MINNESOTA
RARE DISEASE ADVISORY COUNCIL

April 4, 2024

To: Senate Committee on Health and Human Services

Re: Support for Unbundling of cell and gene therapy products

Dear Chair Wiklund, Ranking Member Utke and Committee Members,

I am writing on behalf of the Minnesota Rare Disease Advisory Council to express support for SF4058 which would unbundle cell and gene therapy products from the current reimbursement (DRG) paid to hospitals for delivery. The Minnesota Rare Disease Advisory Council (RDAC) is an executive branch state agency whose mission is to improve diagnosis and care for the 1 in 10 Minnesotans living with a rare disease of which there are over 7,000. We do this by being a policy resource for legislators and state agencies, providing support tools and rare disease education for the medical community, and convening and unifying the rare disease communities in Minnesota.

Cell and gene therapies represent an unprecedented advancement in our ability to address genetic diseases that were once considered beyond the reach of medicine. The majority of rare diseases are chronic, complex, and genetic. Historically, treatment options have been severely limited. The FDA anticipates that the rate of approvals for gene therapies will continue to accelerate in the next few years, many of which will be focused on rare diseases[1].

However, these potentially curative treatments carry a high up-front cost that challenges our reimbursement system, including Medicaid, whose structure was designed to pay for costs spread out over the lifetime of an individual with a chronic condition[2]. According to Centers for Medicare & Medicaid Services (CMS), "cell and gene therapies have high upfront costs but have the potential to reduce health care spending over time by addressing the underlying causes of disease, reducing the severity of illness, and reducing health care utilization." With a number of gene therapies already FDA approved, the lack of adequate reimbursement pathways sets up a scenario where highly effective and lifesaving treatments are available yet Inaccessible to individuals who desperately need them.

One of the key challenges related to cell and gene therapy is ensuring that the clinical benefit these one-time administered products deliver are durable over time. By unbundling the product from the other associated costs of delivering this care and their set reimbursement rates, payers can enter into value-based agreements directly with manufacturers. CMS has recommended that states take this step prior to participating in a pilot program that aims to assist states and manufacturers in entering into outcomes-based agreements within a framework that lowers prices for states and ties payment to outcomes. (cite)

The Council urges you to support SF4058 as one step toward building reimbursement pathways that ensure that available treatments for the rare disease community are accessible and sustainable into the future.

Sincerely,

Erica Barnes, Executive Director

[1] https://www.biospace.com/article/fda-braces-for-looming-boom-in-cell-and-gene-therapy-submissions/
[2] https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501(23)00077-3