CREATING A RARE DISEASE ADVISORY COUNCIL IN YOUR STATE

NORD®
National Organization
for Rare Disorders

What's Inside

WELCOME

Dear Fellow Rare Disease Advocate,

Thank you for your interest in Rare Disease Advisory Councils (RDACs)! The National Organization for Rare Disorders (NORD) is proud to launch Project RDAC—an initiative designed to optimize existing RDACs and increase the number of effective RDACs across the country. As part of the Project RDAC initiative, NORD will develop and distribute resources to help Rare Disease Advisory Councils at every step of their journey to ensure they are impactful and high-functioning platforms for the rare disease community.

In this first toolkit, NORD policy staff and RDAC leaders have come together to share best practices and advice from existing councils. Additionally, you will also find a step-by-step guide to building a coalition to help advance an RDAC in your state. From our experience, these first steps of building out your coalition and determining the purpose of the council before exploring legislation are critical to the future success of your RDAC.

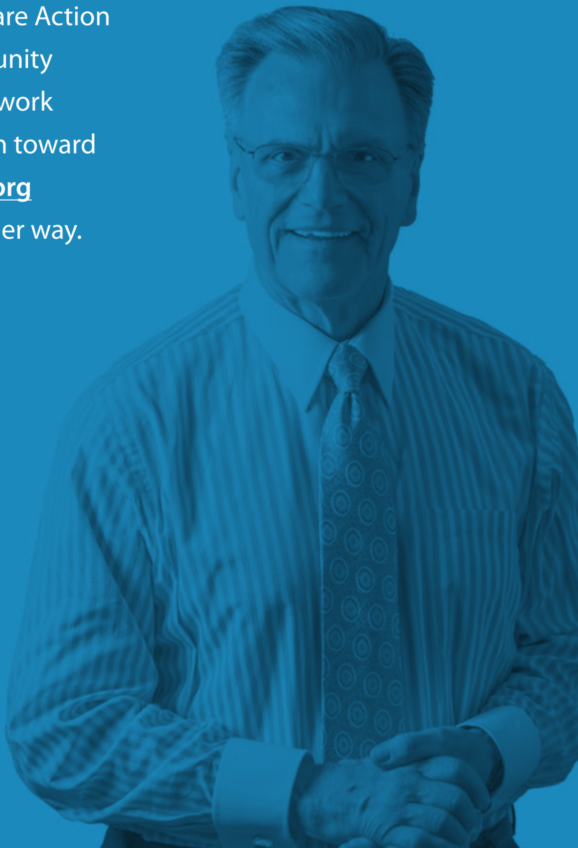
We hope you will find this toolkit helpful as you work with NORD, its Rare Action Network® of Ambassadors and volunteers, and the rare disease community to establish an RDAC in your state. NORD's policy staff stands ready to work with you to help make this goal a reality. Before setting out on the path toward building a successful RDAC, please contact us at **RDAC@rarediseases.org** so that you can be appropriately engaged with any efforts already under way.

Thank you for all you do!

Alone we are rare. Together we are strong.®

Sincerely,

Peter L. Saltonstall
President and Chief Executive Officer
National Organization for Rare Disorders



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OVERVIEW OF RARE DISEASE ADVISORY COUNCILS

What is a Rare Disease Advisory Council?

A Rare Disease Advisory Council, or RDAC, is an advisory body that gives the rare disease community a stronger voice in state government. RDACs address the needs of the rare disease community by providing stakeholders an opportunity to make formal recommendations to state leaders about the most important issues they face, including the need for increased research and awareness, knowledgeable health care providers, and timely access to affordable diagnostics, treatments and cures.

The first RDAC was created in North Carolina in 2015 by patients, caregivers, families and providers. Since then, advocates in several states have created RDACs to better represent their community and articulate needs to state elected officials and decision makers. Each RDAC is different and tailored to the state's needs, but overall, they have the same goal of increasing the rare disease community's voice in state government.

The main differences between existing RDACs include:

1. **Number of members on the council** - North Carolina has the smallest number of members at 6 and Pennsylvania, with 24 members, has the largest council to date.
2. **Membership diversity** - Each council includes a variety of stakeholders from health departments, elected legislative officials (or their designees), academic researchers, health care providers, health insurers, drug manufacturers, patient advocacy organizations, patients and caregivers.
3. **Where the council is housed** - Each statute outlines where the council is housed or is administratively attached. Examples so far include: state departments of health, universities, non-profits, or state Medicaid programs.
4. **Funding** - Most councils do not have funding from state governments; however, many councils include language in their law that allows them to seek outside funding and donations.
5. **Duties and accountability** - Each council has different directives in their laws about what key rare disease policies to work on and to what extent they are required to report on their findings and recommendations.

Each RDAC is different and is in various stages of implementation. To learn more about a specific state's RDAC or to get more involved in an ongoing effort to create to create an RDAC, please visit https://rarediseases.org/rdac-overview/ or contact us at RDAC@rarediseases.org.

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LIFECYCLE OF AN RDAC

LIFECYCLE OF AN RDAC

This is just an example of how an RDAC might be implemented. This process will inevitably vary depending on the state. The purpose of this toolkit is to outline the initial coalition building phase to start an RDAC in your state.

START

Coalition Building Phase for a Rare Disease Advocate (covered in this toolkit)

Idea to start an RDAC

Reach out to NORD's policy staff at RDAC@rarediseases.org for a status update on RDAC efforts in your state (Please note: In states where RDAC development efforts are already underway, NORD can help connect you to existing RDACs or coalitions)

Build a coalition (see page 6 for more info)

Host initial coalition meeting to provide overview of RDAC (see page 8 for more info)

Host second coalition meeting to discuss needs of the rare disease community in your state

Use section 5 (Page 10): "Identifying your state's needs" to help fill in the NORD model legislation

Legislative Phase (to be covered in a future NORD RDAC toolkit)

Work with the coalition to fill in the draft model legislation to meet your state's needs

Identify members of the state legislature who would be interested in supporting and introducing the legislation (i.e. "sponsoring" the legislation)

Meet with legislative sponsors and confirm his or her support

Assist the legislative sponsors with introduction of the RDAC legislation and work to address any questions/concerns they may have with the draft legislation

Closely monitor the legislative process and stay in touch with the sponsors throughout to see if there are ways you can help

LEGISLATIVE PROCESS

1. RDAC legislation is introduced in state legislature
2. Committee consideration/hearing and vote
3. Report bill out of committee
4. Floor consideration and vote
5. Resolve differences between two chambers
6. Governor's desk for signature
7. RDAC enacted!

Implementation Phase (to be covered in a future NORD RDAC toolkit)

RDAC members appointed

First RDAC meeting held

Workplan based on RDAC duties and requirements developed

Recommendations developed

Recommendations compiled into a report

Recommendations submitted to state government decisionmakers!

FINISH

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BEST PRACTICES FOR THE EARLY STAGES OF STARTING AN RDAC FROM RDAC LEADERS

Identify your council's goal and purpose first. This will allow you to hit the ground running.

SHARON - NC

Be flexible as a coalition to meet your state's current needs!

REP MARSH - NH

Be patient. Not all change is going to happen instantly.

MARIE - PA

It's important to learn about other advocates' interests and about their communication styles.

KRISTA - NH

Reach out to RDACs in other states for ideas.

TERRY JO - TN

Find a professional lobbyist (children's hospital, drug manufacturers, etc. …) who is willing to donate their time. A lobbyist can help provide details on the legislative process in your state.

ERICA - MN

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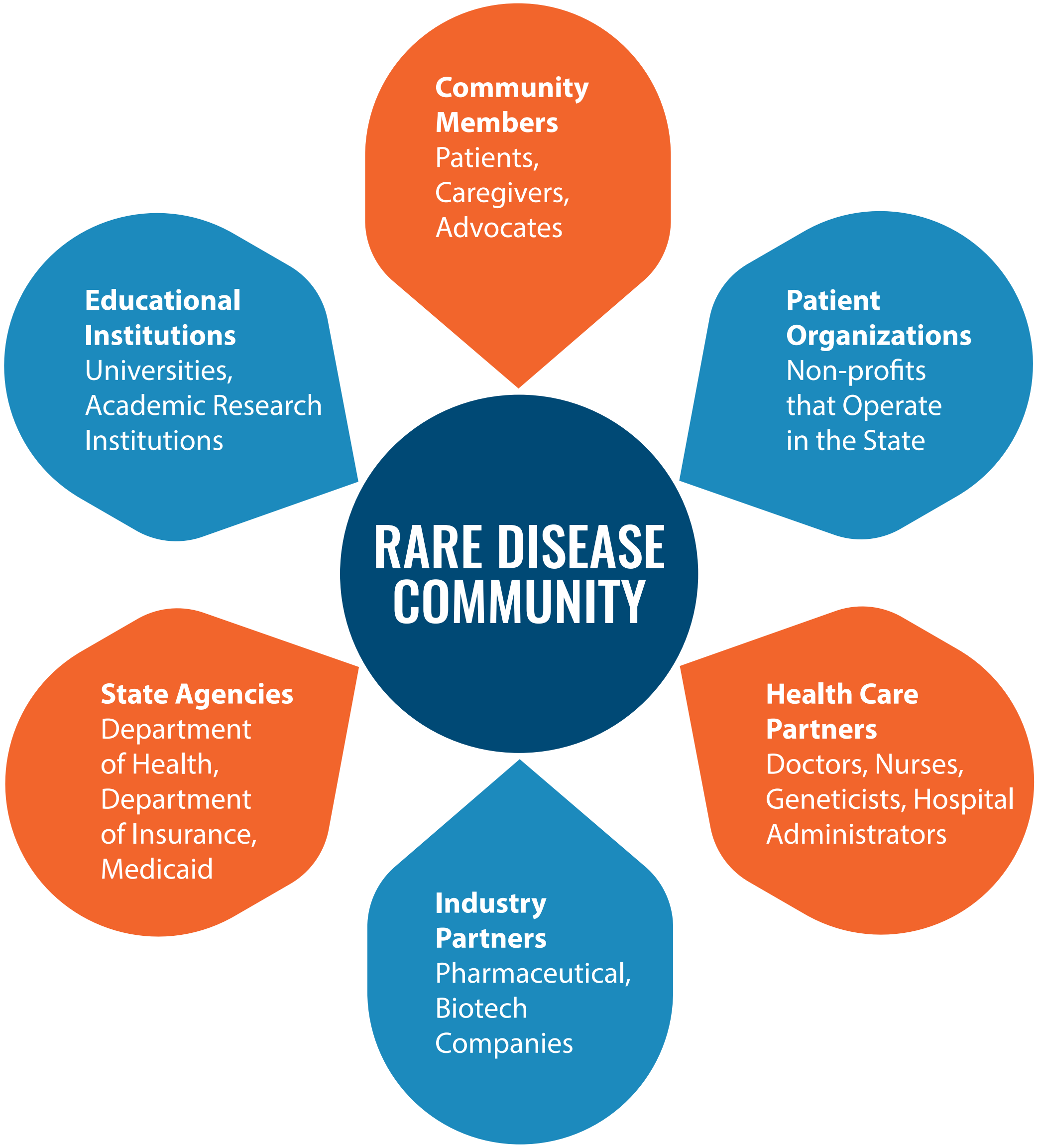
A GUIDE TO COALITION BUILDING

RDACs are more successful when there is a diverse coalition helping from the start. Therefore, when building out an RDAC coalition, it is important to have representation from a wide variety of stakeholders in the rare disease community. Taking this approach will ensure each community stakeholder's voice is heard and provides the best opportunity to shape the RDAC to be inclusive of your state's specific needs.

Step 1: Recruiting Members for the RDAC Coalition

1. See below for a graphic of suggested coalition members that will help meet the goal of ensuring broad representation. In addition, NORD's policy staff has a coalition planning worksheet that can guide your outreach. Please reach out to RDAC@rarediseases.org to request the coalition planning worksheet.



RARE DISEASE COMMUNITY

Community Members
Patients, Caregivers, Advocates

Patient Organizations
Non-profits that Operate in the State

Health Care Partners
Doctors, Nurses, Geneticists, Hospital Administrators

Industry Partners
Pharmaceutical, Biotech Companies

State Agencies
Department of Health, Department of Insurance, Medicaid

Educational Institutions
Universities, Academic Research Institutions

Step 2: Inviting People and Organizations to Join the RDAC Coalition

1. After identifying a wide array of individuals and organizations for the RDAC coalition, use our template email to invite them to an introductory meeting. NORD's policy staff is happy to assist with setting up a virtual platform for your introductory coalition meeting and can assist with communicating out the opportunity to the Rare Action Network® (RAN®) members in your state. Please reach out at RDAC@rarediseases.org to request the template email.
2. Sharing RDAC efforts on social media is a good way to maximize your reach and allows others to be part of the coalition building process. NORD has a social media toolkit that includes some sample social posts. Please reach out at RDAC@rarediseases.org to request it.
3. Ask the individuals who join the coalition to reach out to their contacts to see who might be interested in becoming coalition members.

Step 3: Coordination of Coalition

1. Share information about the new coalition and its mission through social media and your organization's website. Consider creating a press release to further your cause and make people aware of your efforts.
2. Create a mailing list of coalition members. Encourage them to utilize this to share resources, provide updates and learn about potential opportunities for the coalition.
3. Send out a Doodle poll or survey that provides different days and times for the first coalition meeting. If you choose to use a survey, you might also want to ask the coalition members what they would like to see covered in the first coalition meeting and their interests in the project.
4. Give the coalition members about one week to respond. It is helpful to give people a reminder a few days before the requested response date.

Step 4: Preparing for the First Coalition Meeting

1. Schedule the first meeting based on the survey results. Try to schedule the meeting 1–2 days after the participants submit their responses so they can be sure to mark their calendars to avoid potential scheduling conflicts.
2. Send the coalition members a calendar invite with the meeting link and/or call-in information. NORD can supply a Zoom or Microsoft Teams link.
3. Send an agenda 4–5 days before the meeting, attaching the meeting agenda to the email invitation. Please reach out at RDAC@rarediseases.org to request a sample agenda to help prepare for your meeting.
4. Send the participants a reminder about the meeting one day before via email.

5. A PowerPoint presentation is a helpful visual to guide your first meeting. NORD has a PowerPoint template that can help structure your first coalition meeting. It is customizable, but as a starting point includes an overview of RDACs, the status of them across the country, and room to add meeting-specific information. Please reach out at RDAC@rarediseases.org to request the PowerPoint template.

Step 5: Facilitating the Meeting

1. Start the call 5–10 minutes before the meeting.
2. If there are less than 20 individuals on the call, have them introduce themselves, identify their organization (if applicable), and ask them to share why they are interested in assisting with building the RDAC.
3. Try to stick to the agenda but encourage an open and natural conversation. If you're concerned that the participants won't be talkative, consider asking a member ahead of time if they would be willing to speak.

Step 6: After the Meeting

1. Tell us how it went! Reach out to the policy staff at NORD to discuss the first meeting and the next steps the coalition has decided to pursue.

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IDENTIFYING YOUR STATE'S NEEDS TO CREATE AN IMPACTFUL, HIGH-FUNCTIONING RDAC

There are a variety of ways for RDAC coalitions to identify the needs of the rare disease community in the state. This needs assessment will ultimately help guide the coalition's decision on what to include in the "Duties" section of the **model bill language** and will help shape the RDAC's responsibilities. This process is also critical to determining what the RDAC will work on when it is signed into law. This section of the legislation will also help shape the direction the RDAC will take.

Model Bill Language: *Suggested bill language to incorporate best interests of the rare disease community. This model language should be tailored to meet each state's specific needs, but incorporates lessons learned and best practices from NORD's experience with RDACs. For a copy of the model legislation, please contact NORD's policy staff at* ***RDAC@rarediseases.org****.*

Through NORD's advocacy work on RDACs in recent years, it has been demonstrated that when a coalition identifies in advance what the council intends to work on, there is a smoother transition to the start of the RDAC's work after the bill is enacted. There are a lot of ways to explore identifying the needs of the rare disease community in your state. Below are a few ideas to guide this analysis in your state. Please reach out to NORD at RDAC@rarediseases.org with any questions or concerns.

Ideas for Identifying Your State's Needs

Conduct Strengths, Weaknesses, Opportunities, Threats (SWOT) Analysis

Conducting a SWOT analysis is an important method to identify current strengths, weaknesses, opportunities and threats for the rare disease community. This provides the coalition with a glimpse of what is currently in place and what can potentially be implemented to better support the community through RDAC legislation. It is important for the analysis to be tailored to your specific state, since each state's rare disease community has unique needs. NORD has a template that offeres an example of what a SWOT analysis for the rare disease community in a state might look like. Please reach out at RDAC@rarediseases.org to request the template.

Review NORD's State Policy Report Card

NORD's State Policy Report Card is a landscape analysis of policies at the state level that impact the rare disease community. It can be a useful tool to help educate you and your coalition on where your state stands on key policies that affect the rare disease community.

The issues outlined in the report card touch on several critical and relevant policy areas at the state level, but with each issue included, there are still many others that are capable of impacting the lives of rare disease patients. This is meant to be a starting point to help educate and start the RDAC coalition down the path of

discussing the specific needs in your state.

The following policies are included in the NORD State Policy Report Card.

Medicaid Eligibility: Medicaid is a joint federal and state program that provides health insurance to low-income individuals. The precise criteria for Medicaid eligibility may vary depending on the state that a patient lives in.

Medicaid Section 1115 Waivers: Section 1115 waivers enable states to waive certain Medicaid requirements in ways that would not otherwise be permitted under federal law. These waivers are intended to help states seek innovative ways to control health care costs and improve services for Medicaid beneficiaries; however some may be harmful to rare disease patients.

Out-of-Pocket Costs: The high out-of-pocket costs for some prescription drugs makes many treatments unaffordable for rare disease patients. However, some states are now implementing policies designed to protect patients.

Step Therapy: Step therapy protocols require patients to take one or more alternative medications before they can access the medicine prescribed by their provider. Patient protections against the improper use of step therapy vary widely by state.

Medical Nutrition: Many rare disorders require medical nutrition, as prescribed by a doctor and in consultation with a registered dietician, to prevent serious disability and allow for normal growth in children and adults. However, it is often expensive and coverage varies by state.

Newborn Screening: The newborn screening program helps to identify hundreds of infants that are born with rare diseases each year. Newborn screening provides early identification and, in most cases, leads to the treatment of serious diseases that could otherwise result in irreversible damage or death at a very young age. State newborn screen programs vary significantly in what conditions are screened.

Protecting Patients in State Regulated Insurance: The individual insurance market allows people, including rare disease patients, to purchase health insurance through their state's marketplace. In recent years, some states have taken action to strengthen or destabilize their marketplaces that affect the overall quality and affordability of health care.

More background and the full report can be found at:

https://rareaction.org/resources-for-advocates/nordreport/

Additional potential policy areas of focus:

- **Telehealth:** Telehealth allows patients and physicians to exchange health information without being in the same room. The COVID-19 pandemic has significantly expanded the utilization of telehealth services. Both the federal government and states have the power to regulate aspects of telehealth, including telehealth licensure requirements, reimbursement rates and eligible services. More information about your state's telehealth laws can be found here.
- **Surprise Medical Billing:** Surprise medical billing is when a patient receives an unexpected and high bill for emergency room or other hospital visits, because a physician is out-of-network even though the hospital is in-network. Read more and see how your state is doing here.

To stay up to date and learn more about NORD's work on any of the above priority issues, please check out our policy statements page or contact us at RDAC@rarediseases.org.

Hold a Public Meeting

Another important way to guide the RDAC coalition in developing the RDAC legislation is through an open discussion about needs and ideal outcomes for a future RDAC by holding a public meeting. Below are some helpful questions to help facilitate the conversation during the public meeting. These questions are examples. Feel free to brainstorm others with NORD policy staff and your coalition.

- What needs to be improved for the rare disease community?
- What policies or resources have been helpful for the rare disease community?
- What are problems that the rare disease community faces on a regular basis?
- For providers, what challenges do you encounter trying to treat your patients with rare diseases?

Conduct One-on-One Conversations with Rare Disease Stakeholders

Another way to help identify your state's needs is through conversations with influential stakeholders in the state. This can be accomplished by setting up one-on-one meetings with various organizations and influential leaders in the state to hear directly from them about what they think the RDAC should ultimately focus on or accomplish. Please work with NORD policy staff to help brainstorm who to connect with in your state that may have valuable insight.

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WHAT TO EXPECT NEXT IN THE RDAC PROCESS

After finalizing your coalition and working on identifying your state's needs and priorities for the rare disease community, it is time to begin the legislative work! Please reach out to NORD policy staff to get the legislative toolkit.

Please note that this process looks different in every state, but see below for a checklist of what to expect next:

1. Finalize RDAC model bill language.
2. Identify any existing relationships between coalition members and lawmakers in the state.
3. Identify key members on health, budget or appropriations committees; in general, legislative sponsors who serve on one of these committees of jurisdiction will have the ability to help move the RDAC faster.
4. Reach out to lawmakers who might be interested in sponsoring the bill and explain to them the importance of there being an RDAC in your state.
5. Connect with the sponsor and confirm their support.
6. Help the sponsor leading up to the introduction by addressing any questions/concerns with the legislative draft.
7. Work with bill sponsor to ensure RDAC legislation is introduced.

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CONCLUSION

Developing an RDAC is a meaningful journey that will positively impact the rare disease community. It has the potential to make an enormous difference in the lives of rare disease patients and their families. Coalition building is essential to create a strong and organized foundation for any RDAC. Thank you for taking the first step in the RDAC process and for being a rare disease advocate! Don't hesitate to reach out to NORD policy staff with any questions at RDAC@rarediseases.org.

Alone we are rare. Together we are strong.®

NORD: Fighting for the rare community every day for nearly 40 years. NORD is committed to the identification, treatment and cure of rare disorders through programs of education, advocacy, research and patient support services. NORD does not recommend or endorse any particular medical treatment but encourages patients to seek the advice of their clinicians. NORD is a registered 501(c)(3) charity organization. NRD-2118