MINNESOTA
RARE DISEASE ADVISORY COUNCIL

May 1st, 2023

TO: Chair Klein; Sen. Seeberger; Sen. Rasmusson; Chair Stephenson; Rep. Kotyza-Witthuhn; Rep. O'Driscoll

FROM: The Minnesota Rare Disease Advisory Council (RDAC)

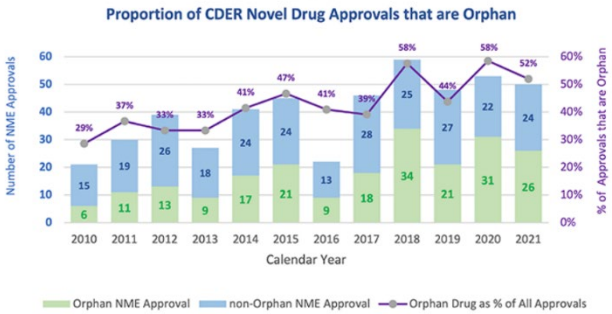
RE: SF 2744

Chair Stephenson and Chair Klein,

The Minnesota Rare Disease Advisory Council strongly supports the inclusion of language in SF 2744 that appoints the Council to the advisory committee of the Prescription Drug Affordability Board.

The rare disease patient community has historically been overlooked and marginalized in research and drug development. While there are over 7,000 rare diseases that collectively affect roughly 1 in 10 of the general population, the medical and pharmaceutical sector once considered these individual populations too small to justify investment in research and drug development. Indeed, rare disease communities were coined "orphans" due to having no home in medical research. Through grassroots advocacy, the Orphan Drug Act of 1983 shifted this mindset and incentivized investment and research in rare diseases. Despite these efforts and despite many rare diseases being chronic and life-threatening, only roughly 5% of all rare diseases currently have an FDA approved treatment.

Thankfully, in recent years, rare disease research and drug development has accelerated at an unprecedented pace, leading to scientific breakthroughs such as gene therapies that were unknown a generation ago, delivering significant clinical benefit to individuals whose devastating diseases were once outside the reach of medicine. For example, spinal muscular atrophy (SMA), a pediatric progressive neurodegenerative disease, was a decade ago untreatable and typically fatal for the children diagnosed. Currently, SMA has a number of treatments, including gene therapy, that can slow or even halt the progression of this disease if delivered early enough. Programs in our publicly funded institutions such as the Rare Disease Clinical Research Network at the National Institutes of Health (NIH), scientific discoveries in our own Minnesota research institutions, and investment from our biotechnology sector are resulting in a significant increase in treatments for rare diseases. Also, many rare disease drugs in development are novel, meaning they are not simply incremental improvements on already on the market drugs but are new medical innovations. Throughout the last decade, rare diseases have become a higher and higher percentage of novel drug approvals at the FDA as the graph below illustrates[1].



Ironically, the very fact that many of these treatments are innovative in nature poses unprecedented challenges to the way they are delivered and paid for. Government entities such as state Medicaid programs, as well as hospitals, are grappling with the reality that current reimbursement models are largely built for treatments that address chronic illnesses with costs spread out over a lifetime, versus single delivery treatments that are potentially curative with a large up-front cost[2]. In addition, 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.

In summary, the development and availability of transformative treatments for rare diseases poses both an incredible opportunity and an enormous challenge for the state of Minnesota. Ensuring that rare disease drug development and research is not restricted nor held to the same standards as common diseases while at the same time building out sustainable reimbursement models that balance value and cost should be a top priority of the state. The Minnesota Rare Disease Advisory Council was created by the legislature in 2019 largely through the grassroots efforts of rare disease patients. It is charged with the express purpose of "advising, consulting, and cooperating with…agencies of state government" and "advising on policy issues and advancing policy initiatives at the state and federal levels", making the Council a resource for the State on any issue that may impact the care and health of the rare disease community. The Minnesota Rare Disease Advisory Council looks forward to the opportunity to fulfill its statutory function of being a voice for the rare disease community through participation and engagement on the advisory committee of the Prescription Drug Affordability Board.

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

[2] https://www.nytimes.com/2023/01/24/health/gene-therapies-cost-zolgensma.html