FROM DATA TO RARE DISEASE CURES: SPEEDING THE WAY

July 28, 2020

Access the recorded webinar here.
This webinar is being recorded.
Question and Answer Session

Submit your questions using the chat function. It can be found at the **left hand side** of the window.
ENTERING A NEW ERA
VIRTUAL EVENT

October 8-9, 2020
#NORDSUMMIT | nordsummit.org

Rare Diseases and Orphan Products Breakthrough Summit®
Upcoming Webinar

Impact of the COVID-19 Pandemic on the Rare Disease Community
August 4th, 2:00pm EDT

Pamela Gavin
Chief Strategy Officer
NORD

Vanessa Boulanger, MPH
Director of Research
NORD

Jeffrey Keefer, MD
VP, Medical Strategy
Head of Pediatric
and Rare Disease
IQVIA

Ali Smyth, PhD
Strategy Director Pediatric
and Rare Disease Center
of Excellence
IQVIA

Murray Aitken
SVP & Executive Director
Institute for Human
Data Science
IQVIA

IQVIA

Register: https://bit.ly/3g1kKDe

NORD
National Organization for Rare Disorders

rarediseases.org
Speakers

Michelle Campbell, PhD
Sr. Clinical Analyst, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, FDA/CDER

Jane Larkindale, DPhil
Executive Director, Rare Disease Cures Accelerator-Data and Analytics Platform and Duchenne Regulatory Science Consortium, C-Path

Vanessa Boulanger, MSc
Director of Research, National Organization for Rare Disorders

U.S. Food & Drug Administration
Critical Path Institute
NORD - National Organization for Rare Disorders
Rare Disease Cures Accelerator-Data and Analytics Platform

Michelle Campbell, PhD
Office of Neuroscience
CDER
# Key Activities Presenting Areas of Challenge

<table>
<thead>
<tr>
<th>Characterization of Disease</th>
<th>Getting Patient Perspectives on their Disease and Treatment</th>
<th>Clinical Study of New Treatments</th>
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<tbody>
<tr>
<td>• What is known about the disease?</td>
<td>• What disease impacts matter most to patients?</td>
<td>• Is the investigational drug available in a form that can be administered?</td>
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<td>• Are there well-defined lab tests—to diagnose the disease?</td>
<td>• What is the landscape of currently available treatments?</td>
<td>• Pre-clinical safety testing done to inform assessment of safety in humans?</td>
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<td>• What is the natural history of the disease?</td>
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<td>• A study design specified?</td>
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<td>• What causes the disease (pathogenesis)?</td>
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<td>• A study protocol?</td>
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<td>• IRB review and approval?</td>
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<td>• IND submitted for FDA review?</td>
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<td>• Plan for patient enrollment?</td>
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<td>• Patient access to the trial site?</td>
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<td>• Plan for study data collection?</td>
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Need for a “Rare Disease Cures Accelerator”

- Adopting a cooperative research approach to accelerate the move from bench to bedside for rare disease cures.
- A “Rare Disease Cures Accelerator” would provide the infrastructure for a cooperative scientific approach to clinical trials readiness in rare diseases.

- **Key components include:**
  - Centralized standardized infrastructure to support and accelerate rare disease characterization
  - Standard core sets of COAs measuring impacts that matter most to patients, ideally applicable to more than one rare disease
  - Global rare disease clinical trials network
Rare Disease Cures Accelerator-Data and Analytics Platform

Through FDA’s Rare Disease Cures Accelerator, we funded the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP)

The RDCA-DAP is a neutral, independent data collaboration and analytics hub that provides a centralized and standardized infrastructure to support and accelerate rare disease characterization, with the goal of accelerating therapy development across rare diseases

Partners: CRITICAL PATH INSTITUTE, NORD®

https://c-path.org/programs/rdca-dap/
The primary objective:

- Establish a data management and data repository system
- Which will house data from existing and planned rare disease clinical studies and trials
- Data to be contributed from different organizations.
Thank you
Rare Disease Cures Accelerator – Data and Analytics Platform

Why should I contribute data?
What does RDCA-DAP do?

**INPUT**
- Clinical Trial Data
- Natural History Data
- Patient Registry
- Clinical Data
- Other Data

**C-PATH PROCESS**
- RDCA-DAP contact with custodian, negotiate data sharing agreement
- Data transfer
- Data exploration and mapping

**C-PATH OUTPUT**
- Feedback to custodians on data gaps, gaps in standardization, other enhancements to collection
- Standardized data returned to custodians
- Analysis and tools available
- Larger dataset available

Tools and analysis developed with data made available
- Data available per custodian’s direction
Why share data with RDCA-DAP?

Your data is powerful, valuable and can be used to benefit not only your disease state, but the entire rare community!

RDCA-DAP is different to other databases because:
• The data is patient-level (allows us to understand the variability of disease)
• Integrates datasets (bigger datasets to look at)
• Across all rare diseases (learnings across diseases)
• We let each contributor tell us how widely we can share their data
What use is data anyway?

Traditional Drug Development Approach

Reliance on limited information and experience

Data and Quantitative Model Based Drug Development Approach

Using all available information:

- Integrated global datasets including relevant populations and endpoints
- Quantitative models of disease progression, patient population and endpoint behavior
Data and models can improve clinical trials

Traditional – all patients

Model- informed
THANK YOU!

Jane Larkindale
jlarkindale@c-path.org
How do I get involved?
Data is key!

Vanessa Boulanger, MSc
Director of Research, NORD
There are over 7,000 rare diseases. More than 90% of rare diseases are without an FDA-approved treatment. Many rare diseases aren’t being studied.
RDCA-DAP Benefits

- **360° view** of disease characterization and natural history
- **Accelerate understanding** of conditions and commercial/research interest; inform the design of trials
- **Encourage greater representativeness** in study samples - steps toward more equitable and inclusive study designs
- **Opportunity** for cross-disease discovery
- **Efficient, effective** use of resources
Data is key!

RARE DISEASE PATIENT REGISTRIES UNLOCK CURES

Joining a registry opens the door for more and better research.

Adding your data can make clinical trials shorter and less expensive, delivering therapies to the community faster.

Your unique experience adds to what we know about a disease. The more patients share their experiences, the better we understand the full spectrum of the disease.

Researchers, health care providers and the FDA need help to understand different diseases. Your data is key for these decision makers.

Learn more about how you can contribute data at: rarediseases.org/RDCA-DAP

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https://rarediseases.org/rdca-dap/resources/
Data is key!

What is a Registry?

https://rarediseases.org/iamrare-registry-program/
Rare Disease Cures Accelerator-Data and Analytics Platform

RDCA-DAP is an FDA-funded initiative that will provide a centralized and standardized infrastructure to support and accelerate rare disease characterization with the goal of accelerating therapy development.

Accelerating Cures and Treatments

Over 350 million people in the world have a rare disease, but only about 10% of rare diseases have an FDA-approved treatment. Drug development can be extremely challenging for rare diseases because of the often small patient populations and a limited understanding of how these diseases manifest and progress. If we can bring together the experience of patients, along with clinical data, we can pave a cleaner path for producing new treatments with better defined and more meaningful outcomes and improve our understanding of the progression of a disease. This would accelerate clinical development, make it less expensive, and encourage new companies to develop rare disease drugs.

The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) is an integrated database and analytics hub that is designed to be used in building novel tools to accelerate drug development across rare diseases. It is being developed by the Critical Path Institute (C-Path) and NORD through a collaborative grant from the FDA (Critical Path Public-Private Partnerships Grant Number U01FD005330 from the US Food and Drug Administration).
Are you ready to join this collective effort to find tomorrow’s treatments today?

- Participate in a natural history study, registry or clinical trial
- Ask the data collector if the consent form allows for data to be shared
- Tell the data collector about RDCA-DAP and encourage them to share the data

If your rare disease doesn’t have an active patient registry, visit [rarediseases.org/iamrare-registry-program](http://rarediseases.org/iamrare-registry-program) to learn more about NORD’s platform.

Encourage sharing of your data by suggesting your study sponsor visit [c-path.org/programs(rdca-dap)](http://c-path.org/programs(rdca-dap)) or email [rdcadap@c-path.org](mailto:rdcadap@c-path.org) to start a conversation.
THANK YOU!

Vanessa Boulanger: vboulanger@rarediseases.org
RDCA-DAP Virtual Workshop

SAVE THE DATE
October 19, 2020

Registration is NOW open: https://bit.ly/RDCADAPWORKSHOP2020
Questions?
Please enter your questions in chat.

Michelle Campbell, PhD
Sr. Clinical Analyst, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, FDA/CDER

Jane Larkindale, DPhil
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Email additional questions to research-programs@rarediseases.org
To contribute data contact: rdcadap@c-path.org
Thank you.

Funding for this webinar was made possible, in part, by the Food and Drug Administration through grant U18 FD 005320. Views expressed in written materials or publications and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services; nor does any mention of trade names, commercial practices, or organization imply endorsement by the United States Government.

Alone we are rare. Together we are strong.