



January 22, 2024

The Honorable Bill Cassidy
Ranking Member
Committee on Health, Education, Labor, and Pensions
United States Senate
Washington, DC, 20510

Dear Ranking Member Cassidy:

The National Health Council (NHC) appreciates your work in exploring issues affecting access to gene therapies.

This request for information (RFI) is a major step in addressing issues that relate to important advances for patients. Gene therapy represents a transformative improvement over traditional treatment modalities — in many cases — because it addresses the underlying genetic cause of a disease rather than treating symptoms.

Created by and for patient organizations more than 100 years ago, the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, sustainable, equitable health care. Made up of more than 170 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations, including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic drug, and payer organizations.

The NHC believes that patients need access to innovative, effective, and safe treatment, including gene therapies, as quickly and affordably as possible. However, gene therapies pose potential challenges for the health care system, which is why this RFI is very timely and welcomed. Congress should work to ensure access and affordability for patients and long-term sustainability of the health care system. Short-term spending now to support access and innovation in gene therapies is likely to result in improved health, greater independence, and likely a long-term reduction in health care costs for people with chronic diseases and disabilities. Unlike many other therapies, gene therapies often have significant up-front costs, while the savings can accrue over a lifetime. For example, studies have shown that gene therapy has high value for patients with severe hemophilia A compared with standard factor VIII prophylaxis. It is estimated that over a 10-year time span, gene therapy may cost \$1M and can offer better quality of life for patients, whereas prophylaxis can cost \$1.7M. While the value is often clear, high up-front costs with long-term savings are not

something our health care financing system is typically equipped to manage. It is vital that the health care ecosystem works with Congress to develop financing solutions that work for patients and the health care system.

While financial risk needs to be considered, the patient benefit must be the driving factor. In advancing access to gene therapies, we must encourage the engagement of patients by developers, payers, providers, and other stakeholders to ensure that any outcome- or value-based measures reflect the needs and priorities of patients, and new safeguards must be created to address new risks.

Some of the specific issues you raised with the questions in the RFI are addressed below.

Patient Engagement and Transparency

The NHC believes that robust patient engagement to identify what therapies are most needed and effective for patients must drive decision-making. When advancing access to gene therapies, Congress must encourage the engagement of patients by developers, payers, providers, and other stakeholders to ensure that any decisions affecting access reflect the needs and priorities of patients.

Coverage

Considering the financial risk to the health care system is critical, but it should not supplant consideration of patients' benefits, risks, or financial impact. For instance, coverage models should consider that investment in costly treatment now may allow an individual to live and work independently in the long-term. This will also allow coverage for gene therapies to meet real patient needs. Coverage and value models may need to differ for diverse types of gene therapies, and Congress should not put forth a one-size-fits-all approach. The NHC has developed a suite of tools in its [Value Classroom](#) to help guide value considerations from a patient perspective.

Congress should work to build flexibility and incentives for companies to engage early with patients, payers, and providers to design the most suitable payment model for gene therapies. There may be different approaches that work for different therapies or classes of therapies. By engaging early with patients, payers, and providers on payment models, companies can get a head start on assessing the cost-benefit analysis of the gene therapy they plan to launch. Creating risk protection for patients, plans, manufacturers, and providers that incentivize access and affordability must be the priority.

The RFI also asks whether coverage should be mandated. The NHC believes the federal government should be the entity that provides incentives for appropriate coverage and removes inappropriate and overly burdensome barriers that may limit access.

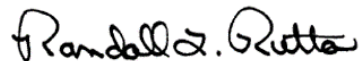
Utilization Management

Much like value decisions and payment models, utilization management (UM) processes should be informed by patient needs and provider recommendations and exist solely to protect against patient harm, waste, fraud, and abuse. The development of prior authorization and other UM protocols is typically done without much or any patient input, and the rationale for such decisions is not always public or accessible to patients. The current application of utilization management can be associated with delays in patient care and the potential for medication adherence issues, potentially resulting in the need for higher cost and more intensive care. As gene therapies advance, we need to ensure that utilization management practices are appropriately used. The NHC has developed a series of [Domains and Values](#) on the issues that we urge Congress to use in guiding any utilization management policy decisions.

Conclusion

Please do not hesitate to contact Eric Gascho, Senior Vice President of Policy and Government Affairs, if you or your staff would like to discuss these issues in greater detail. He is reachable via e-mail at egascho@nhcouncil.org.

Sincerely,



Randall L. Rutta
Chief Executive Officer