CRITICAL PATH INSTITUTE

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National Organization for Rare Disorders

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

Opening Remarks

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

Context and Motivation– 2019 Project Launch

- An estimated 7,000+ rare diseases
  - Total number of Americans living with a rare disease is estimated to be 25 to 30 million.
  - Progress has been made in diagnosing and treating a variety of rare diseases BUT most have no available treatments
- Regulators working with rare disease patients, investigators, and companies, mostly one at a time, and most struggling with the same challenges:
  - Vast knowledge gaps about the natural course of the disease and small dispersed patient populations that make it hard to do the randomized clinical trials that save lives.
- Responding to the need for a better solution.

RDCA - Targeting Some Very Challenging Areas in Rare Disease Drug Development

Global Rare Disease Clinical Trials Network

Clinical Study of New Treatments

- Vision would be to develop a “trial-ready” network of investigators and clinical sites for rare diseases to provide a fast-track implementation path for evaluating promising therapies, and a standardized approach to planning and conducting clinical trials.
- **May 2020**: To inform future planning for collaboration, FDA issues a Request for Information and Comment on Rare Disease Clinical Trial Networks
  - Requesting public input on practical steps and successful approaches relating to the start-up, implementation and sustainment of global clinical trials networks, including specific considerations for establishing such networks for a range of rare diseases.

Developing standard core sets of Clinical Outcome Assessment (COAs) for a given disease/or domain

Getting Patient Perspective on Disease and Treatment

- Developing COAs that are fit-for-purpose for intended use in drug development can be a lengthy resource-intensive process
- CDER is piloting a grant program to support the development of standard core COAs and related endpoints for specific disease indications/ domains
  - A standard core set can include different types of COAs (e.g. PROs, ClinROs, ObsROs, PerfOs) and endpoints including a minimum list of impacts that matter most to patients, and are likely to demonstrate change and should be reported in a clinical trial.
  - A standard core set might be relevant across several rare disease populations or subgroups or be focused on attributes of a specific disease

Standard Core COAs with Relevance to Rare Disease Drug Development

FDA announced 2 grant awards in 2019*

- **Clinical Outcome Assessments for Acute Pain Therapeutics in Infants and Young Children** (COA APTIC) – project will identify COAs and endpoints for use when developing acute pain therapeutics in infants and young children, primarily those 0-2 years.
- Northwestern University Clinical Outcome Assessment Team (NUCOAT) – project will develop and validate **COAs for physical function with applicability across a range of chronic conditions, including rare diseases** that assess physical function using patient-reported and performance outcomes.

Funding Opportunity Newly Released in 2020

The purpose of this FOA is to solicit applications to support development of publicly available core set(s) of COAs and their related endpoints for the following four areas:

- **Fluid overload in nephrotic syndrome**
- **Age appropriate domains of pediatric daily functioning**
- The **mechanics of swallowing and speech** from infancy to adulthood
- **Treatment effects in systemic sclerosis**

The deadline to submit an application to this FOA, RFA-FD-21-004, is October 14, 2020.

* https://www.fda.gov/drugs/development-approval-process-drugs/cder-pilot-grant-program-standard-core-clinical-outcome-assessments-coas-and-their-related-endpoints

Infrastructure to support and accelerate rare disease characterization

Characterization of Disease

- Efficient comprehensive characterization of natural history of a given rare disease targeted for clinical development
- Rigorous analysis with attention to established data quality standards in order to be most useful to clinical trial design and regulatory review
- A standardized rare disease natural history study data platform to provide a sustainable approach
  - Provide a disease-neutral background data framework for the conduct of standardized natural history studies.
  - Disease-specific needs would be layered onto this framework to provide a rapid means for standardized, yet customized, development of natural history studies for any given disease.

RDCA-DAP -- Achievements in Year 1

- Project launched in September of 2019 and open for data sharing.
  - Currently over 40 rare disease datasets in-house from 10 disease areas, which we are using to help design the analytics platform.
- Concept of RDCA-DAP discussed with hundreds of stakeholders through public meetings, webinars , focus groups to understand what rare disease community needs from the DAP
  - Stakeholders include industry, regulators, patient groups, academics and individual patients.
  - Learnings are informing how to make DAP as useful as possible for as many as possible.
- Using this feedback to finalize data governance and data sharing policies to encourage data sharing and make it as easy as possible to get data into the platform, access the data, and accelerate ability to share and use rare disease data.
  - Working towards implementing FAIR principles to share data as simply as possible while respecting the needs of data contributors, which will maximize the value of the platform.
- Developing strategy around standards and ontologies to aid in maximizing data integration, usability and searchability
- Built a series of prototypes that demonstrate how data may be searched and used in the analytics platform,
  - Also building examples of how the data may be used to develop more advanced drug development tools as will be shown at this workshop.

RDCA-DAP -- Milestones for Year 2:

- **Opening the Data and Analytics Platform for initial queries and data sharing**– early version should be available in first quarter 2021; will preview at this workshop.
- **Provision of standardized feedback** to early data custodians around structure and format of their data, recommendations for future data collection
- **Setting up collaborations** with at least one additional partner **collecting data across multiple diseases**; data transfer.
- **Setting up collaborations** with outside entities to work towards establishment and **development of rare disease standards and ontologies**
- **Completion of current prototype projects**, engagement in additional projects to develop rare disease drug development tools and analyses to accelerate medical product development for rare diseases.

Today's Meeting Objectives

- Explain how RDCA-DAP will work to accelerate rare disease drug development
- Demonstrate RDCA-DAP'S first year progress and development
  - How the platform helps search and use data
  - Case studies of use of data to support drug development
- Share ways to get involved and the benefits of engaging with RDCA-DAP

- We thank you for joining us today!

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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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