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Rare Disease Cures Accelerator-Data and Analytics Platform
Virtual Workshop 2020

Up Next: *The Rare Disease Cures Accelerator- how the data and analytics platform accelerates rare disease drug development*

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Michelle Campbell, PhD

Sr. Clinical Analyst, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience

FDA/CDER

The Rare Disease Cures Accelerator: How the Data and Analytics Platform Accelerate Rare Disease Drug Development

Michelle Campbell, PhD
Office of Neuroscience
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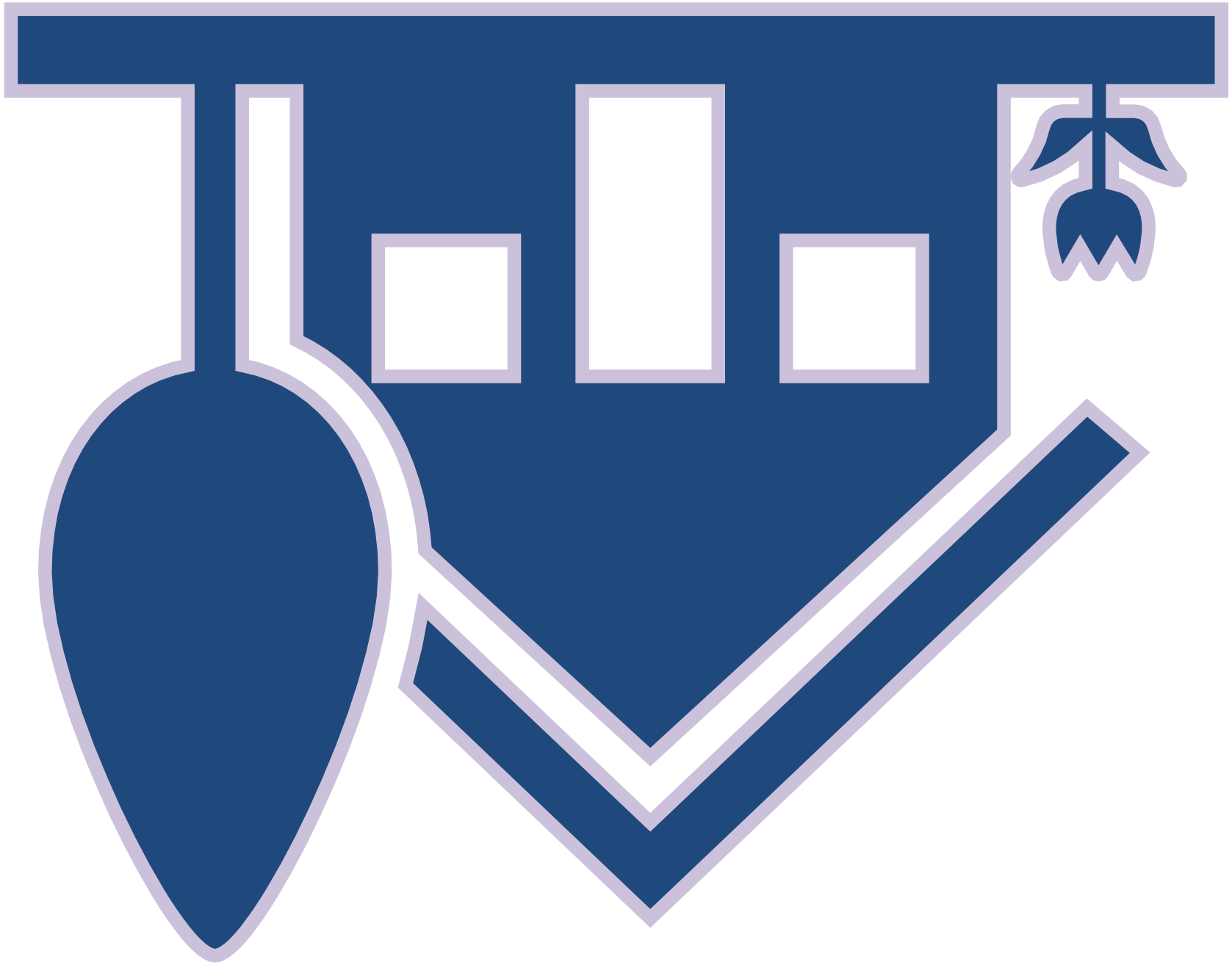
What We Know

- 7 million rare diseases
  - Only 10% have an approved treatments
- Making strides in rare disease drug approvals
  - In August 2020 the first oral drug was approved to treat patients two months of age and older with spinal muscular atrophy (SMA)
  - In June and August 2020 two different monoclonal antibodies were approved to treat patients with neuromyelitis optica spectrum disorder (NMOSD)
- We still have work to do



Large patient population

Have established endpoints

Core set of symptoms

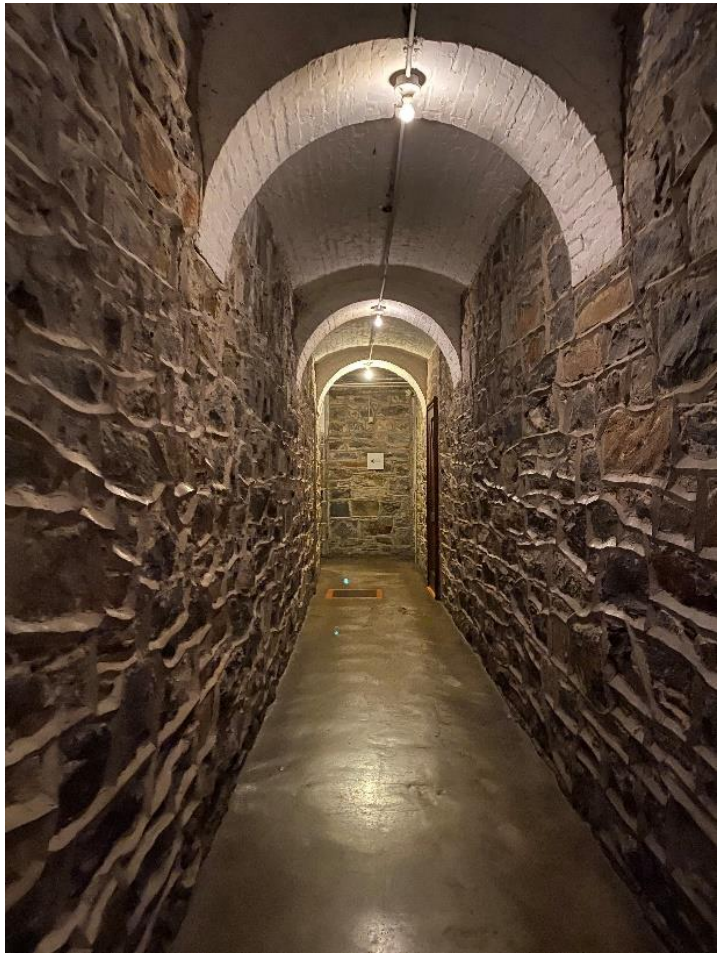
May have animal models of disease

Have an understanding of the disease course

Common Challenges in Disease Drug Development

- Natural history often poorly understood
- Possible unknown cause of disease
- Genotypic/phenotypic heterogeneity within a disease
- Often serious/life-threatening, progressive
- Lack of established efficacy endpoints
- Small and often disperse patient populations

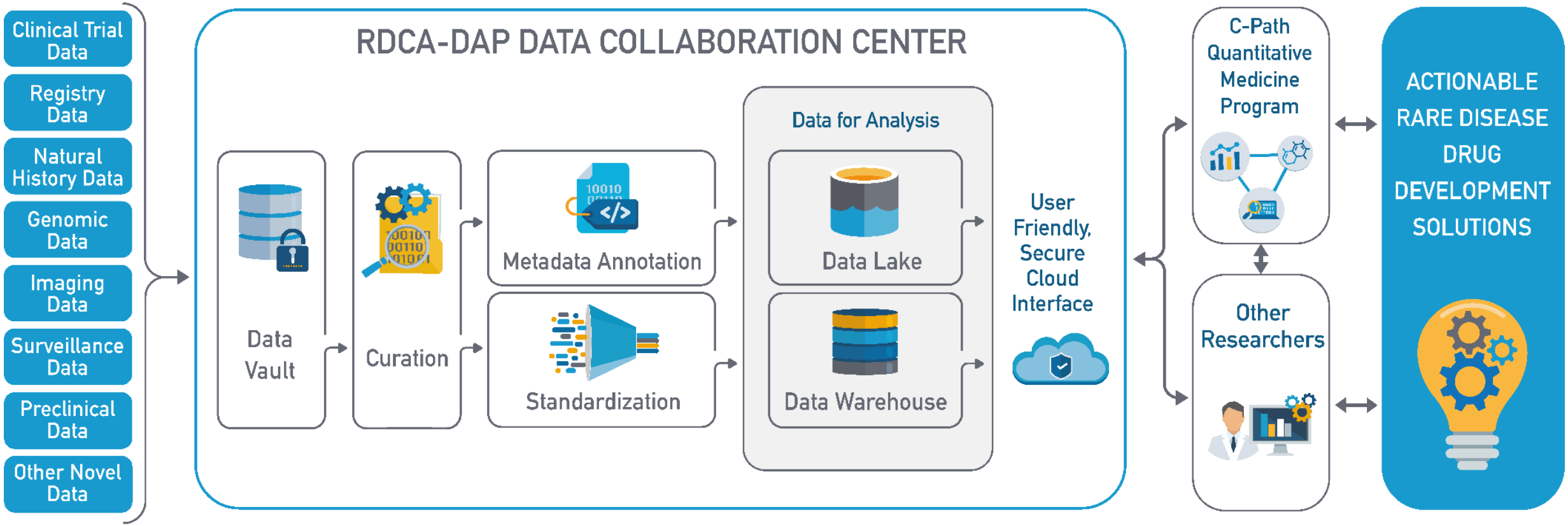
How Do We Address These Challenges



- By building a solid foundation
- By integrating data from multiple data sources into a central repository
- By building a platform to enhance clinical trials readiness in the pre-competitive space

HOW DO WE ACCELERATE RARE DISEASE DRUG DEVELOPMENT?

RDCA-DAP


Clinical Trial Data
Registry Data
Natural History Data
Genomic Data
Imaging Data
Surveillance Data
Preclinical Data
Other Novel Data
RDCA-DAP DATA COLLABORATION CENTER
Data Vault
Curation
Metadata Annotation
Standardization
Data for Analysis
Data Lake
Data Warehouse
User Friendly, Secure Cloud Interface
C-Path Quantitative Medicine Program
Other Researchers
ACTIONABLE RARE DISEASE DRUG DEVELOPMENT SOLUTIONS

RDCA-DAP: Long-term goal for impact on drug development

- Development of more efficient and effective clinical trial protocols
- Standardized data that can be extracted in CDISC format for regulatory submissions
- Aggregated data will allow for a better understanding of the variance in disease progression across broad range of patients aiding in development of optimized clinical trial protocols (endpoints, inclusion criteria, length and size of trial)
- Analytics and simulation tools to help optimize your trial protocol for your therapy
- Ability to look at dynamics of change in outcome measures and biomarkers in individual disease states and in related diseases and understand sources of variation in rate of change.
- Ability to potentially find and match historical or contemporary control patients to enrich your placebo arm and reduce numbers of patients.

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THANK YOU!

Don't forget to answer survey questions.

For more information, email rdcadap@c-path.org

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