



Regulatory considerations for using the RDCA-DAP program

Klaus Romero MD MS FCP

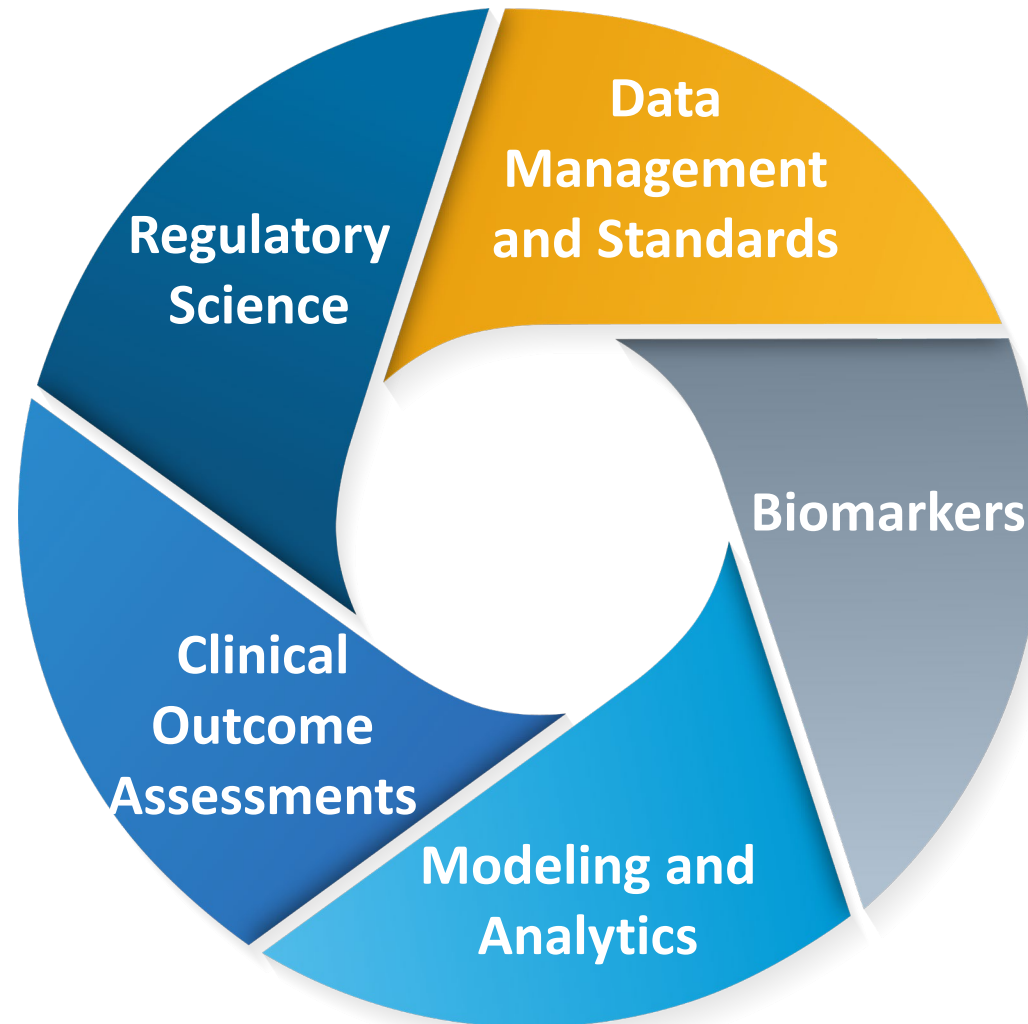
Chief Executive Officer

Critical Path Institute

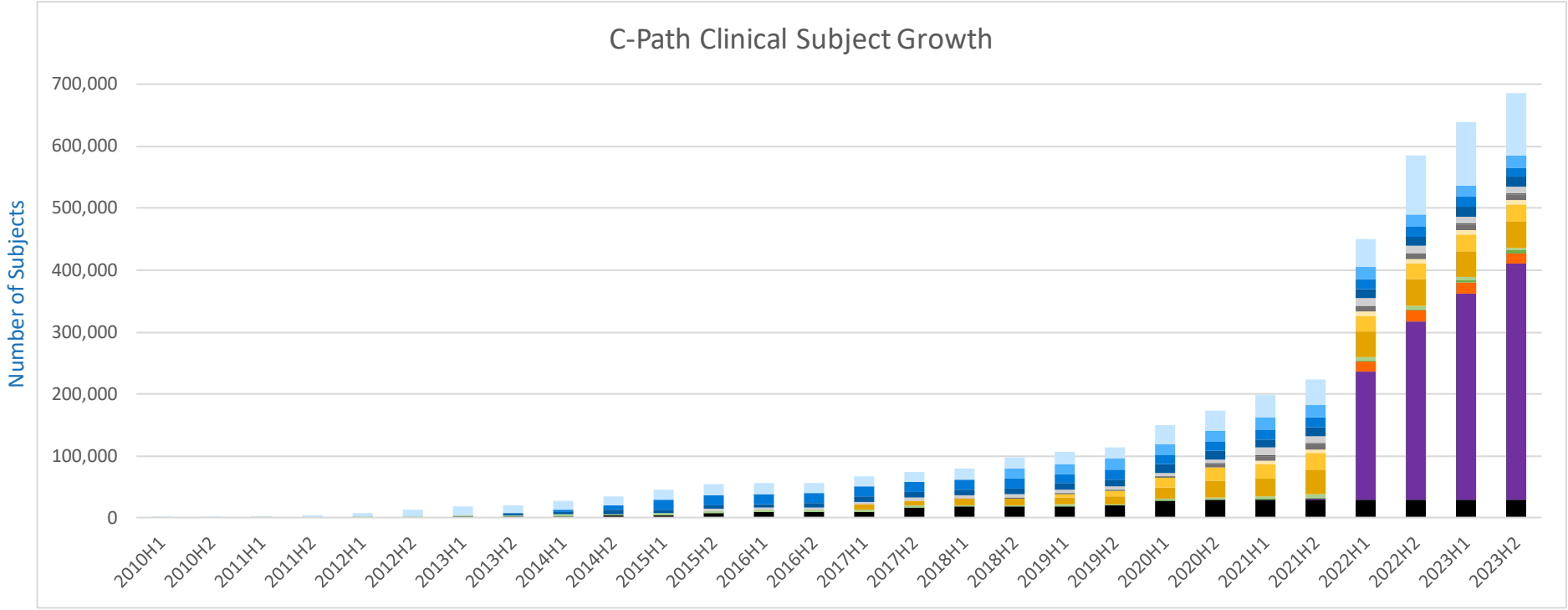
Advancing Drug Development. Improving Lives. Together.



C-Path Core Competencies



Total Data Integrated



Clinical Data

Studies	422
Subjects	689,886

Nonclinical Data

Studies	153
Subjects	13,082

Neuro	
Alzheimer's Disease	100,812
Huntington's Disease	19,665
Multiple Sclerosis	15,626
Parkinson's Disease	15,056

Rare	
Duchenne's Muscular Dystrophy	11,442
Friedreich's Ataxia	1,572
Rare Diseases	8,941

CURE Drug Repurposing	17,145
-----------------------	--------

IHP	
Sickle Cell Disease	7,921
Transplant Therapeutics	26,264
Type 1 Diabetes	42,581

Neonatal	379,403
----------	---------

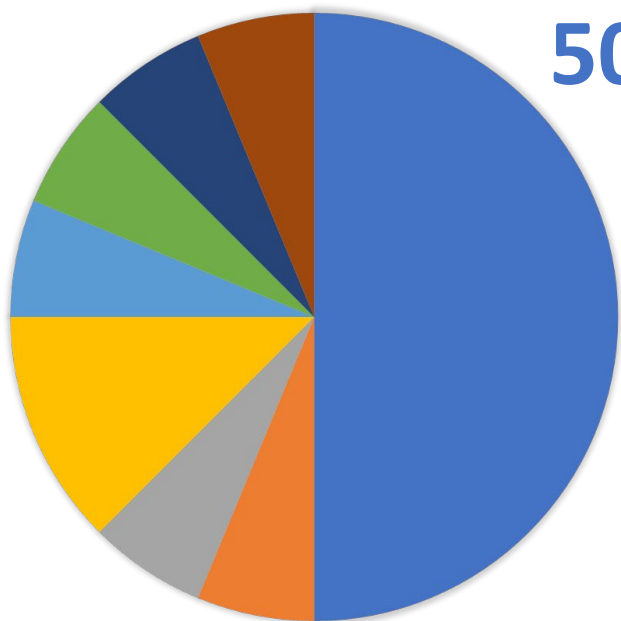
TSSP	
Polycystic Kidney Disease	4,519
Safety Testing	4,749

Tuberculosis	30,389
--------------	--------

Note: Studies currently undergoing curation are only counted in Total Studies until evaluated.

FDA Regulatory Endorsements to Date

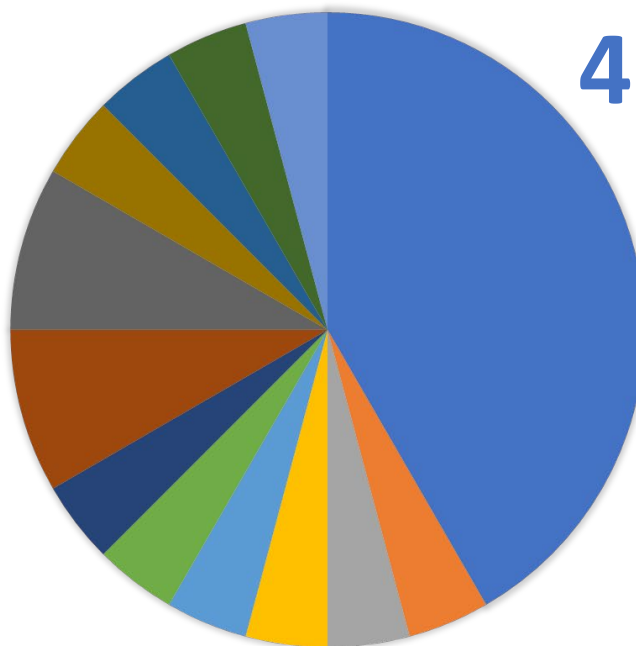
C-Path
50%



Qualifications

8 of 16
Qualification

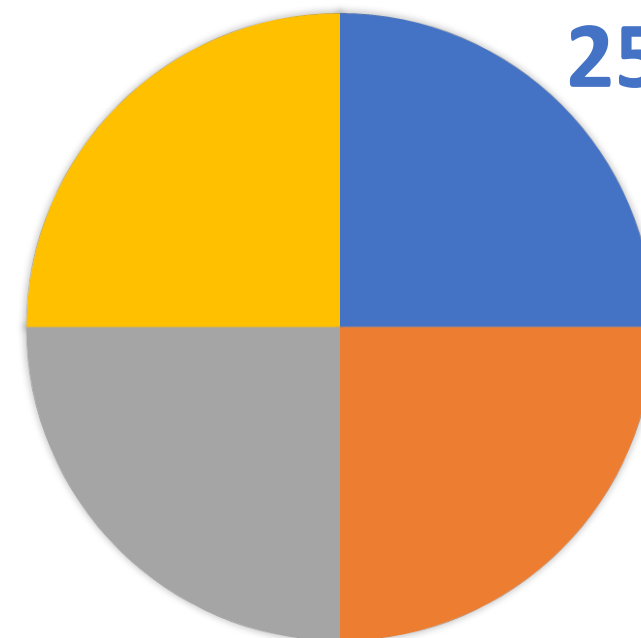
C-Path
42%



Letter of Support

10 of 24
Letters of Support

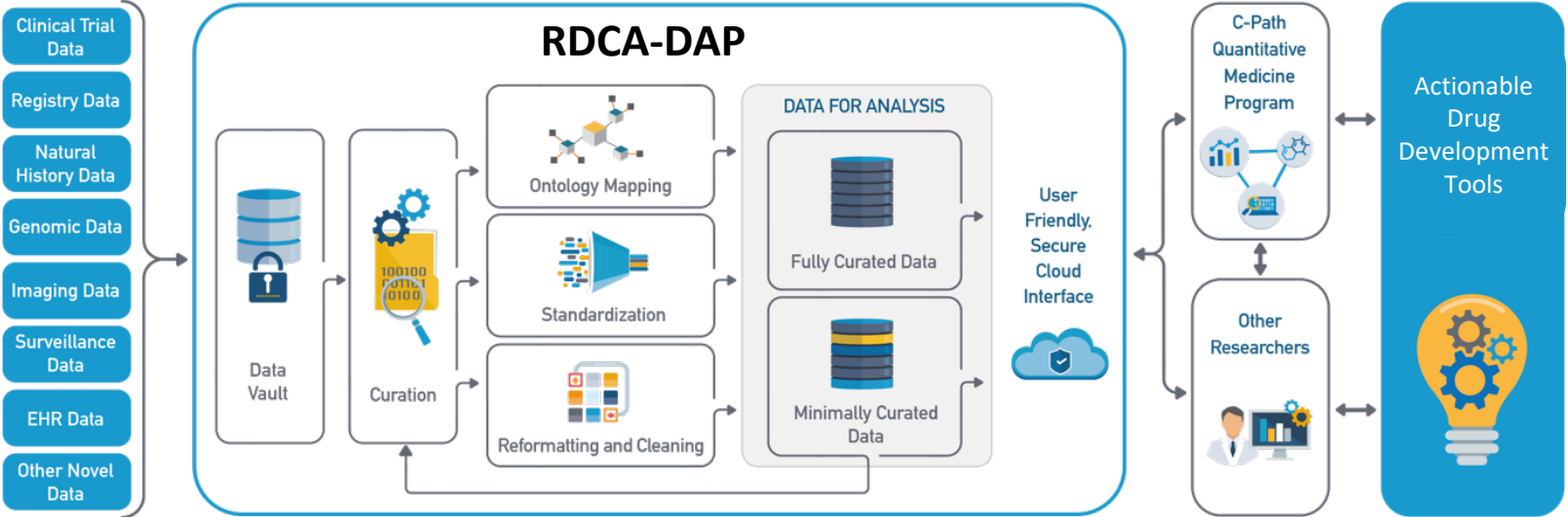
C-Path
25%



Fit for Purpose

1 of 4
Fit for Purpose

Totality of Evidence: Impacting FA Patients



Friedreich's Ataxia aggregated
database
Standardized & Accessible to
Industry



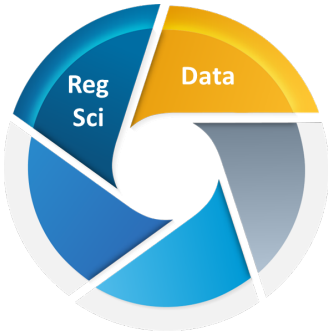
Database exploration, FA-COMS study
selection:
Propensity match analysis to support
MOXIe open-label extension trial



“The data collected in the natural
history study were critical to
showing the drug’s benefit”
(Reata CEO J. Warren Huff in “FDA
Widens Path for Rare-Disease
Treatments With New Approval,”
WSJ, March 1st 2023)

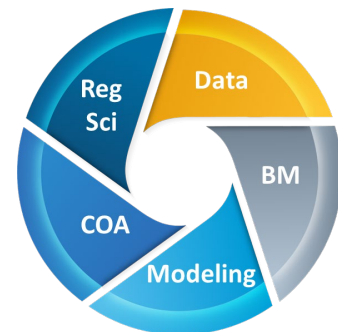
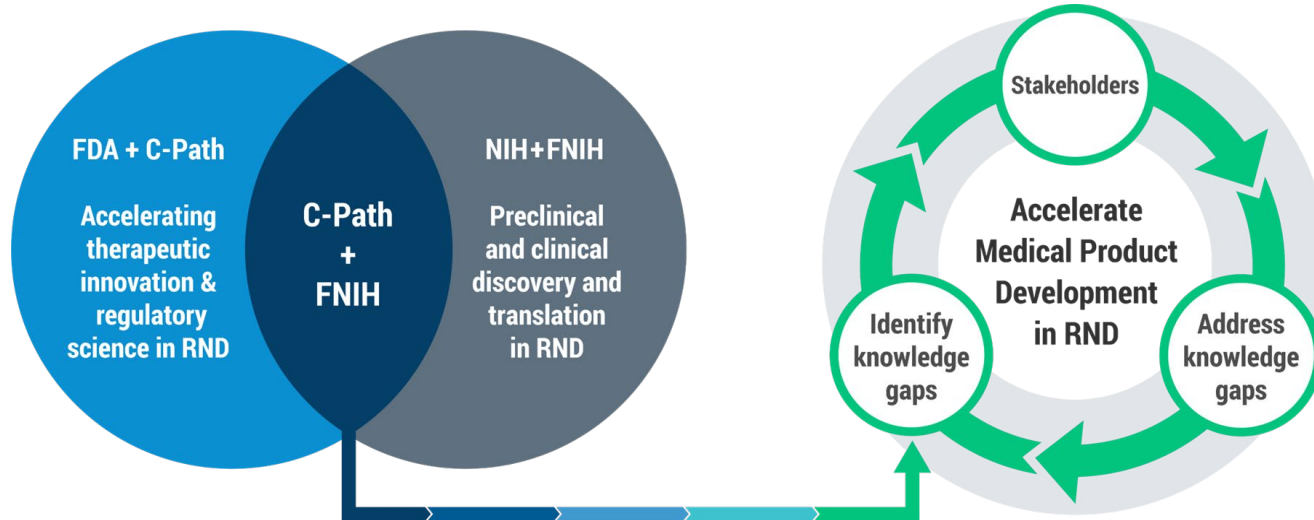


First drug approved for FA

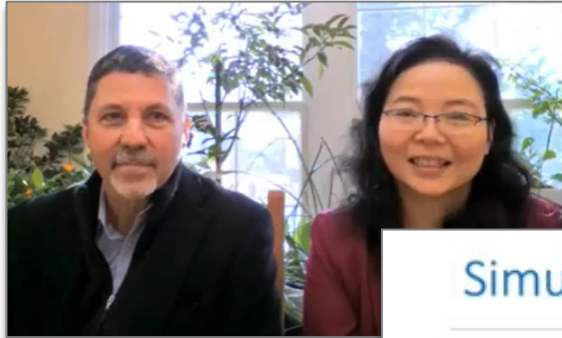






Bringing the ALS Community Together

- ACT for ALS supported Public Private Partnership involving PWLE, NIH/FNIH/FDA, sponsors
- Extensive outreach and landscaping performed to identify unmet needs in ALS and other RNDs
- Involvement of all C-Path Matrix Resources
- 5-Year ALS Plan involving WGs
 - RDCA-DAP Clinical Data Warehouse (Federated to AMP-ALS)
 - COA/DHTs/Biomarkers
 - PWLE Advisory
 - Disease staging/CTS
 - Learn and Confirm connection with other PPP members
- Collaboration with RDCA-DAP to establish Task Groups in PSP, FTD, rare epilepsies



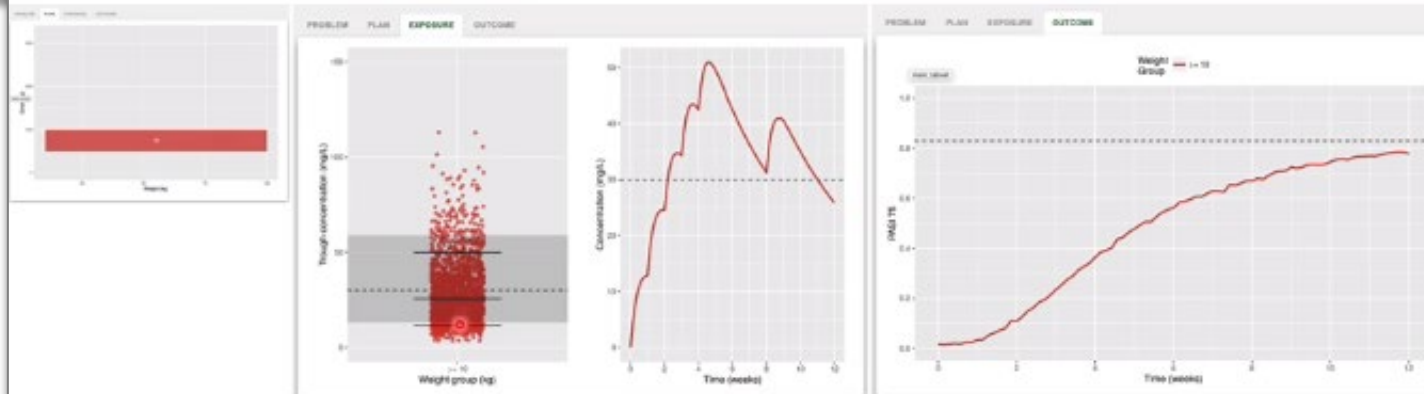
Making MIDD a house-hold term



Completed	Completed	In Progress	In Progress
			
Introduction to MIDD Training Course	Overview - Model Informed Drug Development	Module 1 - MIDD Landscape for Drug Development and...	Module 2 - Review of the Various Model Types Commonly Used for MIDD

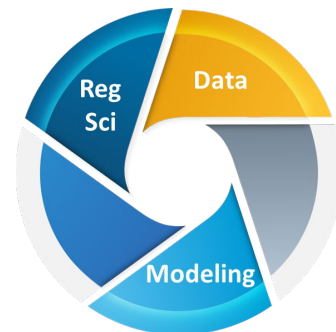
CDER Office of Communications

Simulating pediatric dosing scenarios



- Explore impact of dose, weight categories on exposure and response
- Is more than one dose group needed?
- What are potential knowledge gaps and assumptions?

<https://metrumrg.shinyapps.io/mrgsolve-demo-acop7/>



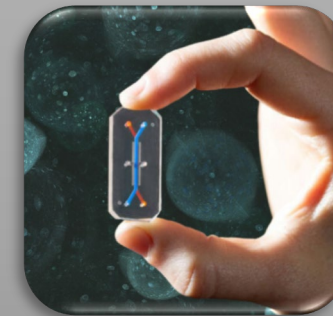
Future Opportunities



Large language models for drug development and scalable for future use in DDT



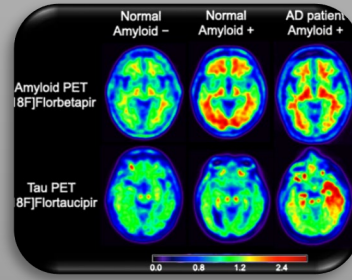
Algorithm literacy for drug development from Digital Drug Development Tools



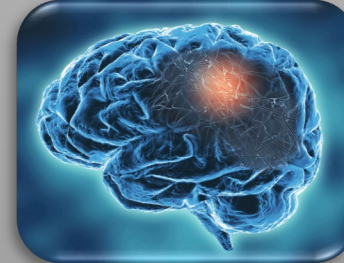
Complex in vitro models



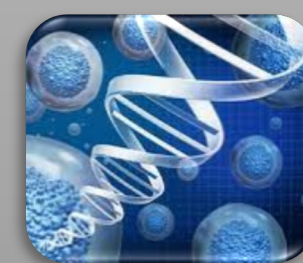
Launch new collaborations



Imaging in neurodegeneration



Optimized staging for neurodegeneration



Regenerative therapies



Advancing Drug Development.
Improving Lives. Together.

c-path.org

