

# Critical Path to TB Drug Regimens (CPTR)

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## Research Areas



### Tool Development

Clinical Trial



### Product Development

## At a Glance

- Status: **Active Consortium**
- Year Launched: **2010**
- Initiating Organization: **Critical Path Institute**
- Initiator Type: **Nonprofit foundation**
- **Rare** disease
- Location: **International**

## Abstract

CPTR is a global public-private-partnership that support advances in regulatory science and facilitates the accelerated development of novel drug regimens, rapid drug susceptibility tests, and diagnostics for TB. This critical work is enabled by a global data sharing initiative, led by the Critical Path Institute (C-Path) and partner organizations which include World Health Organization (WHO), Global Alliance for TB (TB Alliance), Bill & Melinda Gates Foundation (BMGF) and multiple data contributors representing industry, academia, and government agencies.

## Mission

The CPTR initiative aims to deliver safer, more efficacious, and faster-acting tuberculosis (TB) regimens by developing and promoting innovative regulatory science essential for supporting new combination drug development in collaboration with its partners. Its focus is as follows:

- Develop and integrate data standards

- Qualify drug development tools and biomarkers through the Food and Drug Administration (FDA)/European Medicines Agency (EMA)
- Develop quantitative disease progression (natural history) models
- Create disease response metrics
- Develop target product profiles and a relational sequencing data platform for drug susceptibility and additional diagnostic assays
- Develop new pharmacokinetic/dynamic measures of drug interactions

## Consortium History

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2010: The CPTR Initiative launched as a public-private partnership initiated by Critical Path Institute (C-Path), the Bill & Melinda Gates Foundation (BMGF) and the Global Alliance for TB Drug Development (TB Alliance).

2010: TB Alliance launched the first-ever clinical trial of a novel combination drug regimen for TB. The trial tested new TB drug candidates in combination with an existing antibiotic. The study met its milestones, validating the approach to regimen development set forth by CPTR and highlighting the promise of a novel regimen. A new TB drug regimen known as PaMZ designed to treat both drug-sensitive and multidrug-resistant TB is moving to a global Phase III clinical trial named STAND.

2013: CPTR expanded scope to include the Rapid Drug Susceptibility Testing (RDST) consortium in partnership with National Institute of Allergy and Infectious Diseases (NIAID). expanded Modeling and Simulation development program.

2013: CPTR formed multiple partnerships to expanded Modeling and Simulation development program. Additional details on this program can be found below.

## Structure & Governance

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The CPTR initiative is built around four operating arms: Regulatory Science Consortium, Drug Development Coalition, Research Resources Group, and Drug Susceptibility Testing Group. Guiding the overall initiative is a Coordinating Group with representatives from the operating arms and CPTR's Advisory Panel. The Advisory Panel is a group of experts that provides input on related policy issues.

Each arm determines the specific structure, membership, and leadership that are most effective for their missions. Each arm generally operates through a series of workgroups, each with a targeted mission and project management plan.

The Regulatory Science Consortium, led by the Critical Path Institute, consists of scientists from industry, regulatory authorities, and academia. The consortium focuses on integrating a combination drug development framework, creating innovative tools, establishing consensus on preferred tools for developing TB drug regimens, and obtaining qualification of tools for specific context of use from regulatory authorities.

The Drug Development Coalition, led by the TB Alliance, consists of drug developers who allow their TB drug candidates and compound classes to be tested in combination with one another to attempt to assemble effective treatment regimens. This coalition is responsible for selecting promising combinations and conducting appropriate clinical trials to evaluate and bring such regimens forth to registration.

The Research Resources Group, led by the Bill & Melinda Gates Foundation, works to create the framework and infrastructure that will support the development of novel TB regimens. This includes increasing clinical trial capacity, raising funds for late-stage clinical development (Phase II and III), promoting understanding of the potential ethical challenges that go along with TB drug development, expanding regulatory guidance globally, providing relevant information on TB drug markets, and ensuring effective and appropriate stakeholder and community engagement.

The Rapid TB Drug Susceptibility Testing (RDST) Consortium, led by the Critical Path Institute, focuses on accelerating the development of a World Health Organization–endorsed clinically useful in vitro diagnostic assay specific to rapid drug susceptibility testing of TB that would support the roll out of new drug regimens. This includes work to continue collaborative research on the molecular mechanisms of drug resistance to drive the development of genomic DST assays, promote new and novel growth-based DST assays, facilitate the development of rapid TB diagnostics to meet target product profiles, improve the strategies for surveillance of TB drug resistance, and construct a data

platform to help advance basic and clinical research, guide diagnostic DST developers, empower global DST surveillance efforts, enable clinical decision making, and engage with patient and physician advocacy groups.

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## Financing

This project is supported by a grant from The Bill & Melinda Gates Foundation.

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## Intellectual Property

One of the guiding principles of the CPTR initiative is to encourage information sharing and collaboration among international organizations, industry, and regulatory agencies to innovate and accelerate TB drug development and get new therapies to patients.

In furtherance of CPTR's mission and global access principles, the Initiative and its members intend that all results, conclusions, and observations arising from its workgroups be made broadly available to members and non-members, alike, to aid and provide guidance in the development and utilization of TB drug regimen and diagnostic development for the benefit of public health worldwide.

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## Patent Engagement

To engage the TB community, CPTR invites stakeholders to participate in its annual workshop. Patient advocates are also invited and encouraged to participate in CPTR's Research Resources Stakeholder and Community Engagement workgroup (SCE-WG). Furthermore, existing clinical data from industry sponsored trials are used in some programs.

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## Data Sharing

One of the goals of the CPTR initiative is to create innovative tools to advance the field. A relational sequencing data platform (ReSeqTB), utilizes a unified data pipeline to process and annotate drug

resistance loci from next generation sequencing files. The platform scheduled for release in October 2016 will also store phenotypic and clinical metadata and is a collaborative effort between C-Path, FIND, WHO, NDWG, CDC and NIAID. A new data standard version 2.0 for tuberculosis is to be released the first quarter of 2016.

## Impact/Accomplishment

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CPTR was created to accelerate the development of new TB regimens by catalyzing innovative testing methods, product development partnerships, and novel development strategies. As a trusted and neutral third party, CPTR is able to convene consortia of industry, academia, patient stakeholders, regulators and government in precompetitive collaborations. The iterative involvement of the FDA and EMA for guidance and qualification of drug development tools and biomarkers for TB has become a hallmark for CPTR.

CPTR has also published papers in peer-reviewed journals that include the landscape for TB drug regimen development, an evidence-based evaluation of the hollow fiber system model, and a proposed need for and execution of a data sharing platform for clinically relevant genotypic and phenotypic information on *Mycobacterium tuberculosis* (Mtb).

Additional accomplishments:

- Developed and published TB data standards in collaboration with the Clinical Data Interchange Standards Consortium.
- Pursued several regulatory pathways with FDA for the Hollow Fiber System Model for TB (HFS-TB) which included submitted comments to the docket for FDA's draft guidance "Pulmonary Tuberculosis: Developing Drugs for Treatment" and a published CID supplement on the systematic review of the HFS-TB for publication with FDA authoring a companion commentary. Click [here](#) for journal supplement.
- Submission of a dossier to EMA on the HFS-TB rendered a positive qualification opinion for the model as a methodology for use in support of selection and development of anti-tuberculosis

regimens.

- Submitted a briefing book to FDA via the pre-investigational new drug (IND) process to review CPTR's data analysis plan and data inventory for liquid culture, with emphasis on time-to-positivity, as a quantitative measure of long-term outcome.
- Initiated planning to develop a database supporting the RDST Consortium's goal to develop a rapid TB drug susceptibility test.

## Links/Social Media Feed

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Homepage	<a href="http://cptrinitiative.org/">http://cptrinitiative.org/</a>
Homepage	<a href="http://c-path.org/programs/cptr">http://c-path.org/programs/cptr</a>
LinkedIn	<a href="https://www.linkedin.com/company/the-critical-path-institute">https://www.linkedin.com/company/the-critical-path-institute</a>
Twitter	@cpathinstitute

## Points of Contact

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## Sponsors & Partners

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