



# Data sharing success – CPTA and RDCA-DAP

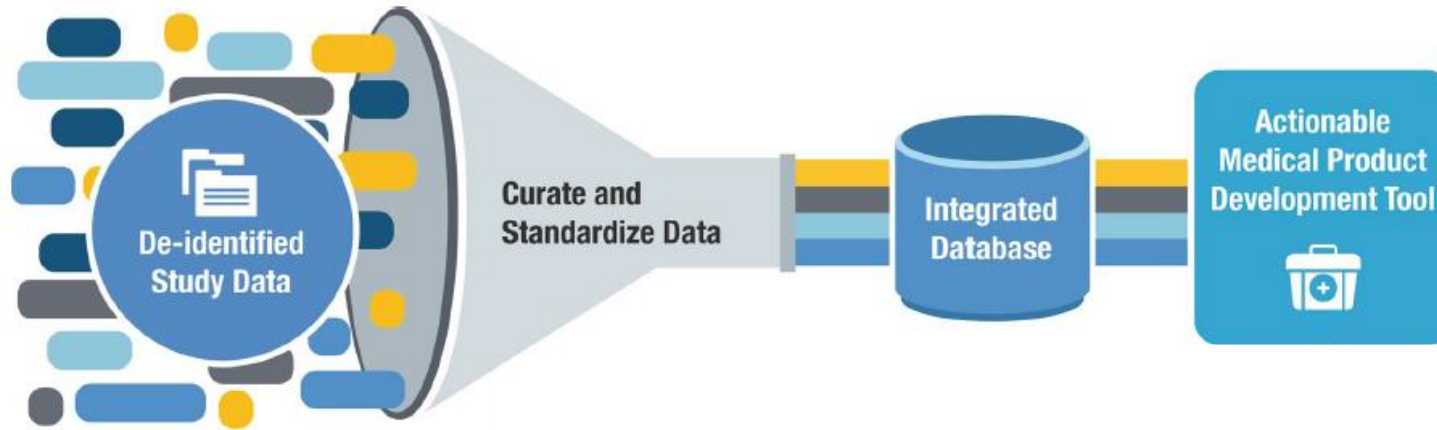
November 4, 2022

Kael White, PhD, Associate Director CPTA



- Critical need for bridging knowledge gaps in clinical trial design in the ataxia space
- Interest in early-stage intervention for SCAs
- Rare SCAs have a limited number of patients to participate in clinical trials, making quantitative characterization of progression more important

The data upon which to do this  
is limited and precious



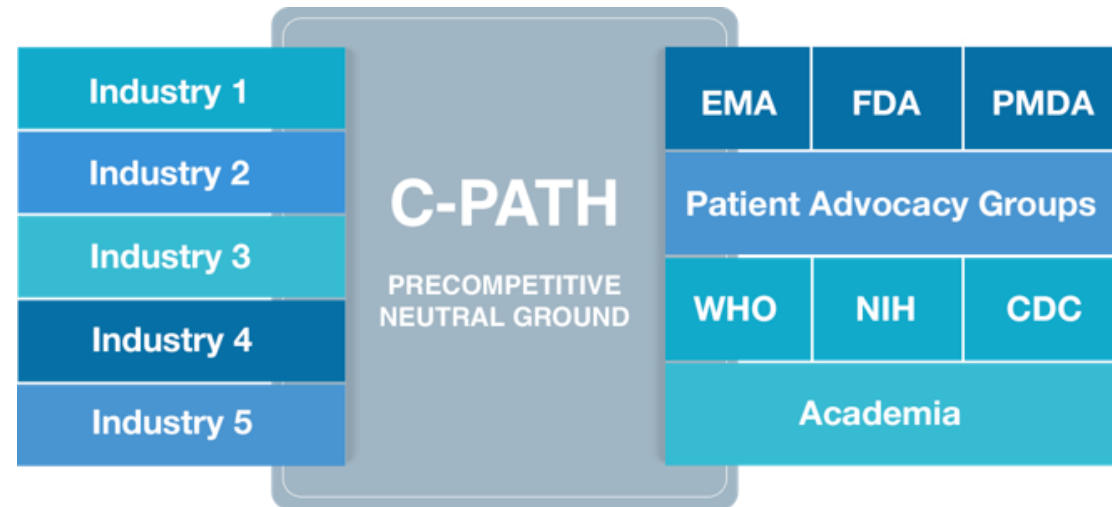
Effective data sharing:

- Is **actionable**, improves decision making
- Often through facilitating **novel** tools and methodologies
- Often done in the **pre-competitive** space
- Requires commitment from **diverse stakeholder** groups

# C-Path is uniquely situated to support data sharing

- Acts as a trusted, neutral third party
- Convenes scientific consortia of industry, academia and government for sharing of data and expertise
  - ✓ The best science
  - ✓ Active consensus building
  - ✓ The broadest experience
  - ✓ Shared risk and costs

- Enable iterative FDA/EMA participation in developing new methods to assess the safety and efficacy of medical products

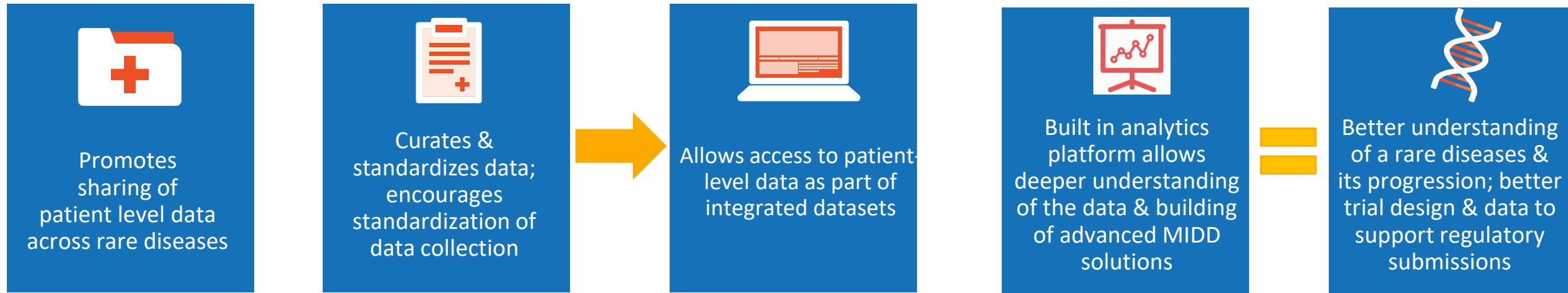


**Official regulatory endorsement of novel methodologies and drug development tools**

# Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP)



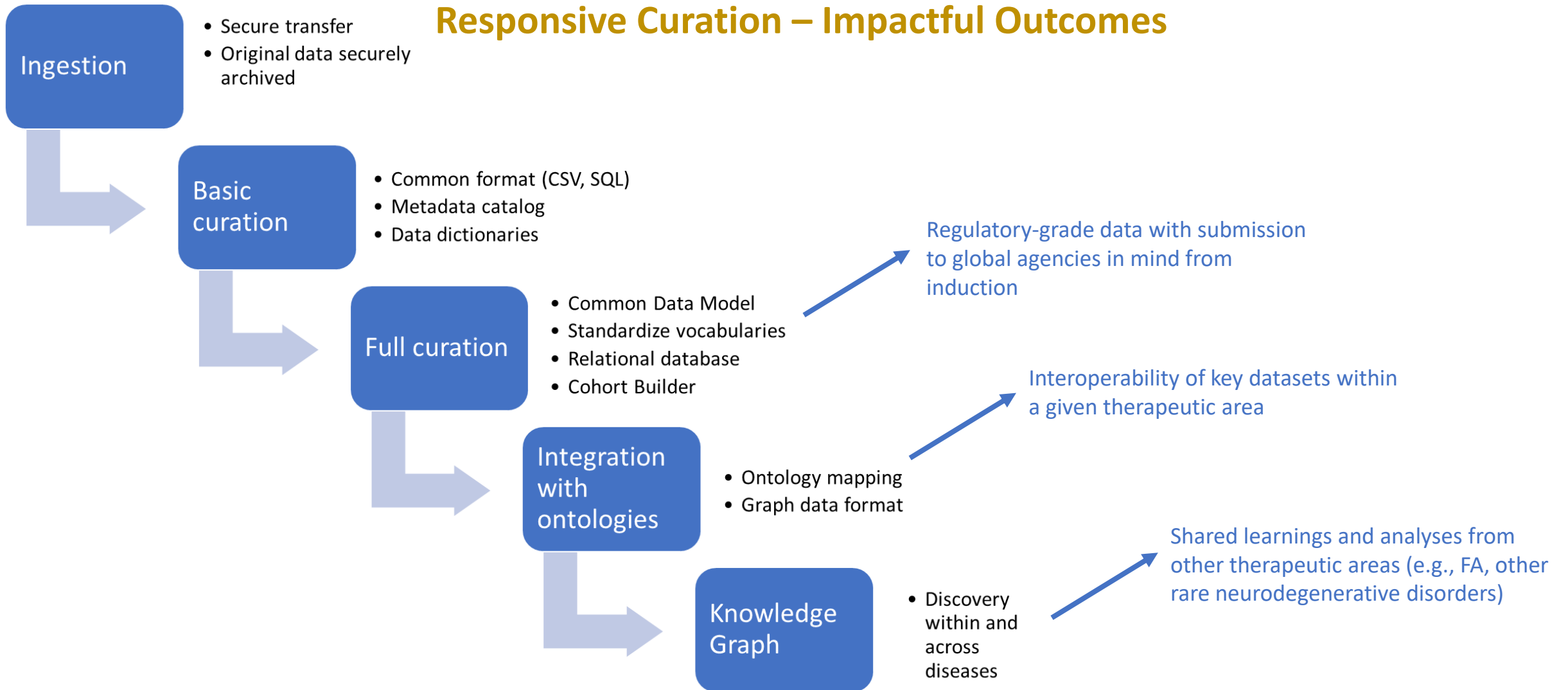
RDCA-DAP: a neutral, independent data collaboration and analytics hub to **promote the sharing of critical data across rare diseases** in order to accelerate the understanding of disease progression and optimize clinical trial design.



RDCA-DAP is an initiative led by Critical Path Institute in collaboration with FDA and NORD

# C-Path ensures high-quality, curated data

## Responsive Curation – Impactful Outcomes



# C-Path is uniquely situated to deliver results

## High-Quality Data with high usability



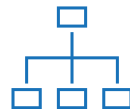
Shared data dictionaries  
and metadata catalogues



Common Data Models  
(where available)



Standardized data  
vocabularies



Relational databasing



Cohort builder



Ontology mapping



## C-Path Core Competencies



Data sharing and management  
(CODR/RDCA-DAP)



Modeling & Analysis



Harmonized image analysis



Fluid/Imaging biomarkers



Clinical Outcomes Assessments



Digital Health Tools



Regulatory Science



## Value Proposition

Knowledge  
Creation

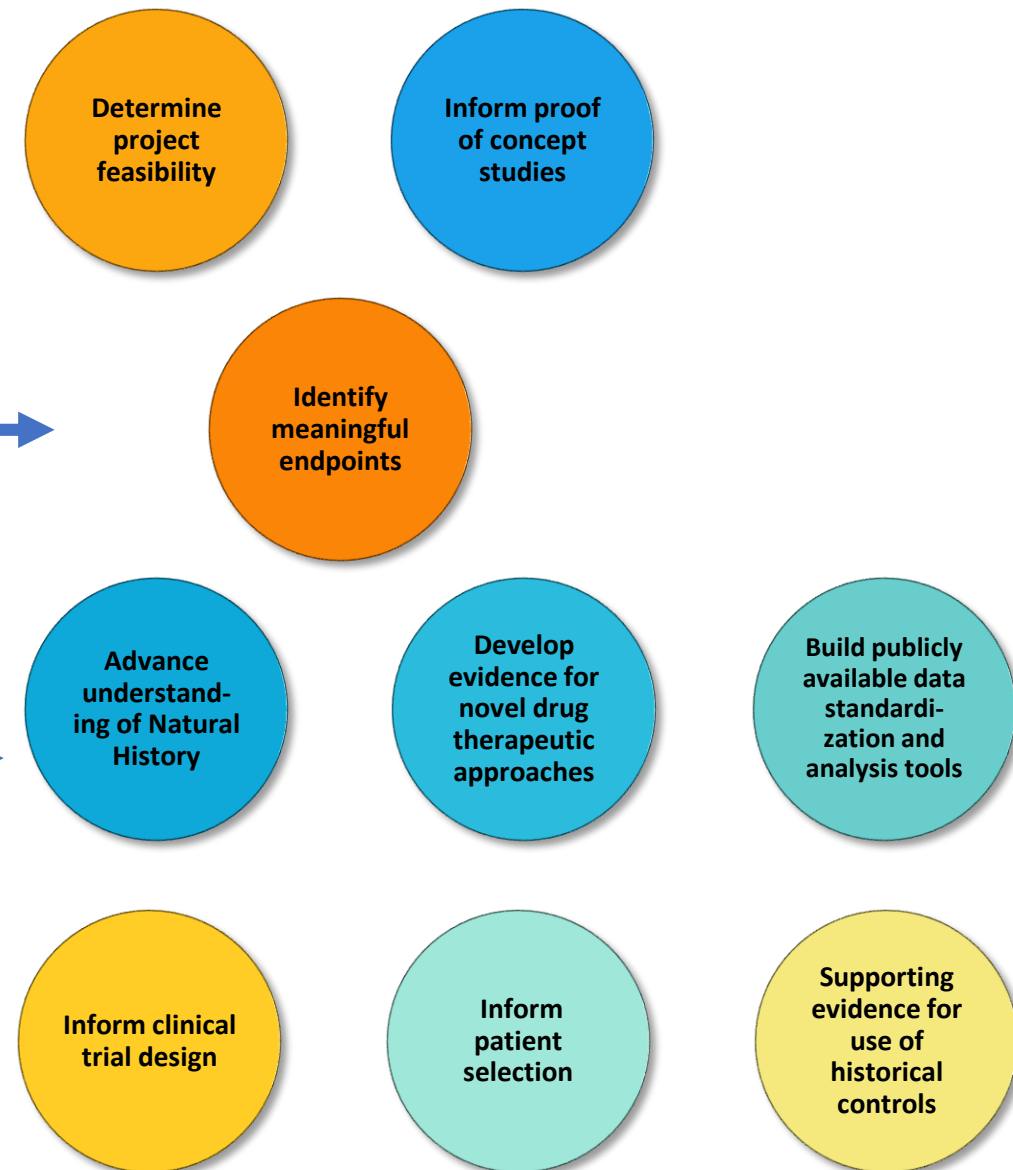
Knowledge  
Sharing

Knowledge  
Application

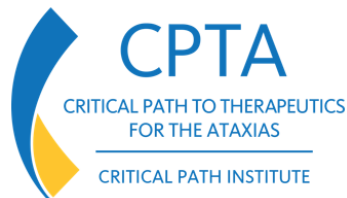
Accelerated  
Progress

# Data utility to support future projects and Research Plan goals

- Data-driven approach to project identification and prioritization
- Evaluation and optimization of COAs and other outcome measures
- Better understanding of disease progression and key stages
- Innovation in clinical trial design through qualitative analyses and novel tools




RDCA-DAP®



CPTA  
CRITICAL PATH TO THERAPEUTICS  
FOR THE ATAXIAS  
CRITICAL PATH INSTITUTE

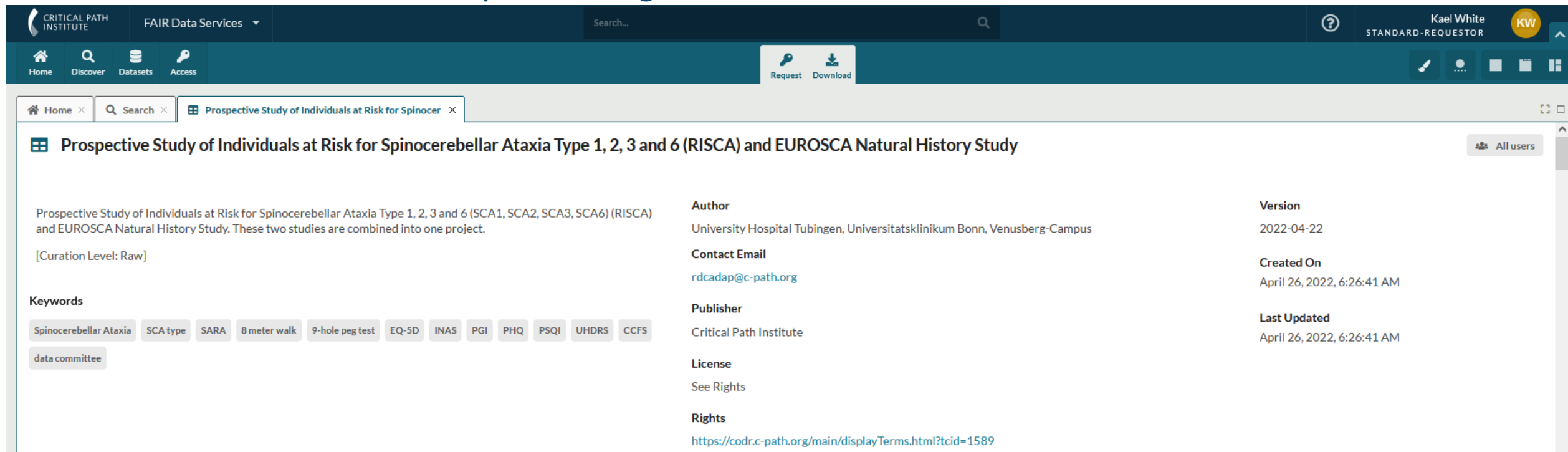
RDCA-DAP provides an innovative, accessible platform that supports CPTA's core mission and Research Plan goals



# EUROSCA and RISCA data on RDCA-DAP

EUROSCA and RISCA data are curated and available for request on the RDCA-DAP

- Meta/data are now searchable and browsable using the FAIR Explorer on the RDCA-DAP website: <https://fair.dap.c-path.org/#/data/home/>
- Access to data can be requested through this website
- Future datasets currently under negotiation will be added to the RDCA-DAP



The screenshot shows the RDCA-DAP website interface. The top navigation bar includes the CPTA logo, 'FAIR Data Services', a search bar, and user information for 'Kael White' (STANDARD-REQUESTOR). Below the navigation bar, there are tabs for 'Home', 'Discover', 'Datasets', and 'Access'. The main content area displays the details for a dataset titled 'Prospective Study of Individuals at Risk for Spinocerebellar Ataxia Type 1, 2, 3 and 6 (RISCA) and EUROSCA Natural History Study'. The dataset description states: 'Prospective Study of Individuals at Risk for Spinocerebellar Ataxia Type 1, 2, 3 and 6 (SCA1, SCA2, SCA3, SCA6) (RISCA) and EUROSCA Natural History Study. These two studies are combined into one project.' The curation level is 'Raw'. The keywords section includes 'Spinocerebellar Ataxia', 'SCA type', 'SARA', '8 meter walk', '9-hole peg test', 'EQ-5D', 'INAS', 'PGI', 'PHQ', 'PSQI', 'UHDRS', 'CCFS', and 'data committee'. The metadata section lists: Author (University Hospital Tubingen, Universitätsklinikum Bonn, Venusberg-Campus), Contact Email (rdcadap@c-path.org), Publisher (Critical Path Institute), License (See Rights), and Rights (https://codr.c-path.org/main/displayTerms.html?tcid=1589). The version is 2022-04-22, created on April 26, 2022, 6:26:41 AM, and last updated on the same date and time. The user 'All users' is shown in the top right corner.



Thank you!

Questions or comments?

Contact Kael White ([jkwhite@c-path.org](mailto:jkwhite@c-path.org))

