



February 20, 2018

Tim Boyd, MPH
Director of State Policy
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Diane Franklin, Chairwoman
MO House of Representatives
201 West Capitol Avenue; Room 206-B
Jefferson City, Missouri 65101

Transmitted via email

Re: NORD Support for HB 2407

Dear Chairwoman Franklin and Members of the Children and Families Committee:

On behalf of the 1-in-10 Missouri residents with one of the nearly 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Committee for the opportunity to provide comments on HB 2407, legislation to create an Advisory Council on Rare Diseases and Personalized Medicine.

NORD and the rare disease community in Missouri are committed to helping people with rare “orphan” diseases and the organizations that serve them. Any condition affecting fewer than 200,000 Americans is considered rare. With nearly 7,000 rare diseases identified and 30 million Americans affected, the population represented by NORD is extraordinarily diverse and often not well-understood by physicians and researchers. This diversity presents immense difficulties for states trying to make appropriate coverage decisions for rare disease patients within their health programs.

For example, based on reports we receive from our patient community and member organizations, state Medicaid agencies often make coverage decisions regarding specialized therapies to treat rare diseases without input from either the public or medical experts. This often results in arbitrary restrictions that block patients from accessing prescribed treatment.

The Advisory Council on Rare Disease and Personalized Medicine created by HB 2407 would give rare disease patients a voice in the drug utilization process and provide educational resources for the review board on critical issues related to access, coverage, and the diseases themselves. The council will work as a partner with the entire HealthNet Division by providing information on both the healthcare provider-patient relationship and access issues surrounding vital life-saving medications and therapies.

In addition, NORD believes that this council can help educate stakeholders on the federal regulatory process that helps fuel the development of innovative new medicines to treat rare diseases. Organizations like NORD are aware that your state is broadly concerned about its role



in providing access to breakthrough medications approved by the Food and Drug Administration. The Advisory Council on Rare Disease and Personalized medicine would be a vital tool in strengthening understanding of these processes and their impact on health program beneficiaries.

Once again, thank you for the opportunity to comment on this legislation. Please feel free to contact me with any follow up questions. In addition, NORD's volunteer State Ambassador for Missouri, Julie Atkin, is available to provide additional insight on how these issues affect patients. Julie can be reached via email at julie.atkin@rareaction.org.

Sincerely,

A handwritten signature in black ink, appearing to read "Tim Boyd".

Tim Boyd, MPH
Director of State Policy

CC: Julie Atkin, NORD Missouri State Ambassador