

RARE → READY

A GENETIC CONDITION COALITION



May 4, 2023

Dear members of the Omnibus Health and Human Services Conference Committee:

Rare & Ready: A Genetic Condition Coalition is a national grassroots advocacy coalition of 46 non-profit rare disease patient organizations. Included in these organizations are patients, caregivers, and advocates based in Minnesota. Our efforts are not disease- or treatment-specific; rather, we seek to address concerns of the broader rare community.

An important policy priority for Rare & Ready is to remove barriers to patient access for innovative, lifesaving, rare disease treatments. Specifically, Rare & Ready is focused on ensuring access to gene therapies for rare conditions as they are approved by the Food and Drug Administration (FDA). Medicaid programs will increasingly scrutinize access and reimbursement policies as more and more high-cost gene therapies come to market. Rare & Ready believes there is a policy opportunity to mitigate some of the concerns of Medicaid programs and develop sustainable reimbursement pathways that hold manufacturers accountable for the clinical outcomes of their therapies, specifically value-based or outcomes-based arrangements.

To create a more sustainable approach to reimbursement for gene therapies and ensure patient access, we urge you to support including HF2395/SF2533 in the final Omnibus Health and Human Services legislation.

Rare disease patients often have few to no treatment options for their conditions and, even if there are therapies on the market, they are often characterized by significant treatment burdens, e.g., multiple infusions per week and continued threat of symptoms. Gene therapies constitute a profound shift in rare disease treatment, moving from treating the symptoms to actually addressing the underlying cause of the disease.

Understanding the huge potential for patients, it is imperative state Medicaid programs, drug manufacturers, medical providers, and patients work together to ensure access to these innovative therapies. Support of HF2395/SF2533 would allow meaningful access to happen.

Sincerely,

Members of the Rare & Ready Coalition

Learn more at www.rareandready.org