Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

March 9, 2023

To: The Honorable Members of the Minnesota Legislature
Minnesota State Capitol
75 Rev. Martin Luther King Jr. Blvd.
St. Paul, MN 55155

Dear Legislators:

The Rare Disease Company Coalition (RDCC) would like to express our strong support for the following bills: **SF 705/HF 988**, **SF 1129/HF 1159**, **HF 2261**, **1029/HF 384**, **and SF 2445**, all of which would have a positive impact on the Minnesota rare disease community. Additionally, are writing to express significant concerns with **SF 168/HF 17**, as these bills would implement policies that would harm rare disease patient access to existing therapies and potentially discourage the development of new rare disease therapies.

The RDCC is a coalition of 21 life science companies committed to changing the paradigm in rare disease treatment by discovering, developing, and delivering life-changing therapies for rare disease patients around the globe. Collectively, RDCC members invested over $15 billion in research and development in 2022 and are working to advance more than 200 rare disease treatments, many of which would be the first ever Food and Drug Administration (FDA)-approved therapies for patients with a given rare disorder. To date, RDCC members have brought 45 treatments to market – the majority of which are the first FDA-approved treatments available for a given disease.

In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people. There are over 7,000 identified rare diseases that impact an estimated 25 to 30 million Americans[1] - that's about 1 in 10 Americans or about 570,000 Minnesotans living with a rare disease. These diseases are devastating and often life-threatening: 80 percent of rare diseases are genetic in origin, 50 percent impact children,[2] with many rare diseases resulting in premature deaths of infants and young children.[3]

The RDCC commends legislators for continuing Minnesota's longstanding commitment to serving rare disease patients by introducing several bills that put rare disease patients at the center of the state's health policy efforts. At the same time, we urge the legislature to reject a new proposal to create a Prescription Drug Affordability Board (PDAB) in Minnesota. A PDAB would hinder the development of, and access to, existing and future rare disease therapies and would significantly undermine the meaningful work being done to bring more treatments to rare disease patients.

SUPPORT SF 705/HF 988: Continued Funding for Minnesota's Rare Disease Advisory Council

In 2019, Gov. Tim Walz signed bipartisan legislation establishing Minnesota's Rare Disease Advisory Council (RDAC). The RDAC comprised of more than 20 diverse stakeholders from across the state's rare disease community

[1] National Center for Advancing Translational Sciences, Genetic and Rare Diseases Information Center. National Institutes of Health. Accessed March 7, 2023. Available at: https://rarediseases.info.nih.gov/about.
[2] https://rarediseases.org/wp-content/uploads/2022/10/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf
[3] https://rarediseases.org/rare-disease-advisory-councils/map/

helps give rare disease patients and families a voice in shaping policy in Minnesota. The RDAC also plays an important role in advising the legislature on issues pertaining to rare disease policy within the state. Since its inception, the Council has made huge strides in understanding and advocating for Minnesota rare disease patients, including completing one of the largest surveys of the rare disease community in the US examining the barriers patients face to accessing care[4]. However, the RDAC's funding expires after 2023, jeopardizing the important work the Council has done on behalf of the rare disease community. SF705/HF 988 would expand and extend the Council's funding through 2027, enabling them to continue thinking and acting strategically on behalf of the rare disease community in the state. It also ensures that patients do not need to rely on "luck in where they live" to seek treatment and access to medicines.[5]

SUPPORT SF 1129/HF 1159: Engaging Rare Disease Experts and Patients in Medical Assistance Formulary Decisions

SF 1129/HF 1159 would add a provider with rare disease expertise to the Medical Assistance formulary committee and require consultation with the RDAC before making formulary changes in Medical Assistance that would impact access to rare disease therapies. As illustrated by the RDAC's extensive survey, rare disease patients in Minnesota face unique challenges in receiving care, and it is vital that patients and providers with rare disease expertise are represented in decisions about formulary design and coverage. Without this important perspective, patients could be left without coverage of their medicine simply because their disease doesn't rise to the top of an agenda due to its "needle in a haystack" representation within the broader Minnesota population.

SUPPORT HF 2261: Ensure coverage and access determinations of newly approved rare disease drugs in Medicaid

HF 2261 would ensure that as Minnesota Medical Assistance is developing coverage criteria for newly approved rare disease drugs that patient access is based on the indications and usage section of the drug's FDA-approved label. Increasingly, state Medicaid agencies and payers are looking at inclusion or exclusion criteria from clinical trials for coverage determinations. These actions arbitrarily restrict access for patients whose lives would be profoundly impacted by new, innovative rare disease therapies.

SUPPORT SF 1029/HF 384: Helping Rare Disease Patients Access Expert Care Out-of-Network

People with rare diseases often face geographic barriers that impact their ability to be diagnosed or treated for their disease. In many cases, there are only a few specialists nationwide, or even worldwide, with expertise in a given rare condition.[6] Therefore, providing access to these specialists, even if they are not included in a patient's network, is another critical part of ensuring rare disease patients receive the care they need, and don't face unnecessary delays in diagnosis and treatment based upon network design. For example, the *Burden of Rare Disease* report from the National Organization for Rare Disorders (NORD) found that "39% of respondents needed to travel 60 or more miles in 2019, compared to 56% who traveled 50 or more miles in 1989.[7] SF 1029/HF 384 would allow rare disease patients to receive care out-of-network without additional cost-sharing and would enable access to the best care possible regardless of location.

[4] https://www.cbacrarediseases.org/accomplishments
[5] https://www.statnews.com/2021/07/26/21-states-give-rare-disease-patients-seat-at-the-table-other-29-need-to-follow/
[6] https://rarediseases.org/wp-content/uploads/2022/10/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf
[7] https://rarediseases.org/wp-content/uploads/2022/10/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf

SUPPORT SF 2445: Improving Rare Disease Diagnosis through Coverage of Rapid Whole Genome Sequencing

Rare disease patients face significant obstacles in getting appropriate care, starting with being accurately diagnosed. On average, it takes 4.8 years[8] and 7 different specialist[8] visits for a rare disease patient to be diagnosed. The lengthy process to answers means years of potentially debilitating symptoms, unnecessary healthcare costs for patients and the system, and potential disease progression that can impact long-term health outcomes. Fortunately, technology like rapid whole genome sequencing (rWGS) has shown tremendous potential in speeding up the journey to diagnosis for patients, especially critically ill children and infants. While Minnesota Medical Assistance followed the strong and growing evidence base around rWGS by adding it as a benefit in 2022, private health plans still largely deny coverage of rWGS as experimental or investigational, leaving those children with an undiagnosed rare disease searching for answers. Consistent with Medical Assistance coverage, SF 2445 would help deliver answers for families more quickly by requiring state-regulated private health plans to cover rWGS for critically ill patients under 21 who meet certain medical necessity criteria.

OPPOSE SF 168/HF 17: Price Controls that Threaten the Development of Life-Changing Rare Disease Therapies

While there is significant positive work happening in Minnesota in support of rare disease patients, the RDCC is certain that much of the progress made for the benefit of rare disease patients will be undermined if the legislature proceeds with creating a PDAB. The current legislative proposal does not adequately account for the unique challenges facing the rare disease community and will hinder the development of and access to existing and future rare disease therapies in the state. Rare disease patients are unique, and the development of therapies to treat these patients is uniquely challenging: taking years of research, combined with countless trials and investigations, and many times without success. SF 168/HF 17 would threaten the already complex nature of rare disease drug development, which would ultimately have the most negative impact on patients themselves. We strongly urge the legislature to preserve and build on its previous work benefiting the rare disease community and decline to move forward with creating a PDAB in Minnesota.

Conclusion

We appreciate the opportunity to provide our rare disease perspective and welcome the opportunity to work together to advance a package of bills that improves the rare disease community's access to care and supports the development of life-changing therapies for the thousands of rare diseases that currently lack treatment.

Should you have any questions, please feel free to contact Amanda Malakoff, Executive Director of the Rare Disease Company Coalition at amanda@rarecoalition.com.

Sincerely,

Amanda Malakoff
Executive Director
Rare Disease Company Coalition

[8] Barriers to Rare Disease Diagnosis, Care and Treatment in the U.S.: A 30 Year Comparative Analysis. National Organization for Rare Disorders. November 19, 2020. Available at https://rarediseases.org/wp-content/uploads/2022/10/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf.