RDAC Stakeholder Webinar

November 16, 2022
Thank You To Our Sponsors!
OPENING REMARKS

Peter L. Saltonstall
NORD President and Chief Executive Officer
• Rare diseases are defined as a disease or condition that affects less than 200,000 Americans

• According to the National Institutes of Health (NIH), there are more than 7000 rare diseases, 2/3 of which have a genetic component to them.

• Approximately 90% of rare diseases do not have an FDA approved treatment.
RARE DISEASE ADVISORY COUNCILS (RDACs)

- A diverse body representing the rare disease community to advise state government on their common obstacles
- Opportunity for government officials and the rare disease community to partner to develop resources necessary to prevent and address barriers in a strategic way
PROJECT RDAC

• Optimize existing RDACs and increase the number of RDACs across the country
• NORD acts as a convener, providing opportunities for the RDACs to collaborate with each other
• Create educational resources to guide RDACs at every step of their journey
  ➢ Webinars
  ➢ Toolkits
  ➢ One Pagers
RECRUITING MEMBERS FOR THE RDAC

Patient Organizations
Non-profits that operate in the state

Health Care Partners
Doctors, Nurses, Geneticists, Hospital Administrators

Community Members
Patients, Advocates, Caregivers

Industry Partners
Pharmaceutical, Biotech Companies

State Agencies
Department of Health, Department of Insurance

Educational Institutions
Universities, Academic Research Institutions
RDAC UPDATES

RDACs Signed into Law in 2022:
- Colorado
- Connecticut
- Georgia
- Maine
- New York

RDAC 2023 States of Interest:
- Arizona
- Michigan
- Arkansas
- Oregon
- California
- Washington
- Indiana
- Texas
- Maryland
The MN RDAC is currently working to launch a Project ECHO focused on increasing clinician expertise in transitioning youth with complex health needs from pediatric to adult care.

The PA RDAC hosted their first Stakeholder Summit which focused on gene therapy and newborn screening.

Figure 1. ECHO model. Retrieved from Project ECHO - Center for Older Adults (unthsc.edu)
NH is working on developing legislation introduced to improve coverage for medical foods in the state.

NC continues to advocate for expanding the number of conditions that their state's newborn screening program. In 2021, NC started screening for 4 additional conditions.

TN created a step therapy reform Workgroup. Some RDAC members were involved in advocating for step therapy reform legislation that was signed into law in May.

VA and NH are planning to pursue telehealth legislation in 2023.
8th Annual State Report Card

- Medicaid Financial Eligibility
- Medical Nutrition
- Newborn Screening
- Prescription Drug Out of Pocket Costs
- Protecting Patients in State Medicaid Programs/State-Regulated Insurance
- Rare Disease Advisory Councils
- Step Therapy (Fail First)
- Telehealth
Panel Discussion 1:
RDAC Coalition Leaders
PANEL DISCUSSION

Moderator: Alyss Patel  
State Policy Manager, Western Region  
NORD

Beth Nguyen  
Georgia Rare Action Network Volunteer  
State Ambassador/Georgia RDAC Chair

Nick Kirchof  
Former Colorado Rare Action Network Volunteer State Ambassador

Libby Airhart  
Louisiana Rare Action Network Volunteer  
State Ambassador/Louisiana RDAC Chair
Panel Discussion 2:
A Look Ahead for RDACs
PANEL DISCUSSION

Moderator: Annissa Reed  
*State Policy Manager, Eastern Region*  
*NORD*

Patrick Flume, M.D.  
*Medical University of South Carolina (MUSC)/South Carolina RDAC Chair*

Nadia Falah, M.D.  
*Duke University/West Virginia RDAC Member*

Matt Pearl  
*Former Utah Rare Action Network Volunteer*  
*State Ambassador/Utah RDAC Member*
YEARS 3 OF PROJECT RDAC

- Continue utilizing NORD’s State Report Card to assist RDACs with identifying policy issues in their state
- Develop a toolkit to assist RDACs in policy engagement in their state
- Expand NORD's Project RDAC website by including information about upcoming RDAC events, upcoming meetings, and links to RDAC’s websites
- Continue hosting webinars for RDAC members and stakeholders throughout the year
- Provide ongoing technical assistance and support to new and existing RDACs
- Continue building diverse coalitions to advocate for RDAC legislation in states of interest
The purpose of the Taskforce is to build a network of effective and engaged advocates, working to improve the lives of patients with rare disorders through advocacy.

The Taskforce will:

✓ **Raise the profile** of issues that impact rare disease patients

✓ **Connect the rare community to policymakers** through story-sharing, legislator engagement and other advocacy activities

✓ **Develop advocacy skills in new volunteers**
HOW IT WORKS!

- We will hold 4 National Advocacy Trainings a year (virtually)
- We will create 4 Regional Taskforces
- Each Region will be led by 2 Volunteer Co-Chairs
- Volunteer Co-Chairs will work with NORD to host 5 meetings a year
HOW IT WORKS!

Through the Taskforce, advocates can:

• Take action virtually, and in-person on important issues
• Share their story with elected officials
• Learn more about policy and advocacy
• Join coalitions on important issues impacting patients
• Submit testimony and letters to the editors

...and more
Questions from the Audience
RESOURCES

NORD’s State Report Card
https://rarediseases.org/nord-state-report-card/

NORD as a Marketing Tool
rarediseases.org/projectrdac/project-rdac-events/

NORD Toolkits/Webinars
https://rarediseases.org/projectrdac/project-rdac-resources

Project RDAC

Rare Disease Advisory Councils (RDACs)

Overview  |  RDAC by State  |  Events  |  FAQs  |  Resources

Project RDAC – Events

Rare Disease Advisory Council Upcoming Coalition Meetings

NORD is working with rare advocacy groups throughout the country to build diverse coalitions to help implement Rare Disease Advisory Councils (RDACs) in their state. The first step in developing an RDAC is to bring together rare disease advocates to discuss unique strategies that their rare disease community faces in their state. Coalition meetings are a platform that allows advocates to provide valuable input to guide ongoing efforts to introduce and pass RDAC legislation across the country. Please see the registration links below to join an upcoming event in your state.

Upcoming Events

RDAC Implementation

Help an RDAC have a positive impact on the rare disease community? Build on your advocacy by helping to create a strong RDAC that effectively works to elevate the voice of the rare community. Get involved!

RDAC Stakeholders Meeting 2021
Recorded: November 16, 2021

Recording: RDAC Stakeholders Meeting
Recorded: December 16, 2020

Toolkit: Implementing a Rare Disease Advisory Council in Your State
*Requires form submission for access

Webinar Recording: Your State Just Enacted an RDAC – Now What?
Recorded: June 10, 2021
NORD® RARE ACTION NETWORK®

JOIN TODAY!

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- CSL Behring
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- Horizon
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- Takeda
- Traverne
- Pfizer
- Otsuka

NORD
Thank you.

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