



## **STATE RARE DISEASE ADVISORY COUNCILS:**

### **STRENGTHENING THE PATIENT VOICE IN GOVERNMENT**

In 2015, rare disease patients, families, caregivers, and providers in North Carolina came together to create the first-ever rare disease advisory council (RDAC) in order to give their community a stronger voice in government. Since then, the idea for an advisory council has spread to other advocates who have pursued similar legislation in their own state. With the support of NORD and other patient organizations, each of these communities is seeking to address barriers that prevent all of the 25 to 30 million men, women, and children living with a rare disease from obtaining proper care.

#### **States Need Help to Properly Represent All 7,000 Rare Diseases**

With over 7,000 known rare diseases, it is incredibly difficult for anyone – let alone busy state policymakers – to have an in-depth understanding of the rare community. This lack of awareness contributes to some common difficulties that rare disease patients face every day, such as delays in diagnosis, misdiagnosis, lack of treatment options, high drug costs, and limited access to medical specialists.

What is more, as research into rare diseases continues to advance and produce new breakthrough treatments for patients, state policies affecting access to these breakthroughs are often determined without consulting individual disease communities. Without greater representation in state government, legislators will be unable to adequately address these barriers to better care for the 1 in 10 of their constituents with a rare disease.

#### **Rare Disease Advisory Councils Empower the Local Patient Advocacy Community**

An RDAC can help tackle government barriers by better representing the needs of the entire rare disease community. A council will give rare disease patients, families, caregivers, and medical professionals an opportunity to make recommendations to state elected officials and other state leaders about their pressing problems. Moreover, the council's work will be accomplished with participation from diverse stakeholders that represent the entire rare community, including patients, caregivers, doctors, insurers, drug manufacturers, and researchers.

#### **How A Rare Disease Advisory Council Works**

Based on feedback from advocates in several states, NORD has identified key features of how a rare disease advisory council should complete its mission and how it should be structured.



### *Purpose and Duties*

The purpose of an RDAC is to advise and make policy recommendations to the governor, the legislature, and other relevant state institutions. This work can take many forms, such as developing new educational resources, conducting a needs assessment in the state, identifying and disseminating best practices for rare disease care, and analyzing individual state programs. In order for an RDAC to achieve its goals, it must meet regularly and consult with rare disease experts and other community stakeholders. Ultimately, an RDAC is required by law to regularly submit to the state a summary of its activities and recommendations for public consideration.

### *Membership Requirements*

First and foremost, it is critical that an RDAC include stakeholders from across the rare disease community. Every RDAC has a similar membership that includes the following:

- Patients and caregivers;
- State health department officials;
- Elected officials from both chambers;
- Academic researchers;
- Health providers (physicians, nurses, geneticists, pharmacists, etc.);
- Hospital administrators; and
- Health industry representation, including drug manufacturers and insurance companies.

Some states have chosen to expand their mandatory membership to include a much broader list of stakeholders. However, every RDAC has the authority to consult with outside experts as it deems necessary.

### **How Rare Disease Advisory Councils Have Already Had an Impact in Other States**

#### *Alabama*

Alabama enacted RDAC legislation in 2018. This council is administered by the University of Alabama at Birmingham. The Alabama RDAC met for the first time in August 2018 but plans to meet at least four times in 2019. As of November 2018, the RDAC is meeting without all mandated roles as they await committee assignments.



### *Connecticut*

Connecticut enacted a Rare Disease Task Force, its variation of an RDAC, in 2017 to study rare diseases. Since then the Task Force has met 11 times and is working towards a report to the Joint Public Health Committee to be published in January 2019. Members of the RDAC have reported that the Task Force has already helped facilitate the sharing of information across different state agencies about resources available to patients in the state. The Task Force expired at the end of 2018 and needs to be either reauthorized or turned into a permanent council.

### *Kentucky*

Kentucky enacted an RDAC in March 2019. The RDAC will be administered by an existing state nonprofit organization that acts as an advisory body. The RDAC will need to report biannually to the governor, the Cabinet for Health and Family Services, and the legislature.

### *Illinois*

Illinois enacted a Rare Disease Advisory Commission, its variation of an RDAC, in August 2016, which first started meeting in January 2017. During the first four months, the Commission met every month. The Commission now meets quarterly with other communication in between. The Commission is currently working towards enhancing its membership and publishing its first series of recommendations to benefit rare disease patients.

### *North Carolina*

North Carolina passed an RDAC in August 2015, which has met thereafter on a monthly basis. Most notably, the Council completed a newborn screening (NBS) white paper titled “Newborn Screening and Early Intervention for the Treatment of Rare Diseases.”<sup>1</sup> The report analyzes North Carolina’s current status of NBS in comparison to other states and future opportunities for improvement. The North Carolina RDAC has also effectively represented the community as organizations and patients from throughout the state have been able to participate and council leadership has prioritized outreach to all stakeholders. You can learn more about the North Carolina Rare Disease Advisory Council here: <http://www.med.unc.edu/ncrarediseasenetwork>

### *Pennsylvania*

Pennsylvania enacted RDAC legislation in 2017. Its RDAC has since held five in-person meetings and numerous conference calls. Thus far, the Secretary of Health has appointed 25 council

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<sup>1</sup> <https://baebies.com/wp-content/uploads/2018/03/Newborn-Screening-and-Early-Intervention-for-the-Treatment-of-Rare-Diseases-a-Win-Win-for-Children-with-Rare-Diseases-and-North-Carolina.pdf>



members to the RDAC, and the RDAC has released its first preliminary annual report<sup>2</sup>. The report highlights accomplishments from the first year, including the completion of an evaluation of available statistics in the community and resources for rare disease patients. Additionally, the RDAC identified six policy goals for the second year and beyond.

### **How You Can Get Involved**

Join NORD and rare disease advocates in supporting an RDAC in your state for the 1 in 10 Americans that live with a rare disease. Visit [www.rareaction.org](http://www.rareaction.org) to learn more or submit questions to [policy@rarediseases.org](mailto:policy@rarediseases.org).

### **States that are Currently Working on a Rare Disease Advisory Council Bill**

- California ([AB 1016](#))
- Massachusetts ([H 1934](#))
- Michigan (not yet filed)
- New York ([A 5762](#) / [S 4497](#))
- Texas ([S 1641](#))
- Utah ([HB 211](#))
- West Virginia ([HB 2863](#))

### **States that have enacted a Rare Disease Advisory Council Bill this Session**

- Kentucky
- Minnesota
- New Hampshire

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<sup>2</sup><https://www.health.pa.gov/topics/Documents/Diseases%20and%20Conditions/Rare%20Disease%20Task%20Force%20Report%20-%20FINAL.pdf>