



February 9, 2024

The Honorable Susan R. Donovan, Chair
House Committee on Health and Human Services
State House
82 Smith Street
Providence, RI 02903

Re: H 7363 – An Act Relating to Health and Safety – Rare Disease Advisory Council

Dear Chair Donovan:

Thank you for this opportunity to comment on H 7363, a bill designed to create a Rare Disease Advisory Council (RDAC) in Rhode Island. We applaud the bill's sponsors for undertaking this effort to create a council that includes patients, caregivers, providers, as well as experts from Medicaid, the life sciences community, and other critical stakeholders.

I am the Executive Director of the Rare Access Action Project; a non-profit organization that advocates on behalf of patient access issues. My family has been affected by rare diseases, and because of that I have spent much of my career in the life sciences advocating for access to rare disease therapies and supporting families living with a variety of rare diseases. Because of my experiences, I am passionate about advocating for access to rare disease therapies.

Only 5% of rare diseases have a treatment approved by the Food and Drug Administration (FDA) and for one-third of individuals with a rare disease, it can take between one and five years to receive a proper diagnosis. Half of all patients diagnosed with a rare disease are children, and as many as 3 in 10 children with a rare disease will not live to see their 5th birthday. For the few that are fortunate to have a treatment, those patients face many barriers to these orphan therapies across our health care system in addition to facing a patient journey filled with misdiagnosis and lack of treatment options.

This leads to very complex issues facing rare patients and families in accessing health care, treatments, and correlated services that they need throughout their patient journey. An RDAC as outlined in H 7363 will bring together experienced providers, patients, advocates, professionals, state personnel and others to explore rare patient needs within the state which will provide Rhode Island with invaluable rare patient focused input and expertise.



Further, H 7363 learns from the experience of other states where RDAC's have been implemented without focusing on meaningful responsibilities for the RDAC and connectivity with state governments. The authors have outlined a specific remit that will allow the RDAC to quickly begin moving forward, engaging community stakeholders in the process, and lay out an agenda for the RDAC and state government to address complex rare patient issues.

For example, Medicaid personnel will be closely linked with the RDAC which will better inform reviews of new prescription medicines for coverage and access, service delivery of rare disease health care professionals, and other areas of importance to Rhode Islander care. We believe that patient knowledge, expertise, and their journey will be extremely valuable when deliberating on program coverage policies, understanding clinical science, and the intersection of their policymaking on patient lives in the state.

RDACs can be very important tools for better informed exploration of issues, challenges, and solutions. You will recall, the federal CURES Act was crafted to bring patient voices more fully into prescription drug development and regulatory approval. This has led to rare patients who are experts in their drug development, understanding clinical data, creating patient registries, and understanding FDA approvals. Yet too often when it comes to state policymaking, patients are not involved in decision-making that affects their ability to access the very therapies they have assisted shepherding forward in collaboration with academia, foundations, and life sciences companies.

H 7363 is an important step forward for rare Rhode Island and RAAP is proud to support this important legislation

Regards,

A handwritten signature in black ink, appearing to read "Michael Eging". The signature is fluid and cursive, with a large loop at the end.

Michael Eging
Executive Director
Rare Access Action Project