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June 9, 2023

VIA ELECTRONIC DELIVERY

Administrator Chiquita Brooks-LaSure
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-1785-P
P.O. Box 8013
Baltimore, MD 21244-8013

RE: IGT Comments on FY 2024 IPPS Proposed Rule [CMS-1785-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") on the Fiscal Year (FY) 2024 Hospital Inpatient Prospective Payment System (IPPS) proposed rule (the "proposed rule").[1] IGT was launched in February of 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access. Through a Corporate Advisory Council, Patient Advocacy Advisory Council, and Scientific, Academic & Medical Council, 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. IGT is devoted to promoting the value of transformative therapies and advocating for policies and practices to ensure patient access to these treatments. Our most vulnerable patients and their families anxiously wait for the life-altering treatments that gene therapies will offer to some of the most debilitating or rare diseases. A full list of our members is available at https://www.gene-therapies.org/about-igt.

IGT commends CMS for taking action in recent years to facilitate policies that bolster access to critical therapies for rare diseases. In the FY 2023 IPPS proposed rule, CMS requested comments on issues related to the classification of rare diseases represented by low volumes in the Medicare Severity Diagnosis Related Group (MS-DRG) claims data. We are disappointed that CMS did not include any proposals in this FY 2024 proposed rule in response to last year's request. As such, IGT reiterates our previous recommendations to CMS to establish (1) a unique MS-DRG for each approved gene therapy in the inpatient setting; and (2) to establish 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.

I. Establish an MS-DRG for Each Approved Gene Therapy

IGT has the same concerns expressed by CMS in last year's IPPS proposed rule that the MS-DRG system as currently structured is inadequate to ensure access to gene therapies, many of which will provide treatment for

[1] 88 Fed. Reg. 26,658 (May 1, 2023).

rare and ultra-rare diseases with low Medicare claims volume. The institute urges CMS to think outside the box in modifying or designing a system that provides adequate reimbursement for future gene therapies, particularly given the number of gene therapies that will target conditions prevalent in populations who have and continue to experience severe health inequities. Many gene therapies in development have the potential to reverse decades of health inequities faced by disadvantaged populations and Medicare's coverage and reimbursement policies will directly determine whether these populations have access to life-changing medical breakthroughs. **IGT recommends that CMS establish an MS-DRG for each approved gene therapy in the inpatient setting** to ensure that the IPPS does not inhibit access to, or the future innovation of, gene therapies.

While CMS has expressed hesitation regarding the creation of lower volume MS-DRGs, this precedent should not preclude CMS from designing a reimbursement system that adequately covers the cost of gene therapies, along with ensuring appropriate reimbursement for the patient care costs incurred by the hospital during the course of treatment. The IPPS and MS-DRG system ("a system of averages") was designed before transformative therapies like gene therapies became a reality – and an expectation for our future healthcare system. Adherence to the current structure simply for the sake of adherence could devastate the innovative drive to facilitate timely access to gene therapies for patients with high unmet clinical need. We encourage CMS to view gene therapies in a different light due to their transformational nature and to use whatever tools necessary to ensure that access is not hindered.

II. Establish an Enhanced NTAP Pathway for Gene Therapies

The Food and Drug Administration (FDA) anticipates that by 2025, the agency will approve 10-20 cell and gene therapies each year. Since publication of the FY 2023 IPPS final rule, FDA has approved four gene therapies.[2] In 2020, FDA cited that the agency had received approximately 900 investigational New Drug (IND) applications specifically for gene therapies.[3] While not all of these gene therapies will be administered on an inpatient basis, the lack of clarity regarding payment parameters for these therapies under bundled payment systems like the IPPS is one of the most pressing patient access concern for the future of gene therapy.

IGT requests that CMS establish an enhanced NTAP pathway specific to gene therapies, similar to the pathways developed for Qualified Infectious Disease Products (QIDPs) and pursuant to the Limited Population Pathway for Antibacterial and Antifungal Drugs (LPADs). The FY 2021 IPPS final rule established a new alternative NTAP pathway for therapies approved through the LPAD. In the FY 2020 IPPS final rule, CMS provided an alternate NTAP pathway for therapies receiving QIDP designation. These newly established alternative pathways will provide streamlined opportunities for novel therapies that qualify to receive NTAP under IPPS. CMS should adopt a similar enhanced NTAP pathway for gene therapies to ensure rapid patient access to these cutting-edge technologies. Our specific recommendations for an enhanced NTAP pathway for gene therapies are discussed below:

Assurance of Satisfying "Newness" and "Substantial Clinical Improvement" Criteria based on Breakthrough Therapy Designation, Priority Review Designation, Accelerated Approval, or RMAT Designation

[2] In chronological order, see: (1) https://www.fda.gov/vaccines-blood-biologics/zynteglo; (2) https://www.fda.gov/vaccines-blood-biologics/skysona; (3) https://www.fda.gov/vaccines-blood-biologics/vaccines/hemgenix; and (4) https://www.fda.gov/vaccines-blood-biologics/vyjuvek.

[3] Press Release, Food and Drug Administration, FDA Continues Strong Support of Innovation in Development of Gene Therapy Products (Jan. 28, 2020).

Both the QIDP and LPAD alternative pathways rely on FDA designations for the products for purposes of satisfying the traditional "newness" or "substantial clinical improvement" criteria.[4] If the FDA designation for a QIDP or LPAD is provided, then CMS accepts the designation as a substitute for the traditional assessment it conducts in terms of whether the product is "new and not substantially similar to an existing technology," as well as in finding that it "represent[s] an advance that substantially improves, relative to technologies previously available, the diagnosis or treatment of Medicare beneficiaries."[5] CMS has similarly deemed medical devices that are part of FDA's Breakthrough Devices Program to satisfy "newness" and "substantial clinical improvement" criteria.[6] **IGT requests that CMS advance a proposal to permit approved gene therapies with FDA designation of Breakthrough Therapy designation, Priority Review designation, Accelerated Approval, or Regenerative Medicine Advanced Therapy (RMAT) designation to be deemed to satisfy the "newness" and "substantial clinical improvement" criteria**.

Breakthrough Therapy is only provided to drugs that are intended to treat serious conditions and for which "preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s)."[7] Priority Review serves to focus attention and resources to review of "drugs, that if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications."[8] FDA created the Accelerated Approval pathway to expedite the availability of novel treatments that address urgent and unmet medical needs of patients with serious and often life-threatening diseases.[9] Under the Accelerated Approval pathway, FDA may approve a drug that demonstrates safety and efficacy in well-controlled clinical trials where efficacy is based on a surrogate endpoint that is reasonably likely to predict clinical benefit.[10] RMAT designation is limited to drugs that satisfy the definition of regenerative medicine therapy; are intended to "treat, modify, reverse, or cure a serious or life-threatening disease or condition;" and preliminary medical evidence indicates that the therapy has the potential to address unmet medical needs for the disease or condition.[11] **IGT urges CMS to deem gene therapies attaining these notable FDA designations or approved through accelerated approval as sufficiently new and demonstrating substantial clinical improvement** for purposes of qualifying for NTAP.

In finalizing CMS' new NTAP policies for medical devices included in the FDA Breakthrough Medical Devices Program, CMS emphasized the importance of these revisions to "address barriers to health care innovation and ensur[e] that Medicare beneficiaries have access to critical and life-saving new cures and technologies that improve beneficiary health outcomes."[12] This same rationale applies as strongly, if not more so, in the case of gene therapies, where the therapy works by addressing the genetic cause of the disease directly to "treat, cure, or prevent a disease or medical condition."[13] Bringing gene therapy to fruition has taken decades of work for

[4] 85 Fed. Reg. 58,432, 58,435 (Sept. 18, 2020).
[5] *Id.*
[6] *Id.* at 58,606.
[7] Breakthrough Therapy, FDA (Jan. 4, 2018), https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy.
[8] Priority Review, FDA (Jan. 4, 2018), https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review.
[9] FDA, New Drug, Antibiotic, and Biological Drug Product Regulations; Accelerated Approval, 57 Fed. Reg. 58,942 (Dec. 11, 1992).
[10] Accelerated Approval, FDA (Jan. 1, 2018), https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/accelerated-approval.
[11] Regenerative Medicine Advanced Therapy Designation, FDA (Oct. 5, 2020), https://www.fda.gov/vaccines-bloodbiologics/cellular-genetherapy-products/regenerative-medicine-advanced-therapy-designation.
[12] 84 Fed. Reg. 42,044, 42,047 (Aug. 16, 2019).
[13] What Is Gene Therapy? How Does It Work?, FDA (Dec. 12, 2017), https://www.fda.gov/consumers/consumerupdates/what-genetherapy-how-does-it-work.

scientists, with an aim of revolutionizing how diseases are treated by addressing the cause of the disease itself.[14] With the potential to cure and prevent rare and often life-threatening disorders, an enhanced gene therapy NTAP pathway is critical for providing a predictable and streamlined process for ensuring rapid access to qualifying gene therapies.

Approval Timing Flexibilities to Ensure Immediate Access

The new LPAD and QIDP NTAP alternative pathways provide flexibility relating to the NTAP requirement for a new technology to receive FDA marketing authorization (e.g., approval or clearance) by July 1 to be eligible in the final rule for NTAP.[15] Instead, CMS provides "conditional" NTAP for products through these pathways that do not receive approval by this deadline but otherwise satisfy NTAP criteria.[16] Qualifying products begin receiving NTAP for qualifying discharges the quarter following the date of FDA marketing authorization provided the technology obtains FDA marketing authorization by July 1 of the fiscal year for which the applicant sought NTAP.[17] **IGT believes that this same timing flexibility should be part of an enhanced gene therapy NTAP pathway**, especially in light of CMS' current proposal to move the approval deadline to May 1. Requiring a waiting period under the traditional NTAP pathway may pose a barrier to patient access to these transformative therapies following FDA approval where the July 1 (or proposed May 1) deadline is not met, particularly given the low volumes anticipated for many gene therapies. These delays would be especially concerning for therapies for progressive and life-threatening diseases where early treatment is essential for avoiding worsening of functions that cannot be regained or, in the worst-case scenarios, mortality. 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. Immediate access to gene therapy will provide substantial benefit for patients' short- and long-term health, their caregivers, the healthcare system, and society.

Enhanced Payment Duration

An enhanced NTAP pathway for gene therapies should provide for a lengthier period of increased reimbursement than the existing two to three years provided under the statute. While the statute outlines the timeframe for eligible NTAP payments, CMS has used its discretion in the past to offer NTAPs beyond this two- or three-year window. Given the uncertainty with data collection for FY 2022 rate setting due to COVID-19, in the FY 2022 IPPS final rule, CMS extended NTAPs for an additional year for products that otherwise would have reached the end of their three-year window.[18] This same logic can apply to gene therapies, many of which won't have large claims volumes to sufficiently establish the appropriate level of costs within the applicable MS-DRG in which they are assigned. Absent the creation of gene therapy-specific MS-DRGs, this reality is more prevalent and worthy of consideration. **CMS should use its regulatory discretion to provide for an NTAP payment for approved gene therapies for at least five years** to ensure that claims volumes reach a satisfactory level to properly inform rate setting and to facilitate continued gene therapy innovation.

IGT would also support CMS working with its partners in the CMS Innovation Center to explore a demonstration that would provide for an extended NTAP payment for gene therapies.

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[14] Gene Therapy Basics, American Society of Cell and Gene Therapy (Oct. 22, 2020), http://patienteducation.asgct.org/gene-therapy101/gene-therapy-basics.
[15] 85 Fed. Reg. at 58,436.
[16] *Id.*
[17] *Id.*
[18] 86 Fed. Reg. 44,774, 44,975 (Aug. 13, 2021).

Increased Add-on Payment to Better Enhance Provider and Patient Access

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. As part of an enhanced NTAP for gene therapies, **IGT requests that CMS increase the add-on payment for qualifying gene therapies to 100%**. CMS has increased the add-on payment for qualifying QIDPs and LPADs to be the lesser of: (1) 75% of the costs of the new medical service or technology; or (2) 75% of the amount by which the costs of the case exceed the standard DRG payment.[19][20] Given the transformative nature of gene therapies, it is essential that Medicare reimbursement does not impede access for Medicare beneficiaries nor stifle future innovation. An enhanced add-on payment would provide more certainty around expected reimbursement parameters for qualifying gene therapies administered in the hospital inpatient setting, thereby better facilitating patient access. While IGT has significant concerns about DRG payment systems and their ability to adequately reimburse for transformative therapies, CMS action to provide an increased add-on payment would be a notable step for the immediate future of gene therapy access.

III. Conclusion

The institute welcomes the 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

[19] 85 Fed. Reg. at 58,739
[20] 42 C.F.R. § 412.88(a)(2)(ii)(B).