Molecular TB DST
Standardization Meeting:
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The CPTR Initiative: Mission and Objectives

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Critical Path to
TB Drug Regimens

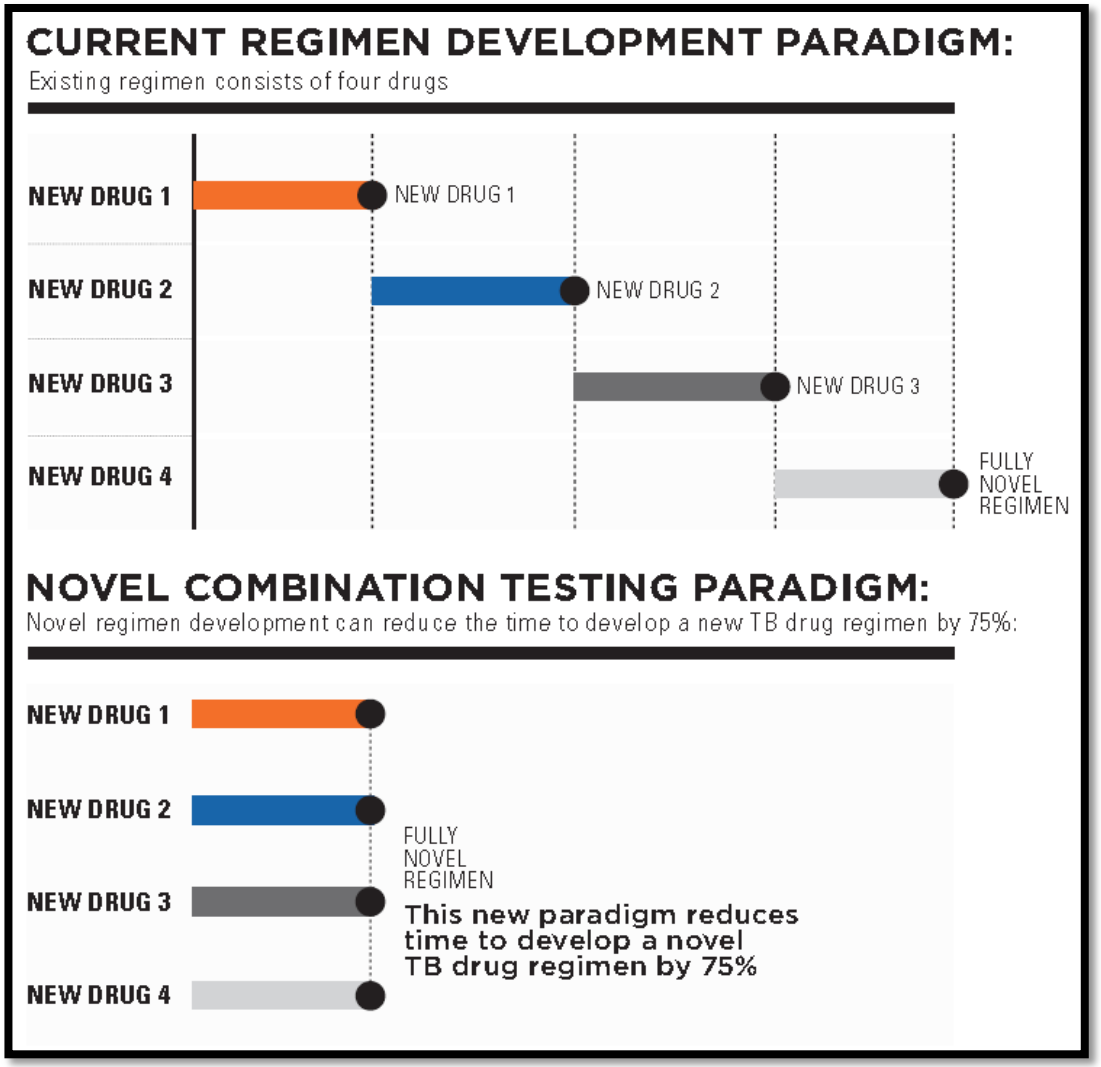
CPTR Mission & Purpose

CPTR

Accelerate the development of new, safe, and highly effective regimens for TB by enabling early testing of drug combinations.

The Challenge

- Need emphasis on combination study approaches rather than development of single agents
- Increasingly “fragile” TB drug development pipeline with the continued divestment of companies in the Anti-infective space
- Emerging need to accelerate the development of a **clinically** useful, **WHO-qualified**, regulatory approvable IVD assay for rapid TB-DST



CPTR Charter Has Expanded

CPTR

Accelerate the development of a regulatory approvable in vitro diagnostic assay for rapid drug susceptibility testing of TB to facilitate drug development and rational use of new drug regimens.

CRITICAL PATH INSTITUTE

BILL & MELINDA GATES foundation

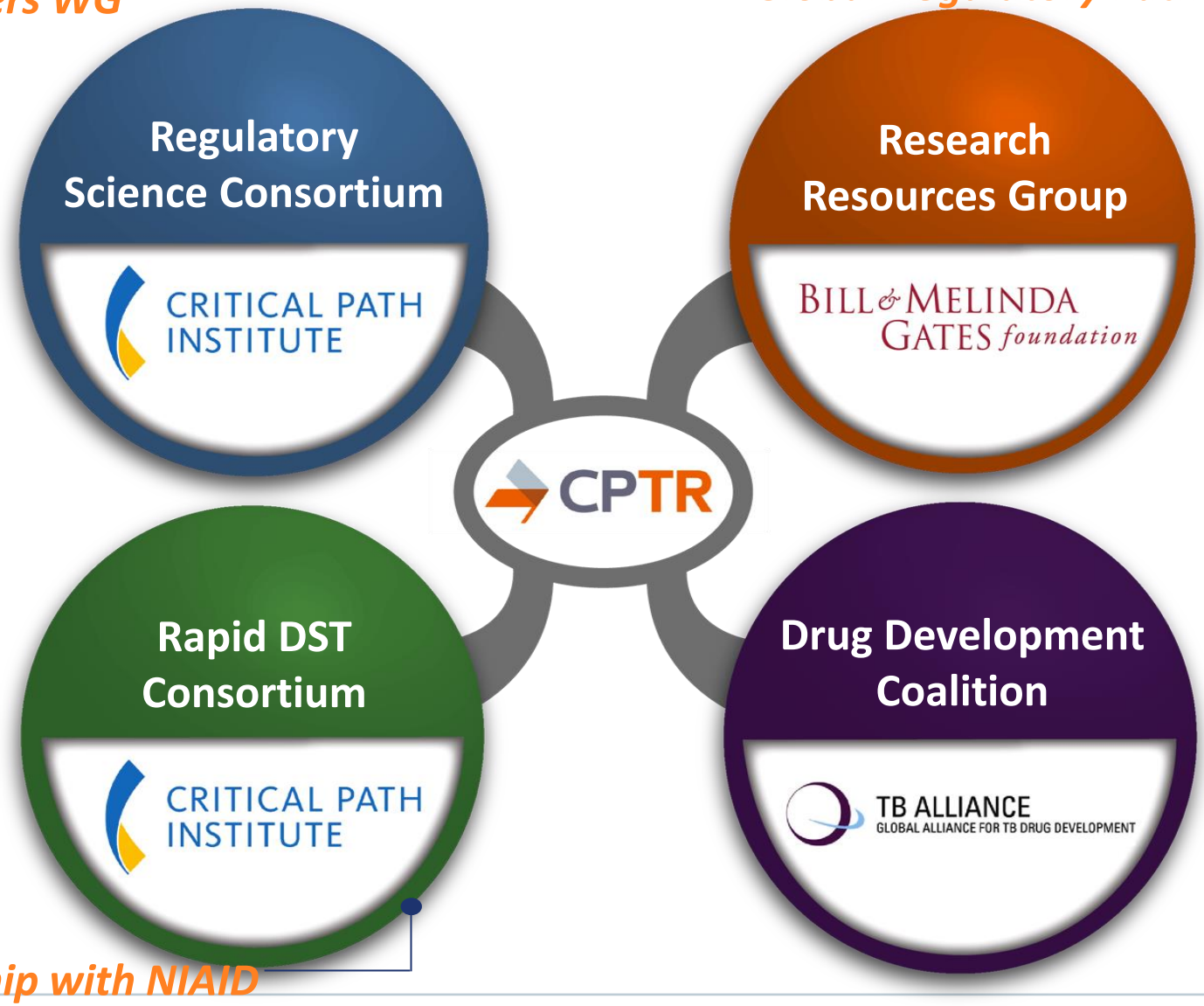
In Partnership with NIAID

TB ALLIANCE
GLOBAL ALLIANCE FOR TB DRUG DEVELOPMENT

CPTR Organizational Structure

Biomarkers WG

Global Regulatory Pathways WG

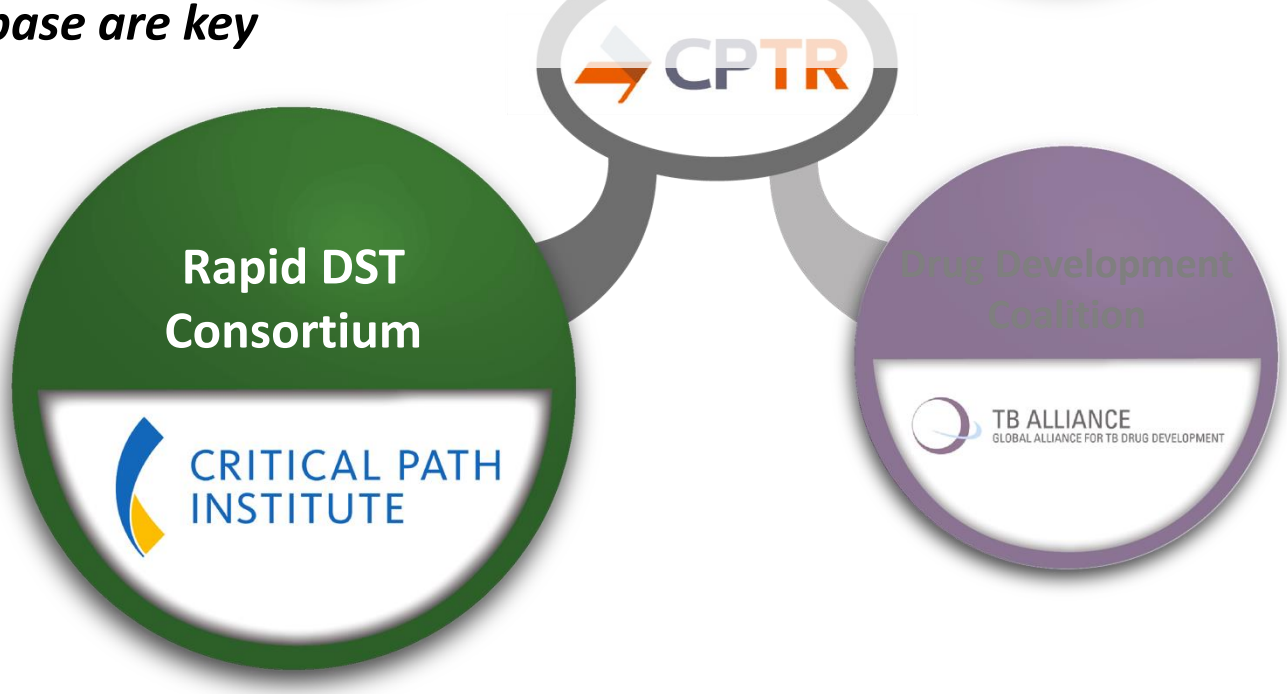


In Partnership with NIAID

Rapid DST Consortium

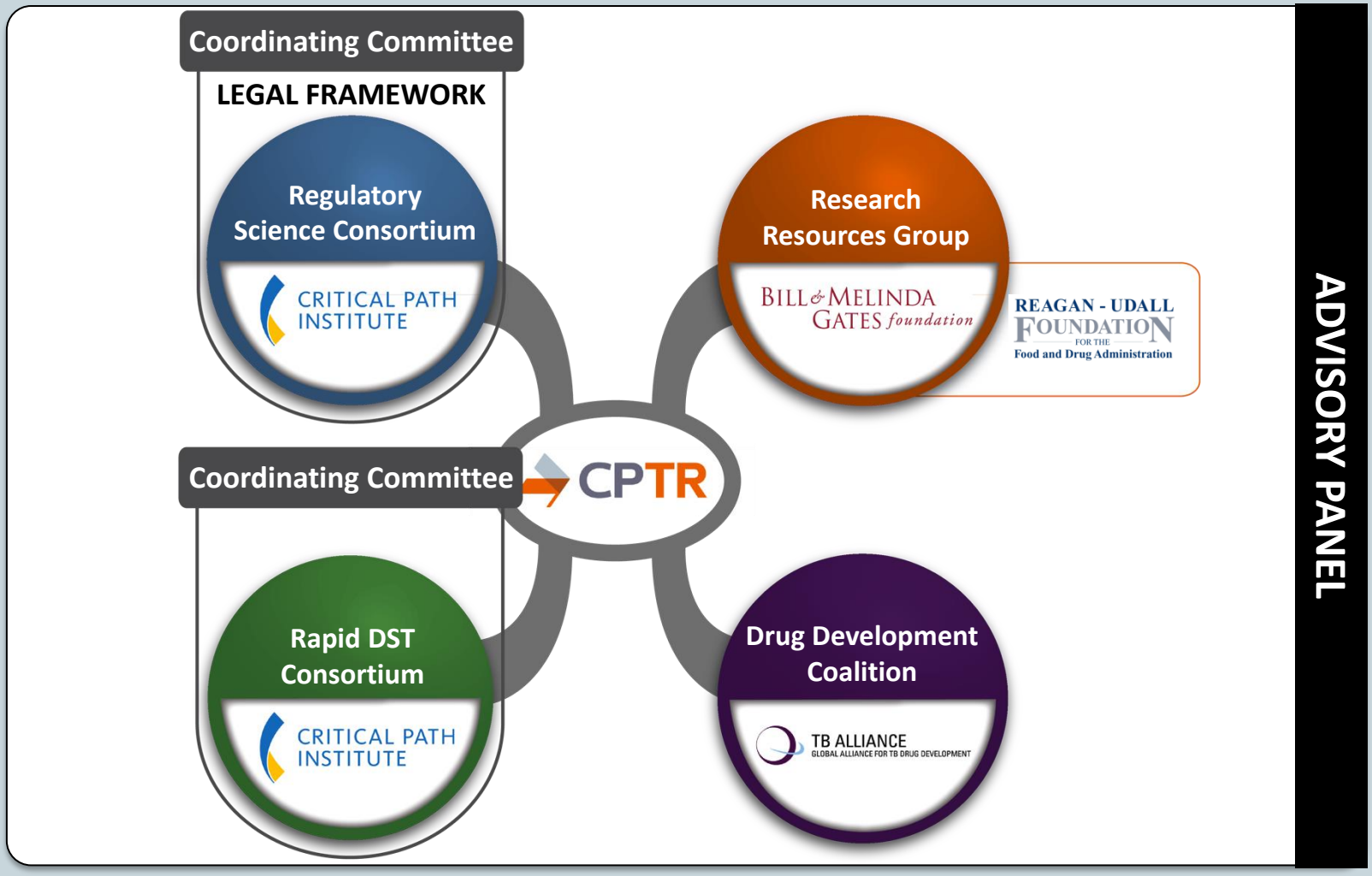
Role in Enabling & Accelerating the Process:

- Support the development of iterative Target Product Profiles (TPP's)
- Increase understanding of resistance trends to support rapid DST development
- Develop models that inform TPP's and policies for use of DST
- Facilitate development of rapid DST assays that could be commercialized
- Reach scientific consensus by sharing expertise, information, ***data and database are key***



CPTR Governance

CPTR Working Group



Consortia “Principles”

- CPTR is a Global Consortium designed to meet the TB Global Health need

- Designed with the intent to align TB Drug Developers, Clinicians, Academicians, Government Agencies (etc.) with DST developers to ensure specifications for programs meet the needs of this community

- Purposeful alignment with CPTR Global Regulatory Pathways team consisting of:
  - ✓ Regulatory experts from Industry
  - ✓ WHO
  - ✓ National Regulatory Agencies in countries of high burden
  - ✓ FDA/EMEA

Confidentiality Basic Principles

- Each Member is obligated to maintain confidentiality of each other Member's Confidential Information
- Member Confidential Information to be used solely for authorized Consortium purposes
- No requirement that a Member contribute any *particular* Confidential Information belonging to such Member
- HOWEVER it is each Member's *duty to share* Confidential Information relevant to Consortium activities
- Ultimate goal of Consortium is broad public dissemination of Tools

Supporting Database Rationale

Expansion and evolution of CPTR Clinical trial database to support Rapid Diagnostic Consortium to enable:

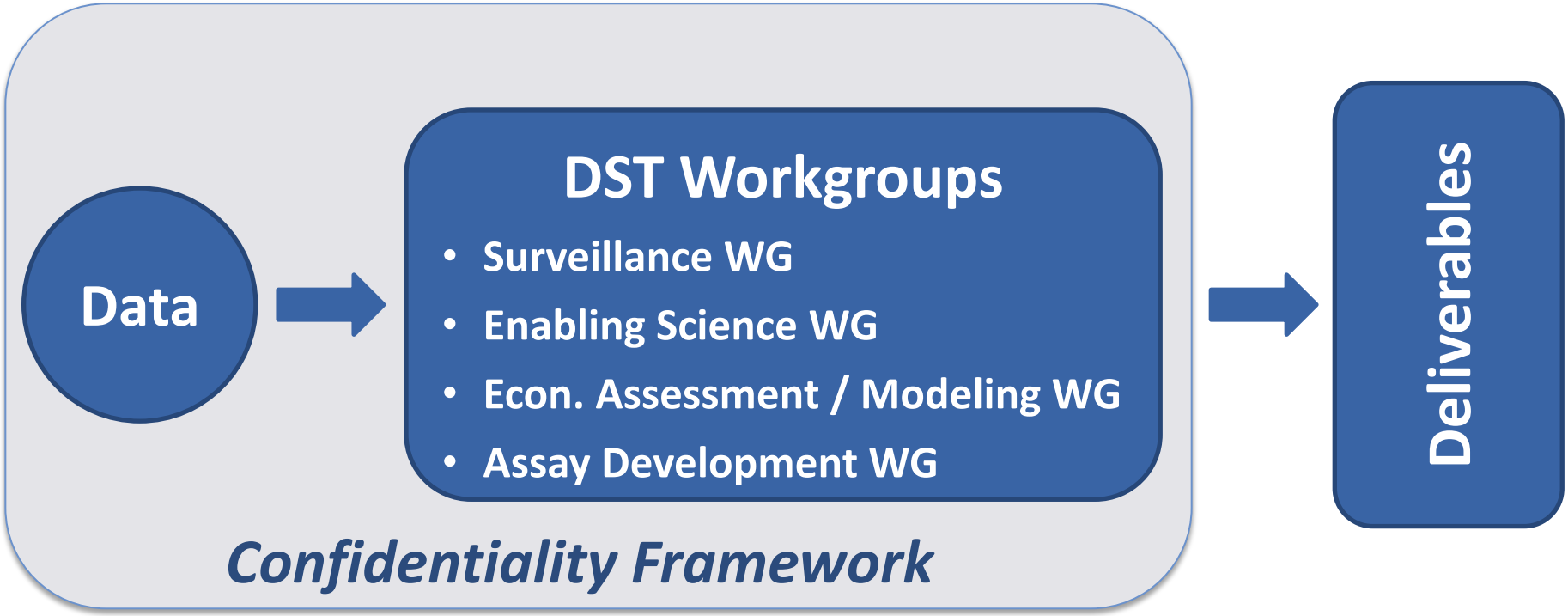
The development of Rapid DST's for existing and novel TB Drug Regimens

- ✓ Research and development
- ✓ Surveillance
- ✓ Resistance data to inform public health and clinical trials
- ✓ Biomarkers
- ✓ Clinical Use

Meet Health Authority Standards to support validation of approved tools and assays.

- ✓ Compliant with health authority requirements for IVD claims

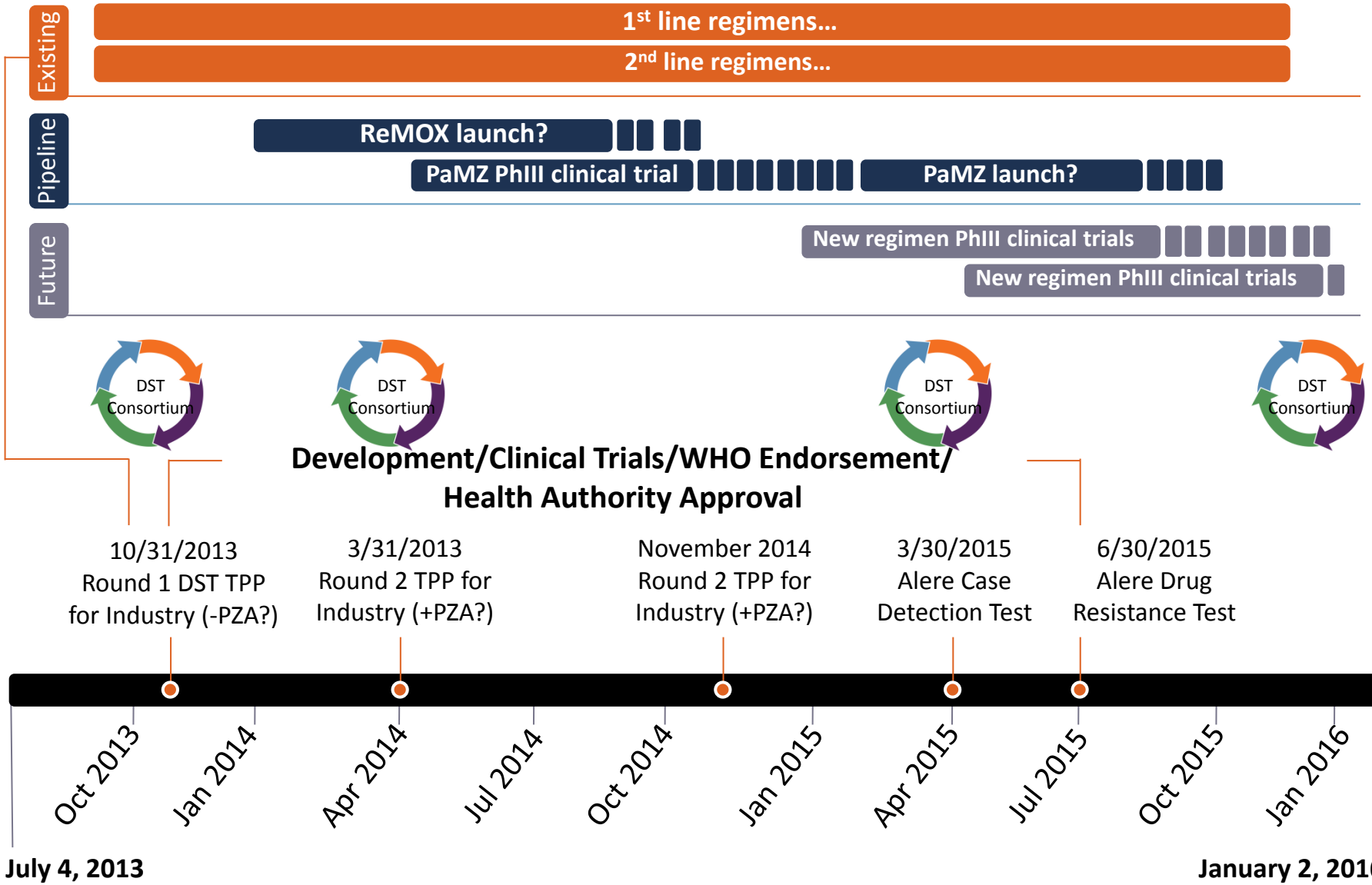
Rapid DST Consortium Framework



Rapid DST Consortium WG's

Work Group	Focus and Purpose
Enabling Science WG	Focus on the discovery and validation of the molecular basis for resistance and correlation of resistance with clinical outcome.
Assay Development WG	Facilitate developing Rapid TB DST assays to meet target product profiles & coordinating with industry to develop commercial in vitro diagnostic assays for TB DST, meeting target product profiles.
Surveillance WG	Increase understanding of the magnitude of drug resistance and trends for resistance in high burden countries and the global geography of drug resistance to inform clinical trial planning and clinical practice. Development of more cost effective strategies for surveillance of TB drug resistance.
Economic Assessment/ Impact Modeling WG	Develop models that will inform decisions needed to develop target product profiles and to inform policy about use of DST with anticipated TB drug regimens.

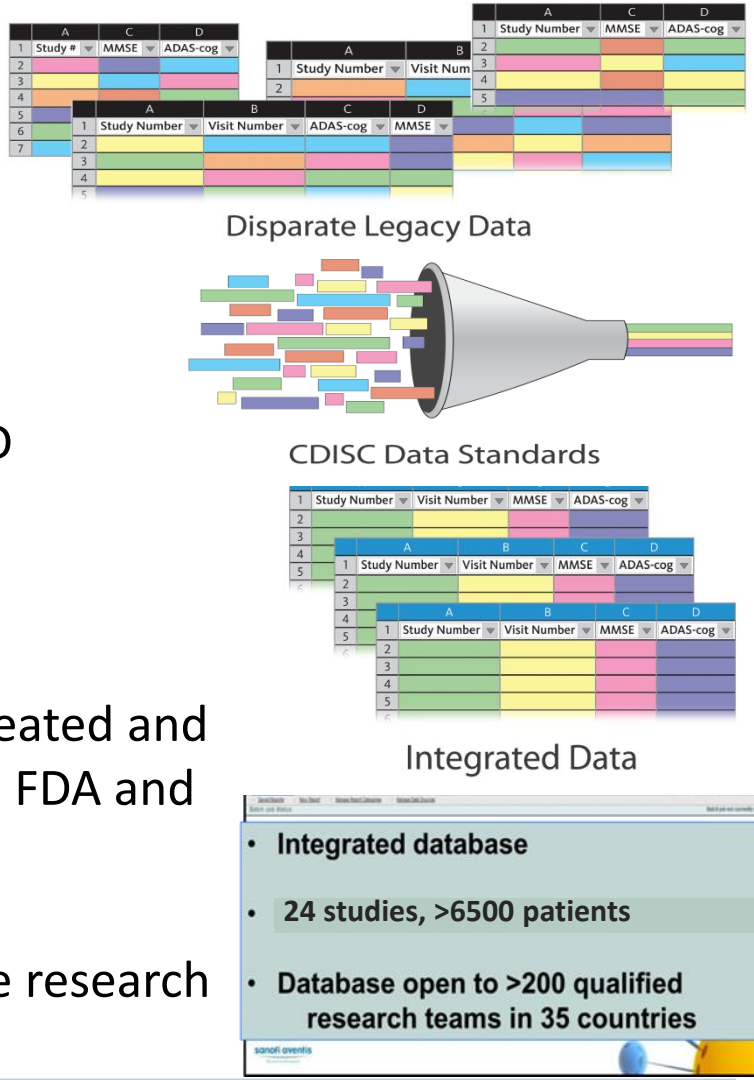
DST TPPs Will Be Iterative



Value of Data Sharing, Standards & Pooling

Start Point

- Nine member companies agreed to share data from 24 Alzheimer's disease (AD) trials
- The data were not in a common format
- The data were remapped to the CDISC AD standard and pooled



Result

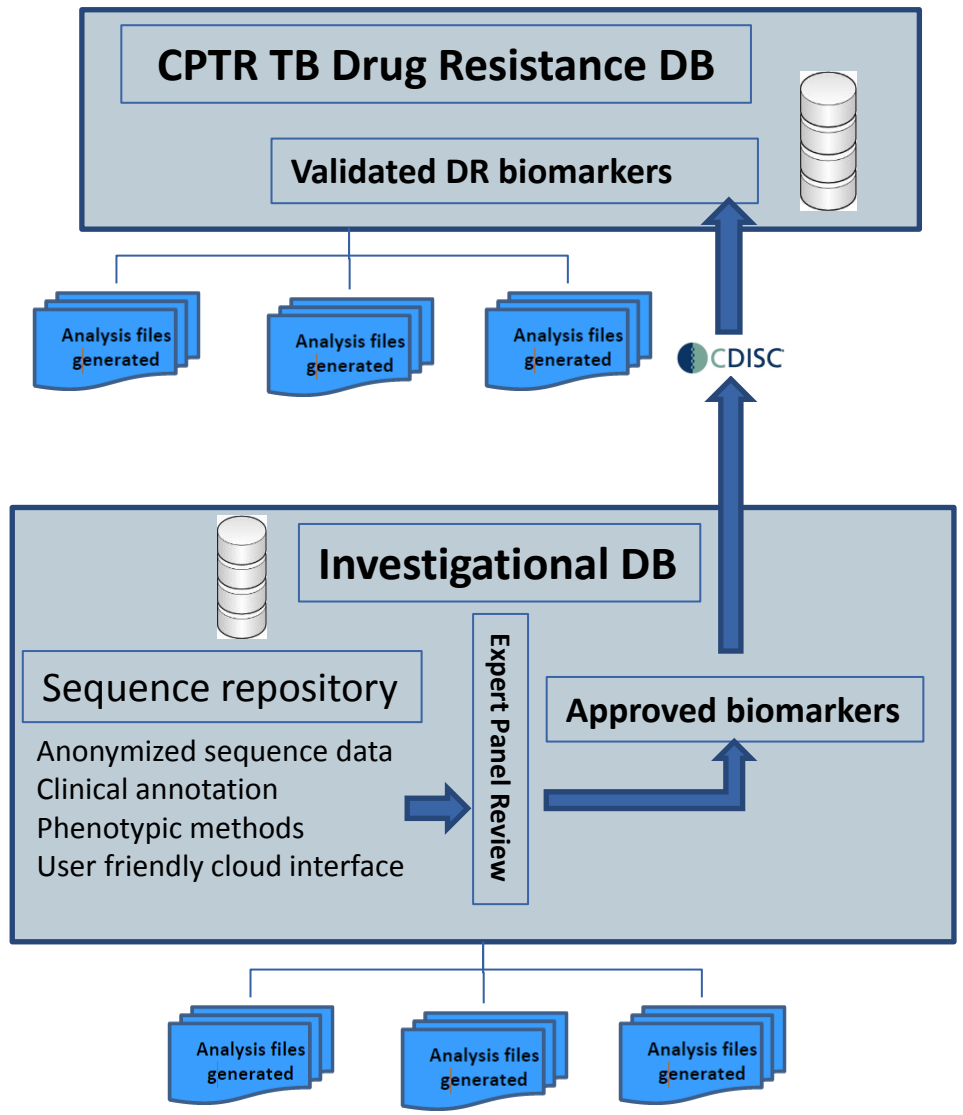
- A new clinical trial simulation tool was created and has been the first model endorsed by the FDA and EMA
- Researchers utilizing database to advance research

A Concept for Partnership Between CPTR & NDWG

Linking Global TB Sequence Researchers

- TB sequence community inputs
- Expert review to advance investigational biomarkers to validated status
- Can use separate or consolidated DBs
- FDA compliant CDISC architecture for regulatory submission DB

Approved members
- Academic labs
- Reference labs
- Commercial companies
- Others…



Thank you

CPTR