MINNESOTA
RARE DISEASE ADVISORY COUNCIL

May 2nd, 2023

TO: Chair Wiklund; Sen. Morrison; Sen. Boldon; Sen Kupec; Sen. Abeler Chair Liebling; Rep. Bierman; Rep. Pinto; Rep. Keeler; Rep Schomacker

FROM: The Minnesota Rare Disease Advisory Council (RDAC)

RE: SF2995/HF2930

Chair Liebling and Chair Wiklund,
I am writing today on behalf of the Minnesota Rare Disease Advisory Council in support of SF2995/HF2930 which includes the following provisions:
SF 705/HF 988: Full funding for the Minnesota Rare Disease Advisory Council.
We appreciate that funding to establish a base operating budget was included in both the Senate and House omnibus bill versions; however, the Council respectfully urges the conference committee to adopt the funding amount appropriated in the Senate version without which the Council will face significant challenges establishing itself as a state agency as well as executing on its numerous legislative mandates. These mandates include (but are not limited to) developing resources for the medical community, identifying best practices related to treatment, diagnosis and care for rare diseases, and advising state agencies on improving polices that impact the rare disease community.
The Minnesota Rare Disease Advisory Council was created in 2019 by the Legislature and funded through FY23 to advise the State and Minnesota medical community on best practices for improving care for the rare disease community. The legislation contains a robust list of duties aimed at improving care for this historically overlooked patient population. In July 2022, the Legislature transitioned the Council out of the University of Minnesota and to an executive branch state agency with all the compliance requirements associated with this structural change. The requested appropriation would establish the agency's base budget, aligning it with its new function and allow for programs and community outreach, the development of support tools for clinicians, and the creation of public policy that increases equity and narrows the disparities in patient outcomes experienced by the rare disease patient population.
SF 1029/HF 384: Network Access Bill ensuring rare disease patient access to the appropriate specialist
Rare disease patients face a unique set of barriers to accessing healthcare over and above patients with more common diseases. One such barrier is an often-limited number of specialists with knowledge of and expertise in their rare disease. The MN Rare Disease Advisory Council conducted and published one of the largest surveys of the rare disease community in the United States. This survey found that 22% of pediatric patients and 17% of adult patient in MN reported difficulties accessing specialists due to "out-of-network" status. The network access bill would ensure that a patient with a diagnosed rare disease or a likely rare disease under a set of criteria can access the appropriate specialist without being charged an out of network fee. Currently, rare disease patients

- Visit an average of 8 providers (4 general care and 4 specialists) prior to diagnosis
- Undergo 2-3 misdiagnoses
- Wait 7-8 years to receive a diagnosis

The Council believes that this legislation will decrease the diagnostic journey for rare disease patients and facilitate their ability to access a provider who can manage their care. This is turn will decrease costs related to ineffective visits that do not result in a diagnosis and inappropriate treatment administered base on a misdiagnosis.

SF 1129/HF 1159: Drug Formulary Review Committee reform.
The rare disease community has unique characteristics that, in turn, make developing treatments uniquely challenging[1]. In 1983, Congress passed the seminal Orphan Drug Act which recognized that the unique challenges of rare disease research and development and led to the establishment of FDA approval pathways for clinical trial design that are better suited to the unique characteristics of the rare disease community. The research and approval pathway for rare diseases is often significantly different from common diseases that have much larger clinical trial patient populations, which necessitates alternative decision-making paradigms when considering patient access and formulary placement. This bill would require the addition of a physician with expertise in rare diseases as well as adding the MN Rare Disease Advisory Council to the formulary review committee's membership, giving rare disease patients a voice in decisions that impact their access to treatment.
In addition, the Council supports the modification from a 7-day notice of formulary review which potentially results in changes to the Preferred Drug list to 30 days' notice. The DFRC was established with the express purpose of increasing transparency in the process of adding or removing treatments from the preferred drug list. Patients who are dependent on treatments and physicians who are administer the treatments need sufficient notice in order to provide meaningful and thoughtful feedback to the drug formulary review committee.

The Council sincerely appreciates the work undertaken by the legislature to pass meaningful polices to improve care for Minnesotans and we look forward to assisting the State on the above priorities into the future.

Sincerely,

Erica Barnes

Erica Barnes, M.A, CCC-SLP
Executive Director

[1] https://www.fda.gov/news-events/fda-voices/cder-continues-make-rare-diseases-priority-drug-approvals-and-programming-speed-therapeutic