December 15, 2022

Division of Dockets Management (HFA-305)
Food and Drug Administration
Docket No: FDA-2022-N-2394
5630 Fishers Lane
Room 1061
Rockville, MD 20852

Submitted electronically

Re: FDA CBER OTAT Public Patient-Focused Drug Development Listening Meeting — Patient Perspectives on Gene Therapy Products; Request for Comments [Docket No. FDA-2022-N2394]

Dear Dr. Bryan:

FasterCures, a center of the Milken Institute, appreciates the opportunity to submit comments to the Food and Drug Administration's (FDA) Center for Biologics Evaluation and Research (CBER) Office of Tissues and Advanced Therapies (OTAT) as part of the Patient-Focused Drug Development Meeting on Patient Perspectives on Gene Therapy Products held on November 15, 2022. FasterCures is driven by a singular goal: to save lives by speeding scientific advancements to all patients. In service to this mission, FasterCures has convened patients, patient groups, nonprofit organizations, product developers, providers, and payers to identify and bring greater attention to potential barriers to gene therapy development and access. Based on this work, we are pleased to share with you three areas of recommendations to strengthen public information about gene therapies.

Over the past several years, FasterCures has brought together our community of stakeholders to share their perspectives on gene therapies on a range of topics relating to product development, the manufacturing process, barriers to access, value assessment, and long-term data follow-up. One consistent theme we hear is that there is significant variability among patients and caregivers (not to mention among providers and payers) on their knowledge and understanding of gene therapies. On one end of the spectrum, there is deep technical expertise, experience, and comfort with these technologies. On the other end—and representing the majority—are individuals grappling with the more fundamental questions about how these therapies work within the body, where they fit in the landscape of other treatment alternatives (if those are available), and the range of risks involved.

All of us in the healthcare field recognize the essential role of education to enable patients and caregivers to take a proactive role in their own care. It is no surprise then that resources for patients on gene therapies have flourished. But whether the information is up to date, balanced, and informative is left for patients and families to determine. Not only does this place additional burdens on patients, but it also creates the potential for misinformation to spread. COVID-19

has shown us the devastating harm of misinformation and how difficult it can be to combat and reverse. Public reactions to (false) claims that COVID-19 vaccines are gene therapies give us insight into the fear that could be propagated about gene therapies specifically. FDA Commissioner Robert Califf has identified misinformation as one of his biggest concerns in healthcare, and has emphasized his commitment "to help the public get accurate, science-based information." To help deliver on this commitment, FasterCures offers the following recommendations:

- **Help make credible information about gene therapies easier for patients and caregivers to find.** A web search of gene therapies leads to millions of results; however, too often it requires patients and caregivers to sift through the information to determine what is reliable and what is not. Some patients are fortunate enough to find their way to patient advocacy organizations, but for many, FDA will be the first port of call.

  On this point, we offer two recommendations. First, we urge FDA to organize the resources on its website so that information related to gene therapies is linked and more straightforward to find (e.g., existing resources like "What Is Gene Therapy?", "Regenerative Medicine 101", and "Basics of Clinical Trials"). Today, these resources reside in multiple areas of the FDA website which places the burden on patients and caregivers to navigate the available information. Gene therapies would benefit from a landing page that acts as a one-stop shop for FDA's resources, much like FDA's COVID-19 page, which links to all related resources and information for both patients and healthcare professionals.

  Second, we encourage FDA to create a collaborative of individuals and independent organizations that would focus on gene therapy education (perhaps modeled on the Patient Engagement Collaborative). Its functions could include: identifying high-quality resources to which FDA can reference, determining ways to distribute these resources so that it can reach more people, moving the field toward more consistency in terminology and descriptions used for gene therapies, identifying gaps in education, and setting expectations for accessibility of content (e.g., understandability, translations). There is likely a role for the Reagan-Udall Foundation in supporting the Agency on creating such a collaborative.

- **Identify and work toward filling the areas where current educational resources are not matching the information need.** We must continuously seek to understand the questions and concerns about gene therapies that may not be adequately addressed in existing educational materials. To that end, FasterCures, with input from its community, developed a journey map to delineate some of the questions that may arise for patients and caregivers when confronted with the option of a cell or gene therapy. Based on this work and subsequent conversations, more education may be needed on: the factors that could affect a patient's eligibility for a clinical trial or for an approved therapy; what happens if a treatment does not work (and the potential impact on eligibility for future treatments); information on viral vectors (especially HIV); and what to expect after treatment. A collaborative like the one we recommend further above could help move the ecosystem toward addressing these gaps with consistent and reliable information.

  More work remains to be done to identify myths and misperceptions about gene therapies, especially among communities who have not historically been given opportunities to engage with the biomedical research system. We encourage FDA to intensify its outreach through community-based organizations working with underrepresented populations throughout the

US to surface areas where trusted information may be needed. This should connect to FDA's efforts to increase enrollment of diverse population in drug development and the work of the Office of Minority Health and Health Equity to help address barriers preventing diverse groups from participating in clinical trials. Driving more participation in clinical trials of gene therapies cannot be achieved without also increasing understanding of the therapies themselves.

- **Invest in provider education**. Provider education must go hand in hand with patient education. At our convenings, providers have shared that the healthcare professionals with in-depth understanding of gene therapies are few and overstretched. Medical school curricula is lagging behind the innovation, thus not producing a new crop of professionals who are prepared to engage on these technologies. As FDA works to understand patient perspectives on gene therapies, we urge FDA to do the same for providers. A single landing page for gene therapy information and resources, as recommended above, would be a step toward easing the burden for providers seeking information.

  Finally, we must do more collectively to strengthen provider-patient dialogue on gene therapies. It is well-documented that a patient's interaction with their physician affects their decisions about care and treatment. Across multiple types of healthcare interventions, we have seen that bias and stereotyping affect providers' willingness to share opportunities to participate in clinical trials or delay the provision of optimal treatment.[i,ii] Provider education must be designed with purpose and intention to address this bias.

Thank you again for the opportunity to offer our input. We are happy to discuss these recommendations further and to help FDA advance any of the ideas shared in this letter.

Best,

Sung Hee Choe
Senior Director
FasterCures, a center of the Milken Institute

[i] Soumya Niranjan et al., "Bias and stereotyping among research and clinical professionals: Perspectives on minority recruitment for oncology clinical trials," *Cancer* 126, no. 9: p. 1958-1968 (March 2020), https://doi.org/10.1002/cncr.32755

[ii] Shawnika J. Hull, Hanna Tessema, Jeri Thuku, and Rachel Scott, "Providers PrEP: Identifying Primary Health care Providers' Biases as Barriers to Provision of Equitable PrEP Services," *JAIDS Journal of Acquired Immune Deficiency Syndromes* 88, no. 2: p 165-172 (October 2021), https://doi.org/10.1097/QAI.0000000000002750