American Society
of Gene + Cell Therapy

September 6, 2022

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U. S. Department of Health and Human Services
200 Independence Avenue, SW
Washington DC 20201

Dear Administrator Brooks-LaSure:

The American Society of Gene and Cell Therapy appreciates the opportunity to comment on CMS-1770-P, the proposed rule updating the Medicare Physician Fee Schedule for 2023.

Our comments are specific to the implementation of Section 900004 of the Infrastructure Investment and Jobs Act ("Infrastructure Act"), which establishes a new requirement for drug manufacturers to provide "refunds" to the government for discarded amounts of a product after administration. As the Centers for Medicare & Medicaid Services (CMS) considers how to implement this provision, we encourage the agency to develop policies that acknowledge the unique nature of gene and cell therapies and provide a flexible and nuanced approach.

About ASGCT

The American Society of Gene and Cell Therapy (ASGCT) is a nonprofit professional membership organization comprised of more than 5,600 scientists, physicians, and other professionals working in gene and cell therapy in settings such as universities, hospitals, and biotechnology companies. Many of our members have spent their careers in this field
performing the underlying research that has led to today's robust pipeline of transformative therapies. A core portion of ASGCT's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. To that end, ASGCT supports Medicare payment policies that foster the adoption of, and patient access to, new therapies, which thereby encourage continued development of these innovative treatments. The Society's support of sufficient and appropriate reimbursement levels to providers to facilitate patient access does not imply endorsement of any individual pricing decisions.

Requiring Manufacturers of Certain Single-Dose Container or Single-Use Package Drugs To Provide Refunds With Respect to Discarded Amounts §§ 414.902 and 414.940)

The Infrastructure Act requires manufacturers to provide a refund to CMS for "certain discarded amounts from a refundable single-dose container or single-used package drug." The amount of the refund is the difference between the discarded amount and an applicable percentage, which is at least ten percent, of total charges for the drug in a given quarter. The Infrastructure Act excluded radiopharmaceutical or imaging agents, certain drugs requiring filtration, and certain new drugs from that requirement. ASGCT requests that CMS exclude gene and cell therapies from this requirement.

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In the proposed rule, CMS does not offer any flexibility in the applicable percentage for drugs with unique circumstance. However, the agency does request comment on potential instances that would merit establishing higher applicable percentages. To that end, we suggest that CMS consider the method of administration and product preparation as potential unique circumstances that would warrant a higher applicable percentage, as these circumstances often present themselves as innate characteristics of the safe and effective administration of gene and cell therapies. In this regard, these characteristics of gene and cell therapies make them markedly different from other Part B drugs and CMS policies should reflect these distinctions.

Many gene and cell therapies necessitate complex preparation and administration techniques. FDA's approved dosage is the exact amount of the product needed for the *patient*. Upstream of entry into the patient, gene and cell therapies often require many additional steps in order to prepare the product. On the high end of the volume spectrum, this can include dilutions and mixing which, for each step, will create product loss in dead volumes of syringes and tubing. On the other end, some gene and cell therapies are injected directly into a tissue to be treated, such as the eye or, in the future, the brain, and use very small volumes (less than 1mL). Volumes this small are technically complex to work with and necessitate higher volumes in the source vial. This ensures enough product to fill syringe and needle dead volumes as well as to allow the product to be drawn up without air. Taken together, the steps involved in the administration of these products by their nature increases the amount of "wastage" as defined by CMS without, in a practical sense, wasting product. Similar to the specific exclusion of filtration in the Act, gene and cell therapies have unique and practical factors as relevant as filtration. We do not believe that this is the Congressional intent of the provision and request that gene and cell therapies be excluded from the provisions in the Infrastructure Act.

In addition, CMS should consider the personalized and limited-dose nature of gene and cell therapies. Generally, gene and cell therapies are designed to be implemented through a one-time (or few-time) dose. Effective administration of the treatment thus necessitates having a sufficient quantity of the therapy available at the one time of administration. Insufficient or "barely enough" amounts could lead to inferior outcomes and risk of the health and well-being of the patient.

We appreciate the opportunity to share our perspective on CMS' proposed rule. ASGCT understands the intent of the Investment Act and we believe that intent can be met by implementing the law with a nuanced approach that accounts for preparation and practical matters in clinical care. If you have any questions, please do not hesitate to contact Margarita Valdez Martínez, Director of Policy and Advocacy, at mvaldez@asgct.org.

Sincerely,

David Barrett, J.D.
Chief Executive Officer