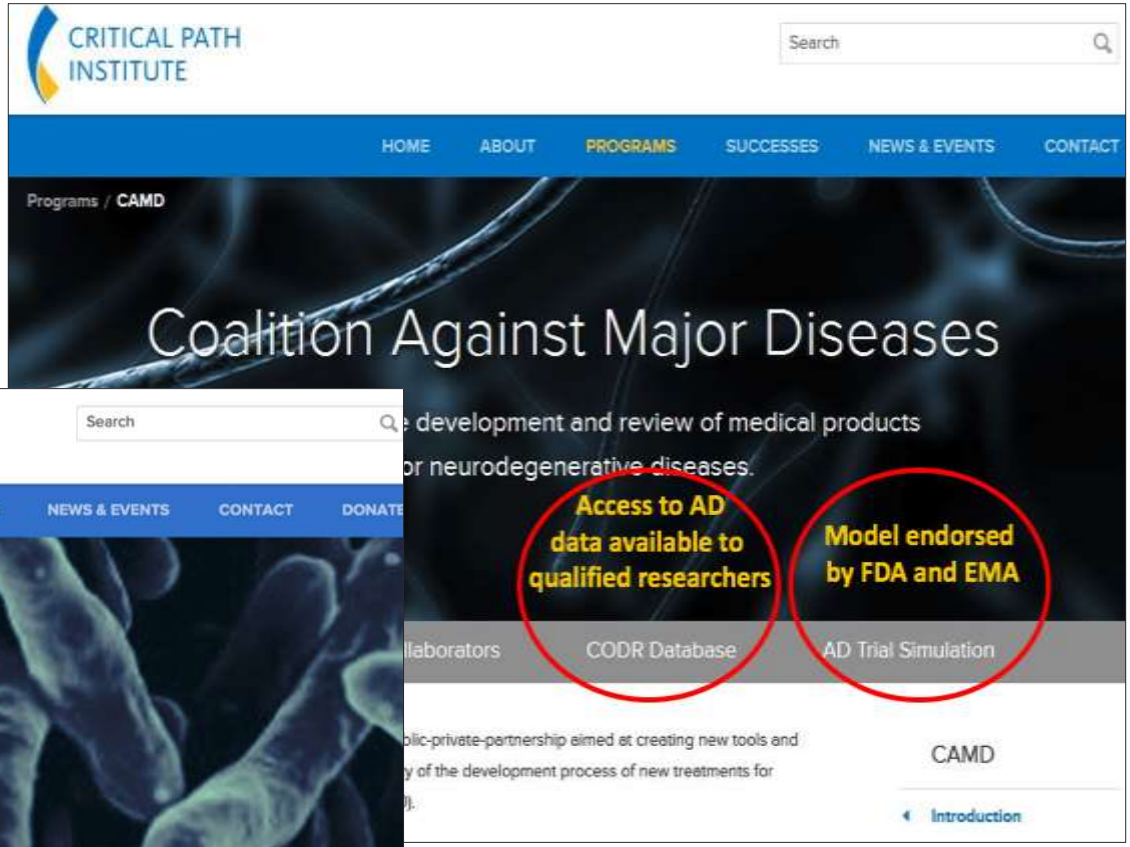
Polycystic Kidney Disease (PKD) Project



Databases Accessible to Research Community



Critical Path Institute (C-Path) Online Data Repository (CODR): Critical Path to TB Drug Regimens (CPTR) Database of CDC studies



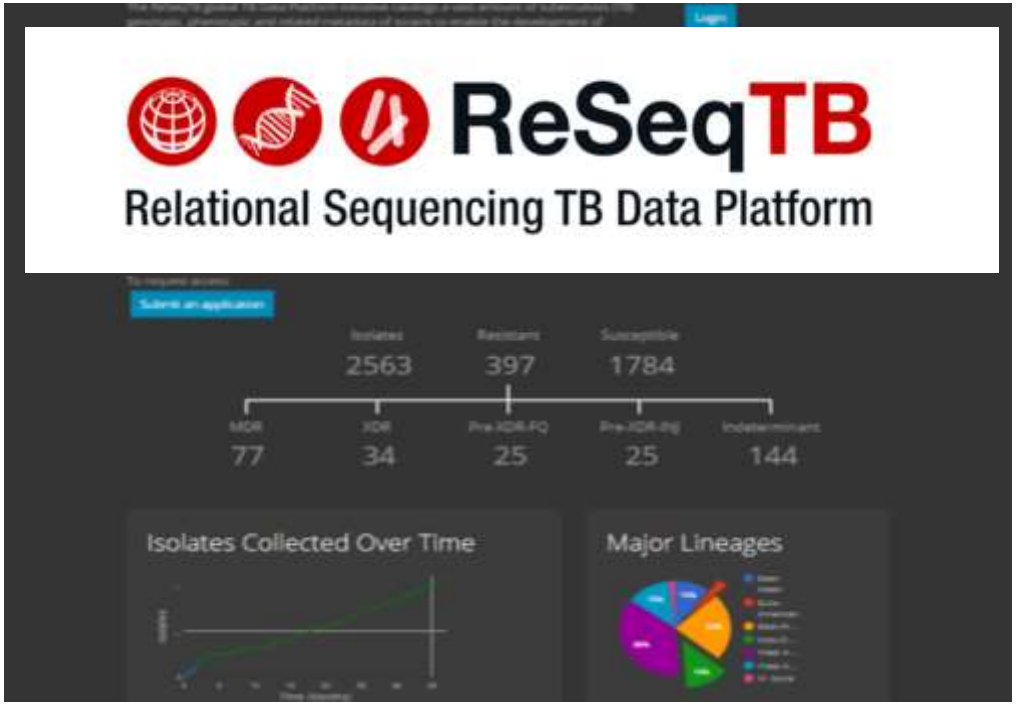
MS data sharing in future plans

Clinical Data Aggregation – Project Examples

- ✓ Alzheimer's clinical trial simulation tool - regulatory endorsement
- ✓ Polycystic kidney disease biomarker – regulatory endorsement
- ✓ Data sharing to enable research beyond consortium objectives (Alzheimer's, Tuberculosis, …)

- New data sharing project encompassing tuberculosis genotypic and phenotypic data, surveillance study data, and clinical trial data – launching 4Q2015

https://www.genomeweb.com/informatics/consortium-seeks-provide-centralized-access-global-tb-data-research-dx-development



Clinical Data Integration – key focus areas

- Tracking changes in privacy regulations and alignment where possible to aid data sharing for research
- Ease of use and interoperability for data standards systems in use for research and clinical care – even more critical with the emergence of digital biomarkers
- Development of incentives to encourage more rapid and efficient sharing of data from academic research efforts
- Highlight the added value that can be gained from re-use of data beyond initial use cases, optimizations for sharing of prospective data, and enrichment of insights via integration

Thank You

www.c-path.org

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