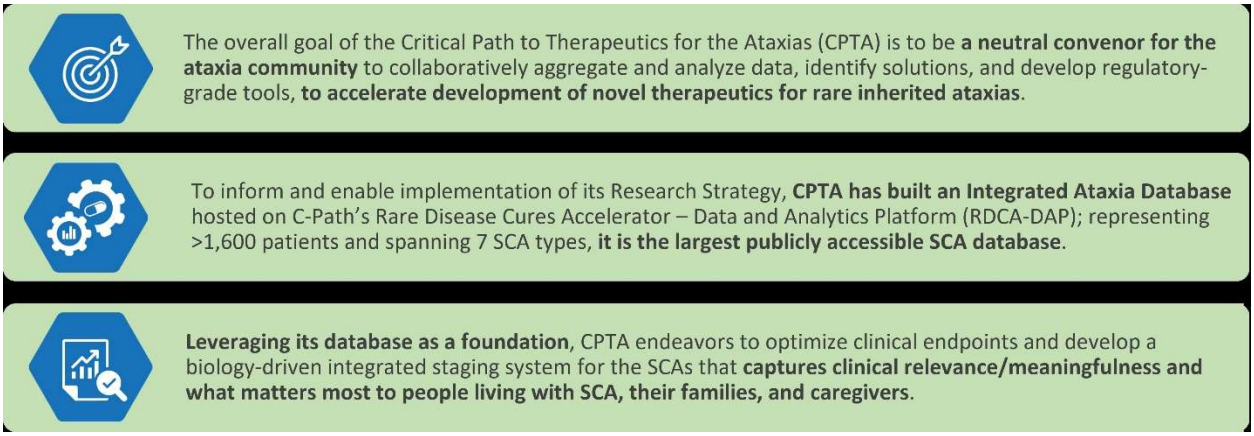
CPTA Mission and Objectives



CPTA Research Plan and Impact

The CPTA Research Plan is collaborative, pre-competitive, consensus-based, data driven, and patient-centric and designed to deliver actionable and regulatory grade solutions to address urgent unmet needs, thereby de-risking, and accelerating drug development for rare inherited ataxias.

Harnessing the power of rare disease data

Launched in November 2022, the CPTA Integrated Ataxia Database is comprised of patient-level data from 3 large natural history datasets, all harmonized and fully curated to regulatory data standards. The CPTA database serves the multi-functions of powering the consortium's Research Plan, informing decision-making and the consortium's regulatory science strategy, and providing the ataxia community with access to high-quality, patient-level data.

More information on data requests/access here: https://portal.cpta.c-path.org/

Impact Potential for CPTA's Tools and Solutions

Drug Development Tool Solution	Impact
SCA Integrated Staging System	Clinical trial enrichment, enable prevention trials
Integrated Ataxia Database	Inform research plan and regulatory decision making
Novel outcome measures / endpoints	Improve ability to detect onset and changes in meaningful symptoms

For more information on CPTA visit: https://c-path.org/program/critical-path-to-therapeutics-for-the-ataxias/

Recent Consortium Highlights and Achievements

- Strategic configuration of CPTA within the new Critical Path for Rare Neurodegenerative Diseases Program (https://c-path.org/program/critical-path-for-rare-neurodegenerative-diseases/)
- Launched the Outcome Measures Working Group
- Convened quarterly industry member strategy meetings to build consensus and hone the consortium's priorities and strategies
- Ingested, curated, and launched the CRC-SCA natural history dataset in the CPTA Integrated Ataxia Database
- Evaluated the consortium's initiatives against relevant, new FDA Guidance
    - Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments Into Endpoints for Regulatory Decision-Making (guidance document)
    - Rare Diseases: Considerations for the Development of Drugs and Biological Products (guidance document)
- Commemorated Ataxia Awareness Day on September 25
- Participated in C-Path's Rare and Orphan Disease annual meeting in Washington, D.C.

CPTA profoundly appreciates its members!

Industry Members:

- Biogen
- Biohaven Pharmaceuticals
- Institut De Recherches Internationales Servier
- PTC Therapeutics

FDA CDER Liaison:

- Michelle Campbell, FDA

Advisory Member Institutions:

- Besta Institute
- Charité University of Medicine, Berlin
- Clinical Data Science Gmbh
- Houston Methodist Hospital
- Medical University of Innsbruck
- Northwestern University
- Paris Brain Institute
- Sorbonne University
- University of Bonn
- University of Chicago
- University of Tübingen

Nonprofit Members:

- National Ataxia Foundation
- Ataxia UK
- Ataxia Charlevoix-Saguenay Foundation
- Ataxia Global Initiative

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