Overview of NORD’s Registry Program

Vanessa Boulanger, Director of Research
RDCA-DAP Launch Meeting September 17, 2019
Overview

• Brief Introduction to NORD
• IAMRARE™ Registry Program: History, Development, and Growth
• Partnerships and Collaborative Research Models
• Real-World Case Studies from NORD’s Registry Community
• Value and Impact of RDCA-DAP in Context
Introduction to NORD
NORD, an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families.

We do this by supporting patients and organizations, accelerating research, providing education, disseminating information and driving public policy.
Intersecting Programmatic Areas

148
Meetings attended on Capitol Hill in 2018

280
Member Organizations

150K+
Phone calls answered annually from patients and caregivers

12.7 Million
Website visits annually in 2018 to NORD’s online rare disease reports

800+
attendees at NORD’s Rare Summit in 2018
Intersecting Programmatic Areas

- Access Support
- Drive Innovation
- Gain Knowledge
- Create Change
- Join Our Rare Family
IAMRARE™ Registry Program: History, Development, and Growth
History of the IAMRARE™ Program

- 2014: First Registry Launches on NORD Platform
- 2015: 10,000 Survey Submissions
- 2016: Launch of First Registries Supported by NORD-FDA Cooperative Agreement
- 2017: NORD-FDA Awardee Selections
- 2018: Registry Community Meetings
- 2019: New Models of Engagement

Key Events:
- 2014: RDCA-DAP Launch Meeting
- 2016: NORD-FDA Workshop
- 2017: 5th Anniversary of Program

Timeline:
- 2014: NORD-FDA Cooperative Agreement is announced
- 2015: Registry Community Meetings
- 2016: New Models of Engagement
- 2017: NORD-FDA Workshop
- 2018: 5th Anniversary of Program
- 2019: RDCA-DAP Launch Meeting
IAMRARE™ Program

- IAMRARE™ Platform
  - Training, User Guides
- Study Resources
  - Core Survey Library
  - Custom Survey Support
  - Templates (e.g. consent, marketing)
  - Centralized IRB Partnership
- IAMRARE™ Community
  - Portal, Meetings, Webinars, Newsletters, Videos, Peer-to-peer Discovery
Design & Build
Training
Optimize & Deploy
Ongoing Support
IAMRARE™ Program

Are you an individual or do you know an individual diagnosed with a rare disorder?

You can help us find out more about rare diseases

Why it’s important that you participate

Make an impact on rare disease research in 3 easy steps!

1. Create an Account
2. Add Participants
3. Take Surveys

For patients, parents, guardians, caregivers and legal representatives

See how the Power of Patients is helping to inform our understanding of rare diseases

IAMRARE™ Registry Program

powered by NORD

rarediseases.org
IAMRARE™ Program

Thoughts from the Front Lines of Rare Disease Research

There are nearly 7,000 rare diseases, some of which affect just a few people. Yet, the combined impact of these conditions together affects 25 million people in the United States alone with rare diseases. On this Rare Disease Day, I'd like to challenge each of you to think about how we can raise the visibility of individuals living with rare diseases, as well as the research needed to help them.

I’d like to introduce you to Dr. Brian Harris, who is using his own gift of storytelling to share the experiences of people with rare diseases. He is the author of ‘Front Lines: Stories of Hope, Healing, and Dignity from Those Who Stare Into the Eyes of the Unseen.’ Through his personal voice, he tells stories of the individuals who are affected by rare diseases and the challenges they face daily.

The need for a next-generation public health response to rare diseases.

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IAMRARE™ Program

Since 2014 Launch:

40+ Registry Partners
10,000+ Participants
80,000+ Survey Submissions

Total Number of IAMRARE™ Survey Submissions

Number of Survey Submissions

Q1 Q2 Q3 Q4


rarediseases.org
NORD Partnership Models

- Industry & Academia
- NORD
- Community Organization
NORD Partnership Models

- MAIN STUDY
  - SUB-STUDY A
    - SUB-STUDY
    - MAIN STUDY
    - SUB-STUDY
    - MAIN STUDY
  - SUB-STUDY B
    - SUB-STUDY
    - MAIN STUDY
    - SUB-STUDY
    - MAIN STUDY
  - SUB-STUDY C
    - SUB-STUDY
    - MAIN STUDY
    - SUB-STUDY
    - MAIN STUDY

registryplatform
Real-World Case Studies from NORD’s Registry Community
Case Studies

SYNGAP1 heterozygosity disrupts sensory processing by reducing touch-related activity within somatosensory cortex circuits

Clayton D. Michaelson1, Emin D. Ozkan1, Massimiliano Aceti2, Sabyasachi Maity3, Nerea Llamosas1, Monica Weldon4, Giulia Mizrahi5, Thomas Vaissiere6, Michael A. Gaffield7, Jason M. Christie8, J. Lloyd Holder Jr.9, Courtney A. Miller10 and Gavin Rumbaugh11.

The Registry Offers a Platform for Patients to Share Their Stories via Surveys

Partnerships to Conduct Research (PaCR) within PCORnet
The Foundation for Prader-Willi Research (FPWR) and Zafgen are pleased to announce that enrollment is now open for PATH for PWS, a natural history study intended to better understand serious medical events in Prader-Willi syndrome (PWS) and evaluate how PWS-related behaviors change over time. The data from this study is intended to inform the development and clinical trial design of potential new treatments for PWS. Those interested in participating can find more information about the study and how to enroll at www.PATHforPWS.com.

Enrollment is now open for the four-year study using the Global PWS Registry, which is powered by the National Organization for Rare Disorders’ (NORD) IAMRARE™ Registry Program. To be eligible for the study, participants must have a confirmed diagnosis of PWS, be at least 5 years of age, live in the United States, Canada or Australia, and be enrolled or willing to enroll in the Global PWS Registry. The primary caregiver of the enrolled person with PWS must have access to the internet to enter study data and consent to being contacted by registry staff.
Value and Impact of RDCA-DAP in Context
Value and Impact of RDCA-DAP

- Transformative collaboration
- Leveraging capabilities and expertise
- Development of new tool(s) and data optimization to accelerate discovery and therapeutic product development
- Flexibility to design solutions to overcome well-known data challenges
- Effective use of resources
- Innovative technologies that drive efficiencies and reduce costs
- Standard-setting and evaluation of measure sets*
- Convening a ready-made collaborative global network to support clinical trials*
RDCA-DAP in Context

**NORD Registry-Now**
- Centralized disease-neutral platform
- Prospective natural history data collection
- Consolidation of stakeholder efforts
- Community-driven

**NORD Registry-Next**
- Evaluate COAs for use across conditions
- Systematic collection of data
- Set standards for recruitment, retention, and engagement
- Education and training

**NORD Registry-Near Future**
- Support natural history study designs that can serve as external control
- In partnership, demonstrate successful usability of natural history for controls
- Define global rare disease data standards
- Return of value to community
- Consolidated source for registry data integration for RDCA-DAP
• Our rare disease research partnerships reflect authentic engagement and sustained collaboration.

• NORD is the primary initiation point for patient organizations interested in participating in the RDCA-DAP.

• Our model can keep data proprietary and separate, but the community together.

• With our partners at C-Path and FDA we are designing solutions to bridge stakeholder needs and deliver impact.

• We hope you will join us as registry partners, data partners, and research project collaborators!
Questions?

Contact: research@rarediseases.org
Thank you.