Alliance for Regenerative Medicine

The Honorable Michael Burgess, M.D.
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
U.S. House of Representatives
2161 Rayburn House Office Building
Washington, DC 20515

Dear Congressman Burgess and Congresswoman DeGette,

On behalf of the Alliance for Regenerative Medicine (ARM), we commend you for your leadership in introducing and advancing *H.R. 766, The Dr. Michael C. Burgess Preventive Health Savings Act,* the bipartisan effort that directs the Congressional Budget Office (CBO) to more accurately reflect the long term, cost-saving potential of preventive healthcare initiatives. ARM strongly encourages the Senate to advance this critical legislation.

ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As the global voice of the cell and gene therapy (CGT) sector, ARM represents more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions and patient advocacy organizations. By convening the sector, facilitating influential exchanges on policies and practices and synthesizing data, we work to enable the development and utilization of advanced therapies so that patients experience the durable benefits of CGTs. Similar to the ambitions of this legislation, ARM is working to build the future of medicine.

Congress should be able to consider the long-term economic benefits of proposed legislation in a manner that accurately accounts for their impact on chronic and severe diseases. However, the way in which the CBO currently "scores" legislation severely constrains the ability of policymakers to properly assess these types of policy proposals. Consequentially, if a CBO score is perceived as excessively expensive, it is likely to hinder progress in ensuring our legislative framework aligns with advances in medical innovations. Notably, this bill requires the CBO to determine if the proposed legislation would reduce spending outside of the 10-year budget window through the use of preventive health services and mandates a description and estimate of the spending reductions to be included in CBO projections if substantial spending reductions are identified.

In recent years, numerous life-changing, and often lifesaving CGTs, have been approved by the Food and Drug Administration (FDA) for some of the most difficult-to-treat conditions. As of April 2024, there were 2,762 engineered cell therapy and genetic medicine developers worldwide sponsoring 1,920 clinical trials across dozens of indications, including rare monogenetic diseases, oncology, cardiovascular, central nervous system, musculoskeletal,

metabolic disorders, ophthalmological disorders, and more.[1] While traditional pharmaceuticals deploy chronic approaches to treat symptoms of disease, CGTs target the root causes, are often administered in a single or a limited number of doses, and offer durable and potentially curative, effects. CGTs can have high upfront costs but reduce health care spending over time by addressing the underlying precipitants of the pathophysiology, reduces the severity of illness, and lowers overall health care utilization. Accordingly, the scale of their benefits cannot be captured in abbreviated treatment intervals, and thus, when evaluating their place in a legislative proposal it is essential to identify their impact over an appropriately extended period of time.

Since CGTs generate long term spending reduction but consolidate their anticipated investment upfront, the current CBO scoring methodology fails to properly capture their value. For many of the diseases targeted by approved CGTs, the standard of care is relatively costly. For example, before the advent of gene therapy, a severe hemophilia B patient requires more than $21 million in lifetime care costs.[2] On traditional treatment regimens, lifetime healthcare costs for a severe sickle cell disease patient range from $4 to $6 million.[3] We appreciate Dr. Burgess highlighting, in a recently published article in The Hill, how challenging the feat of increasing access to CGTs is for our healthcare system.[4] While patients with conditions treated by CGTs already experience many clinical and societal roadblocks to receiving advanced pharmaceutical care, ARM aligns with Dr. Burgess' approach to addressing concerns around payment adequacy by leveraging innovative contracting options that demonstrate their cost effectiveness over time. Similarly, H.R. 766 encourages a comprehensive evaluation of outcomes by proposing to expand the CBO scoring methodology to demonstrate how certain therapies present long-term cost-effectiveness.

To assist providers offering clinical relief beyond disease state maintenance, ARM supports legislative proposals that take a global approach to assessing the impact of preventive care. H.R. 766, if enacted, will send a promising signal to patients, researchers and innovators that novel treatments will be examined for the full scope of their benefits. ARM greatly appreciates your efforts to advance *H.R. 766, The Dr. Michael C. Burgess Preventive Health Savings Act* and will continue to advocate for the appropriate assessment of preventative health services. Thank you again, and if you have any questions, please contact me at ecischke@alliancerm.org.

Sincerely,

Erica Cischke, MPH
Vice President, Government Affairs

[1] Alliance for Regenerative Medicine. "Sector Snapshot" (April 2024) available at: https://alliancerm.org/wp-content/uploads/2024/05/Sector-Snapshot-4.30.2024.pdf
[2] Li, Nanxin, Eileen K. Sawyer, Konrad Maruszczyk, Greg Guzauskas, Marta T. Slomka, Tom Burke, Antony P. Martin, Jamie O'Hara, Matt Stevenson, and Michael Recht. "Adult Lifetime Cost of Hemophilia B Management in the US: Payer and Societal Perspectives from a Decision Analytic Model." Journal of Medical Economics, 2021, available at: https://www.tandfonline.com/doi/full/10.1080/13696998.2021.1891088
[3] Subica, Andrew M. "CRISPR in Public Health: The Health Equity Implications and Role of Community in Gene-Editing Research and Applications." American journal of public health vol. 113,8, 2023, available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10323846/
4 Michael Burgess, "How to pay for 21st century medicine", The Hill, 2024, available at: https://thehill.com/opinion/healthcare/4587874-how-to-pay-for-21st-century-medicine/