C-Path's Translational Therapeutics Accelerator: A Unique Solution to Successfully Bridge the Valley of Death in Academic Drug Discovery and Development

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Problem

The drug discovery and development 'valley of death' remains a challenge for new therapies coming out of academic research laboratories. These efforts need to be matured to garner interest from the investment community and pharma to drive the technology towards the clinic.

Solution

Translational Therapeutics Accelerator

The Critical Path Institute's (C-Path) Translational Therapeutics Accelerator (TRxA) is a global drug discovery and development program focused on supporting academic scientists in defining optimal strategies for advancing new, cutting-edge therapeutics from the lab to patients.

Current Success

Founded in June 2022, in its inaugural RFP TRxA was able to critically evaluate submitted applications and selected two projects for support in stages 1 and 2. For RFP2023, TRxA received 47 pre-proposals and envisions funding between 4-6 projects in diverse therapeutic areas.

Filling the Innovation Gap

Breakthroughs in the ability to probe and understand biologic systems during the past three to four decades have enabled the drug discovery community to develop new therapeutic agents and change the course of many life-shortening diseases. Despite this success, bridging the gap between promising basic science discoveries and the development of effective therapies remains risky and prone to high failure rates, with fewer than 1 in 10,000 early translational programs successfully achieving Food and Drug Administration (FDA) approval.

Academia De-risking Therapeutic Targets and Modalities

The pharmaceutical industry has realized that academia and not-for-profits significantly contribute to the discovery of new medicines and are funding collaborations and other initiatives to capture this innovation, as evidenced by the growing tendencies of pharmaceutical companies to license assets at earlier stages of development. It is estimated that 13% of FDA-approved new medical entities over the past 30 years originated from university affiliated research groups. Despite this, academic-originated basic science often fails to convert into meaningful therapies for patients.

Funding of basic research directed towards novel targets and modalities is inherently risky, therefore investors are less likely to fund at the R&D stage. In later stages of drug development, when a discovery program has been 'de-risked', there is considerably more investment available but conversely fewer programs available. This failure to advance basic scientific discoveries into real-world patient impact has been termed the 'valley of death.'

Projects with robust quality data packages and a regulatory strategy will increase investment and expedite successful commercialization.

Critical Path Institute (C-Path)

C-Path is a non-profit that leads collaborations to accelerate drug development and advance better treatments for people worldwide. As a neutral convener of patient groups, academia, pharmaceutical companies and regulatory agencies, C-Path brings a breadth of scientific and drug development planning not available in other accelerators. TRxA is uniquely situated to leverage the expertise available through C-Path's >20 disease-based consortia, as well as regulatory expertise and project management, to empower your program and your institution to succeed. TRxA does not take IP ownership.

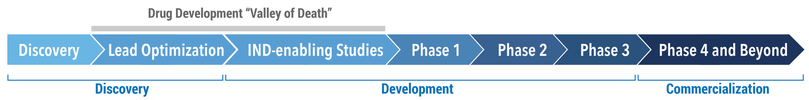
TRxA's Approach

TRxA operates as a not-for-profit drug accelerator that provides funding to academic researchers, as well as:

- Tactical and strategic drug discovery and development expertise, including regulatory science considerations.
- Resources and hands on guidance, working closely with researchers to develop comprehensive data packages for potential drug candidates, a key to garnering interest from biotechnology and pharmaceutical companies to invest in clinical trials.
- Engagement of contract research organizations (CROs) to perform critical discovery phase experiments and/or validate academic studies.

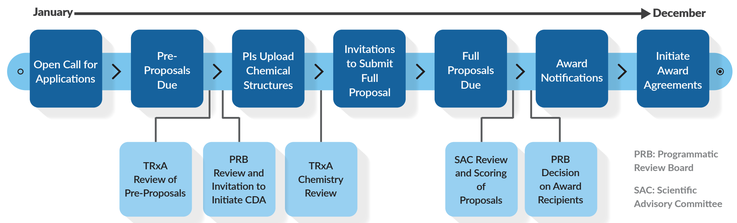
What We Fund

TRxA offers funding and support for three types of small molecule translational projects, ranging from early lead optimization to IND enabling studies.



TRxA is disease and target agnostic, has no geographical restrictions for grantees and can engage with the most appropriate CROs without consideration of where the work will be performed.

Application Process



Levels of Funding Available

Stage 1 Early lead compound to late lead series – **funding up to $250,000 for up to 12 months**

Stage 2 Late lead series to selection of clinical candidate – **funding up to $500,000 for up to 12 months**

Stage 3 Candidate selection through IND enabling studies – **funding up to $1M for up to 12 months**

Epigenetic therapy for Prader-Willi syndrome by novel small molecule G9a inhibitors

Dr. Y. Jiang and Dr. J. Jin; Yale University & Icahn School of Medicine at Mt. Sinai

The project has identified a development candidate and is scaling material and advancing to efficacy studies in a mouse model of Prader-Willi syndrome.

Establishing a pleiotropic brain-penetrant small-molecule to impede glioblastoma

Dr. C. Hulme, Dr. W. Montfort and Dr. S. Banerjee; University of Arizona & University of Dundee

The project is currently in lead optimization stage and is rapidly advancing chemical matter with desired physicochemical properties. Progress made has led to additional external funding.

Funding Success

RFP Year	# of Appl.	% CDA	% Full Appl.	% Funded
2022	15	40.0%	33.3%	13.3%
2023	47	31.9%	21.3%	12.8%

For more information about funding opportunities through C-Path's TRxA: **c-path.org/trxa**

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