



February 23, 2020

The Honorable Stephen M. Casey  
Chairman  
House Health and Human Services Committee  
625 Park Avenue  
Woonsocket, RI 02895

**Re: Opposition to House Bill 5081 – Rare Disease Medication Accessibility, Affordability, and Reinsurance Act**

Dear Chairman Casey and Members of the House Health and Human Services Committee,

On behalf of the 1-in-10 individuals in Rhode Island with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) appreciates your interest in establishing a rare disease advisory council in Rhode Island. NORD writes today, however, to express our opposition to House Bill 5081 (HB 5081) in its current form. If enacted, this legislation would address only some of the multi-faceted needs of a small part of the rare disease community in Rhode Island.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services. We believe that all individuals with a rare disease should have access to quality and affordable health care that is best suited to meet their medical needs.

Typically, the needs of rare disease patients and families do not receive adequate attention from policy makers in state government due to the nature and complexity of the more than 7,000 known rare diseases. To amplify the voice of the rare disease community in state government, NORD launched [Project RDAC](#) at the end of 2020 which aims to increase the number of rare disease advisory councils or "RDACs" across the country. At this time, 16 states have created RDACs (Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, and West Virginia) and have proven them to be a valuable resource for the rare disease community and state government officials.

Although HB 5081 may be well-intentioned, the rare disease advisory council that would be established under this bill fails to address the varied needs of the rare disease community. The council's primary responsibility as set forth in the bill would address only reinsurance for high-cost medications and therapies. Unfortunately, 90% of rare diseases do not have an FDA approved treatment for the condition, which means that a significant portion of the rare disease community would see no benefit from this particular rare disease advisory council. There are many additional functions that a rare disease advisory council can and should have to help the state's diverse rare disease community.



Based on our experience with other RDACs around the country, NORD urges you to make the following modifications to the RDAC provisions included in HB 5081:

- 1. Add additional duties to the legislation that would help improve the lives of rare disease patients in the state, not just those in need of access to high-cost medications and therapies.** A few examples of recommended duties for the RDAC to undertake from NORD's model language include: (1) consulting with experts to develop policy recommendations to improve patient access; (2) evaluate and make recommendations to improve Medicaid coverage and treatment for rare disease patients; (3) publish a list of publicly accessible resources on the state's website; and (4) establish best practices and protocols for future state of emergencies to aid rare disease patients. NORD is happy to coordinate an opportunity for the Rhode Island rare disease community to weigh in and provide additional feedback on possible duties to strengthen the council.
- 2. Increase broad representation from the rare disease community.** NORD applauds the inclusion of insurers and prescribers on the council, but other key stakeholders in the rare disease community, including patients and caregivers, are not included in the current council membership. Broad representation from the rare disease community is critical to a council's success, so NORD would recommend modifying the council membership to explicitly include rare disease patients, caregivers, other health care providers such as nurses or genetic counselors and researchers. NORD is happy to supply the full list of recommend stakeholders from our model language.

We are also concerned that the provisions creating an RDAC in Rhode Island have been incorporated into a broader bill that includes controversial provisions that could jeopardize passage. Therefore, we urge you to 1) enhance the RDAC provisions in line with our recommendations above and 2) consider splitting the RDAC bill from the other provisions to maximize the potential for enactment as soon as possible.

Unless these changes are made, we are concerned that HB 5081 would establish an RDAC in name only and will not advance the complex needs of the Rhode Island rare disease community. We are happy to assist with additional guidance and look forward to working with the Committee and Representative McNamara on an RDAC that would increase the voice of the rare disease patient in Rhode Island. If you have any questions or need further assistance, please contact Rose Gallagher at [rgallagher@rarediseases.org](mailto:rgallagher@rarediseases.org).

Sincerely,

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National Organization for Rare Disorders

Annissa Reed  
State Policy Manager, Eastern Region  
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CC: Representative Joseph McNamara