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June 10, 2024

VIA ELECTRONIC DELIVERY

Administrator Chiquita Brooks-LaSure
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-1808-P
P.O. Box 8013
Baltimore, MD 21244-8013

RE: IGT Comments on FY 2025 IPPS Proposed Rule [CMS-1808-P]

Dear Administrator Brooks-LaSure:

The Institute for Gene Therapies (IGT or "the Institute") is pleased to submit these comments to the Centers for Medicare and Medicaid Services (CMS or "the Agency") regarding the Fiscal Year (FY) 2025 Hospital Inpatient Prospective Payment Systems (IPPS) Proposed Rule ("Proposed Rule").[1] IGT launched in February 2020 as a multi-stakeholder coalition that advocates for a modernized regulatory and reimbursement framework to promote the development of transformative gene therapies and enhance patient access. The Institute represents a wide array of patient advocacy groups, gene therapy manufacturers, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies through the collaboration of the Corporate Advisory Council, Patient Advocacy Advisory Council, and Scientific, Academic and Medical Council. IGT is dedicated to promoting the value of transformative gene therapies and advocating for policies and practices to ensure that patients that require these treatments can access them. Our most vulnerable patients and their families anxiously wait to access these gene therapies and the life-altering benefits they offer for the treatment of some of the most debilitating and rare diseases. A full list of our members is available at https://www.gene-therapies.org/_files/ugd/b11210_c2ec04ef7d9c496887611cfb16f24388.pdf?index=true.

Adequate reimbursement in both Medicare and Medicaid is essential to ensure access and reduce disparities, in addition to supporting providers who administer these therapies. Gene therapies have the potential to transform how we treat diseases in this country, particularly serious conditions with high unmet medical need. When access to these treatments is improved across the population, we have the tools to help reverse decades of health inequities. Scores of gene therapies currently in clinical trials in the US are designed to be administered in the inpatient setting, addressing rare and ultra-rare diseases, central nervous system diseases, and conditions disproportionately impacting historically disadvantaged populations.[2] In many cases, gene therapies halt but cannot reverse the effects of a disease by addressing the underlying genetic cause. For this reason, any delay in access to an approved gene therapy can result in patients continuing to suffer irreversible damage caused by their disease that may otherwise be avoided. IGT commends CMS for acting in recent years to facilitate policies that bolster access to critical therapies for rare diseases, including the finalization of the Medicaid "multiple best

[1] 89 Fed. Reg. 35,934 (May 2, 2024).
[2] See ClinicalTrials.gov.

prices" regulation that will enable greater adoption of value-based payments[3] along with the development of the Cell and Gene Therapy Access Model.[4] We welcome every opportunity to engage with CMS to advance policies to improve access to these innovative therapies in programs under the Agency's purview.

The Institute has and continues to support the establishment of policies for an enhanced New Technology Add-on Payment (NTAP) pathway for gene therapies, as CMS explores mechanisms to address payment concerns for patients with rare diseases and conditions.[5] As gene therapy manufacturers work closely with providers to develop the expertise to safely and effectively administer gene therapies to patients, it is imperative that regardless of the setting of care (e.g., inpatient v. outpatient) providers administering these treatments receive adequate reimbursement. Reimbursement should not dictate the setting of care for such transformative therapies, and we hope that CMS will lead the efforts to ensure that access is promoted across payers and settings.

The Institute supports policies that provide assurance of fulfillment of the NTAP Substantial Clinical Improvement (SCI) criterion upon FDA approval of the gene therapy applicant, flexibilities for NTAP approval timing and immediate access, an enhanced payment duration, and an increased add-on payment rate for gene therapies. To that effect, IGT provides comments in response to the following proposals included in *Section II.E Add-On Payments for New Services and Technologies for FY 2025* of the Proposed Rule:

- Proposed FY 2025 Applications for New Technology Add-On Payments (Traditional Pathway) (II.E.6) Recommendations
- Proposed Change to the Method for Determining Whether a Technology Would Be Within Its 2- to 3-Year Newness Period When Considering Eligibility for New Technology Add-On Payments (II.E.7)
- Proposed Change to the Calculation of the Inpatient New Technology Add-On Payment for Gene Therapies Indicated for Sickle Cell Disease (II.E.9)

I. Proposed FY 2025 Applications for New Technology Add-On Payments (Traditional Pathway) (II.E.6)

In the Proposed Rule, CMS received 16 NTAP applications for FY 2025 under the traditional pathway. Among these, three applications were submitted for gene therapies, Casgevy (exagamglogene autotemcel) for the treatment of Sickle Cell Disease (SCD), Casgevy for the treatment of Transfusion-Dependent Beta Thalassemia (TDT), and Lyfgenia (lovotibeglogene autotemcel) for the treatment of SCD. The FDA approval of gene therapies for these diseases has the potential to transform the treatment paradigm and prognosis for rare conditions that previously lacked suitable or available treatments. IGT is proud to highlight the increase in FDA approved gene therapies now available to patients across the US, including Medicare beneficiaries, and we strongly support the three gene therapy NTAP applications. In its discussion of these applications, however, CMS presents concerns about these therapies' fulfillment of the Substantial Clinical Improvement (SCI) criterion.

IGT strongly disagrees with these concerns and urges CMS to confirm in the Final Rule that all three satisfy the SCI criterion. For beneficiaries with sickle cell disease (SCD), the available therapy of hematopoietic stem cell transplantation is a potentially curative treatment. However, patients face significant barriers to access. Fewer than 25% of people with SCD have a matched sibling who could potentially serve as a donor, and the potential

[3] 85 Fed. Reg. 87,000 (Dec. 31, 2020).
[4] CMS Innovation Center, Cell and Gene Therapy (CGT) Access Model: https://www.cms.gov/priorities/innovation/innovation-models/cgt.
[5] IGT Comments on FY 2024 IPPS Proposed Rule (Jun. 9, 2023), https://www.gene-therapies.org/_files/ugd/b11210_d01b7e91290c4dc2b1d12df3d72a6b23.pdf?index=true.

side effects of attempting a stem cell transplant may be too risky for some patients.[6] For patients with beta thalassemia, available treatments have historically included regular blood transfusions or transplantation of bone marrow, options that present significant risk and complications; in the young population, bone marrow transplant results in a 23% rejection rate, which can ultimately become fatal.[7,8] For a large number of patients with SCD and beta thalassemia, a gene therapy is the only transformative, durable, and potentially curative treatment option and thus, represents a substantial improvement and advance in treatment, as outlined in regulation.

Furthermore, in the future, IGT asks CMS to consider amending its NTAP regulations to plainly state that an FDA-approved gene therapy that has a Breakthrough Therapy and/or Regenerative Medicines and Advanced Therapy (RMAT) designation automatically satisfies the SCI criterion.

The NTAP Program was established with the specific aim of supporting timely access to, and incentivizing innovation for, novel therapies within the Medicare population. CMS implemented the SCI criterion "to ensure that new technology can be demonstrated to provide a substantial clinical improvement based on verifiable evidence."[9]

We believe that CMS' own NTAP regulations provide ample support for a clear determination that gene therapies with an FDA Breakthrough Therapy and/or RMAT Designation represent not only a substantial, but transformational, clinical improvement over existing treatments or therapies. Today, for a new service or technology to represent a substantial clinical improvement, CMS' regulations require that the new technology represent an advance that substantially improves, relative to services or technologies previously available, the diagnosis or treatment of Medicare beneficiaries.[10] This requirement is established, for example, if the new technology offers a treatment option for a patient population unresponsive to, or ineligible for, currently available treatments.[11] Alternatively, the SCI criterion can be satisfied by demonstrating *just one* of the following seven outcomes: (1) a reduction in at least one clinically significant adverse event, including a reduction in mortality or a clinically significant complication; (2) a decreased rate of at least one subsequent diagnostic or therapeutic intervention; (3) a decreased number of future hospitalizations or physician visits; (4) a more rapid beneficial resolution of the disease process treatment including, but not limited to, a reduced length of stay or recovery time; (5) an improvement in one or more activities of daily living; (6) an improved quality of life; (7) a demonstrated greater medication adherence or compliance.[12]

An FDA-approved gene therapy that has a Breakthrough Therapy and/or RMAT designation should be deemed to automatically satisfy the SCI criterion given how the FDA awards such designations (i.e., in part, on the basis of a similar definition of meeting an unmet medical need).

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[6] Modified Stem Cell Transplant Procedure Shows Favorable Results in Adults Living with Severe Sickle Cell Disease (Dec. 12, 2023); available at: https://www.hematology.org/newsroom/press-releases/2023/lba4.
[7] Beta-Thalassemia (2024); available at: https://www.hopkinsmedicine.org/health/conditions-and-diseases/beta-thalassemia.
[8] Current status of beta-thalassemia and its treatment strategies (Nov. 5, 2021); available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8683628/.
[9] 66 Fed. Reg. 46,902 (Sept. 7, 2001).
[10] 42 C.F.R. § 412.87(b)(1).
[11] 42 C.F.R. § 412.87(b)(1)(ii)(A).
[12] 42 C.F.R. § 412.87(b)(1)(ii)(C).

II. Proposed Change to the Method for Determining Whether a Technology Would Be Within Its 2- to 3-Year Newness Period When Considering Eligibility for New Technology Add-On Payments (II.E.7)

In the Proposed Rule, CMS proposes to extend new technology add-on payments for an additional fiscal year for technologies whose three-year anniversary date of US market entry occurs on or after October 1 of that same fiscal year. CMS proposes this policy change to be applicable to payments beginning in 2026 with technologies initially approved for NTAP in FY 2025 or a subsequent year. IGT commends this provision for increased payment duration and flexibility. IGT has previously recommended approval timing flexibilities to ensure immediate access and enhanced payment duration.[13] Further, given the lower prevalence of the rare diseases in the Medicare population that available gene therapies address (and will in the future), CMS will likely need at least three years of NTAP payments to gather sufficient data to update the DRG. The nature and intent of the NTAP program is to provide additional payment to treatments that are deemed innovative, which is a hallmark of the gene therapy field.

III. Proposed Change to the Calculation of the Inpatient New Technology Add-On Payment for Gene Therapies Indicated for Sickle Cell Disease (II.E.9)

The ability of bundled payment systems, like the IPPS, to adequately reimburse for gene therapies is one of the most significant concerns for gene therapy stakeholders seeking to ensure provider and patient access to these transformative therapies. In this Proposed Rule, CMS proposes an enhanced NTAP payment rate of 75% for gene therapies indicated for SCD, which is to be effective in FY 2025. Though IGT supports an enhanced payment rate, the Institute, as submitted previously, requests that CMS further expand this proposal to further increase the add-on payment and to extend this increased rate to all qualifying gene therapies. **In alignment with our previous recommendations and CMS' authority under the Social Security Act, the NTAP payment amount for gene therapies should be 100% of the cost of the therapy, not 65 or 75%.** [14] Given the transformative nature of gene therapies, it is essential that Medicare reimbursement does not impede access for Medicare beneficiaries nor stifle future innovation. Even under an enhanced 75% add-on payment, hospitals may be put at financial risk when providing gene therapy to Medicare beneficiaries. This enhanced add-on payment (at 100% of the cost of the therapy) would provide more certainty around expected reimbursement parameters for qualifying gene therapies administered in the hospital inpatient setting, thereby better facilitating patient access. Reimbursement at invoice cost will help ensure access, continued innovation, reduction in health disparities, positive impacts on other payer coverage decisions, and it also commensurately recognizes the durable and transformative value that gene therapies offer to patients, their families, and society.

Additionally, CMS states that the Agency seeks comment regarding a proposal to make the 75% add-on payment percentage "available only to applicants that meet certain additional criteria, such as attesting to offering and/or participating in outcome-based pricing arrangements with purchasers (without regard to whether the specific purchaser availed itself of the outcome-based arrangements), or otherwise engaging in behaviors that promote access to these therapies at lower cost." **IGT does not support adding a mandate related to outcomes-based contracts or other undefined behaviors to the qualifications needed to be eligible for NTAP.** No such mandates are provided in the statutory authority governing NTAP eligibility, and CMS' efforts to implement broader acceptance and adoption of alternative payment and contracting mechanisms are at the infancy stage. Currently, there is no mechanism by which fee-for-service Medicare can engage in value-based payment

[13] IGT Comments on FY 2024 IPPS Proposed Rule (Jun. 9, 2023); available at: https://www.gene-therapies.org/_files/ugd/b11210_d01b7e91290c4dc2b1d12df3d72a6b23.pdf?index=true.
[14] IGT Comments on FY 2024 IPPS Proposed Rule (Jun. 9, 2023); available at: https://www.gene-therapies.org/_files/ugd/b11210_d01b7e91290c4dc2b1d12df3d72a6b23.pdf?index=true.

arrangements; introducing this mandate now, without an established mechanism, could undermine the Agency's goal of improving timely access.

IV. Conclusion

IGT appreciates the opportunity to comment on key gene therapy payment issues under the Medicare IPPS that have significant implications for patient access. IGT welcomes the continued opportunity to engage with CMS over the coming years regarding broader payment concepts to ensure a strong future for gene therapy across payer systems. We would be pleased to serve as a resource on gene therapy issues and answer any questions regarding these comments.

Sincerely,

John R. Feore, III
Director, Health Policy and Advocacy
Institute for Gene Therapies