July 20, 2020

The Honorable Aaron Michlewitz
Chair, House Ways and Means Committee
Room 243
24 Beacon St.
Boston, MA 02133

Re: Support for H.4268 – an Act to Create a Rare Disease Advisory Council

Dear Chairman Michlewitz,

On behalf of the undersigned organizations representing individuals with chronic, acute or rare diseases in Massachusetts, we thank you for your work during COVID-19 and urge you to swiftly move House Bill 4268 (H.4268) through your committee. H.4268 establishes a rare disease advisory council (RDAC) within the state, which if passed, would help to give a voice to the 1-in-10 individuals living with a rare disease in the state of Massachusetts.

Though we are facing unprecedented times during the COVID-19 pandemic, we write to urge that H.4268 is passed this session. There is no greater time for a council like this in Massachusetts to help address issues pertaining to the rare disease community arising from this current or future crisis. Rare diseases are present across a broad spectrum of medical conditions and their needs have not subsided during this crisis. For example, across the country, 74% of rare disease patients have had a medical appointment cancelled and approximately 70% are concerned about medication and medical supply shortages.[1]

Creating an RDAC in Massachusetts will give rare disease patients a unified voice in Massachusetts state government. Additionally, the RDAC will be a valuable advisory body to elected officials and other state leaders on rare disease research, beneficiary access to treatments, and best practices for the care of those with rare diseases. It will also be tasked with creating a rare disease plan for the state and developing methods to publicize the profile of the social and economic burden of rare diseases to ensure health care providers are informed.

The RDAC represents enormous value to our organizations and the communities we serve by allowing them to directly hear from a diverse group of stakeholders interested in identifying and solving pressing challenges. In addition, the RDAC would help relieve some of the burden from the state by expeditiously delivering direct feedback, solutions, and resources with one community voice.

In creating this council, Massachusetts would join fourteen other states that have already enacted similar legislation in support of their rare disease community and proven that the RDAC can be an invaluable resource. Those states include: Alabama, Connecticut, Illinois, Kentucky,

---

[1] https://rarediseases.org/new-community-survey-from-nord-reveals-significant-impact-of-covid-19-on-americans-living-with-rare-diseases/

Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Pennsylvania, Tennessee, Utah, and West Virginia.

Once again, on behalf of the undersigned organizations, we thank you for considering H.4268 that would enable the creation of a Rare Disease Advisory Council. Please support this legislation to give a voice to Massachusetts residents living with rare diseases. For any questions, please feel free to contact Heidi Ross with the National Organization for Rare Disorders via email at hross@rarediseases.org or Julie Gortze with Rare New England via email at julie@rarenewengland.org. Thank you for your consideration.

Sincerely,

CARES Foundation, Inc.
Cure SMA
Epilepsy Foundation New England
Friedreich's Ataxia Research Alliance (FARA)
Friends of FSH Research
FSHD Society
Girls Chronically Rock
Hemophilia Federation of America
Immune Deficiency Foundation
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
Massachusetts Chapter of the Marfan Foundation
National MALS Foundation
National Organization for Rare Disorders
National Tay-Sachs & Allied Diseases Assn. (NTSAD)
Neurofibromatosis Northeast
Neuropathy Action Foundation
New England Hemophilia Association
Next Step
Our Odyssey
Pulmonary Fibrosis Foundation
Rare New England
Scleroderma Foundation
The ALS Association, Massachusetts Chapter
The Coalition Against Pediatric Pain
The Leukemia & Lymphoma Society
VHL Alliance Inc
Wishes for Elliott: Advancing SCN8A Research

CC: Members of the House Ways and Means Committee