

December 18, 2020

VIA ELECTRONIC DELIVERY

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

RE: Gene Therapy Policy Proposal for the Fiscal Year 2022 Medicare Inpatient Prospective Payment System Proposed Rule

Dear Administrator Verma,

The Institute for Gene Therapies (IGT) is writing to submit a policy proposal for the Centers for Medicare and Medicaid Services (CMS) to consider for inclusion in the Fiscal Year (FY) 2022 Medicare Inpatient Prospective Payment System (IPPS) rulemaking cycle. IGT commends CMS for taking action in recent years to provide designated New Technology Add-on Payment (NTAP) pathways as a mechanism for bolstering access to certain types of critical treatments. IGT submits this letter to respectfully request that CMS seek stakeholder feedback on a proposal to develop a gene therapy NTAP pathway to facilitate rapid provider and patient access to and enhanced reimbursement for gene therapies with Breakthrough Therapy designation, Priority Review designation, Accelerated Approval, or Regenerative Medicine Advanced Therapy (RMAT) designation.

Scientists have been working for decades to deliver on the promise of gene therapy, with a goal of revolutionizing treatment paradigms and replacing life-long chronic therapies with treatments intended for one-time administration or providing potentially curative therapies for diseases for which no treatment options exist. Scientists in the United States have delivered on this vision, with several transformative gene therapies receiving Food and Drug Administration (FDA) approval since 2017 and a robust pipeline of therapies for treating an array of life-threatening and devastating diseases. Payer systems, however, are in need of modernization to reflect the unique coverage, coding, and payment parameters necessary for facilitating long-term access to gene therapies. IGT urges CMS to begin to take action now for providing access to gene therapies in the inpatient setting through establishment of an NTAP pathway for gene therapies with Breakthrough Therapy designation, Priority Review designation, Accelerated Approval, or RMAT designation.

Introduction to the Institute for Gene Therapies

IGT was launched in February of 2020, with a focus on advocating 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 aims to inform the conversation regarding the value of transformative therapies and advocate for policies and practices to ensure patient access to these treatments. A full list of our members is available at <https://www.gene-therapies.org/advisory-councils>.

Proposal to Establish NTAP Pathway for Gene Therapies

IGT requests that CMS include in the IPPS Proposed Rule for FY 2022 a proposal to establish an 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 FDA anticipates that by 2025, the Agency will approve ten to twenty cell and gene therapies each year.¹ In 2020, FDA cited that the Agency had received approximately 900 Investigational New Drug (IND) applications specifically for gene therapies.² While not all of these gene therapies will be administered on an inpatient basis, the lack of clarity regarding payment parameters that will apply initially and long-term for these therapies under bundled payment systems like the IPPS is one of the most, if not the most, pressing patient access concern for the future of gene therapy. IGT looks forward to working with CMS on these longer-term reimbursement parameters as they apply to gene therapy but seeks to remain focused on the NTAP pathway for the purposes of this letter.

As CMS is aware, the FY 2021 IPPS Final Rule finalized a proposal establishing a new alternative NTAP pathway for therapies approved through the LPAD. In the FY 2020 IPPS Final Rule, CMS had finalized a proposal providing an alternate NTAP pathway for therapies receiving QIDP designation. The QIDP NTAP pathway began in FY 2021, while the LPAD NTAP Pathway will commence in FY 2022. These newly established alternative pathways will provide streamlined opportunities for novel therapies that qualify to receive NTAP under IPPS. IGT respectfully requests that CMS adopt a similar pathway for gene therapies to ensure rapid patient access to these cutting-edge technologies.

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

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.³ 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

¹ Press Announcement, Food and Drug Administration (FDA), Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on New Policies to Advance Development of Safe and Effective Cell and Gene Therapies (Jan. 15, 2019).

² Press Announcement, FDA, FDA Continues Strong Support of Innovation in Development of Gene Therapy Products (Jan. 28, 2020).

³ 85 Fed. Reg. 58,432, 58,435 (Sept. 18, 2020).

available, the diagnosis or treatment of Medicare beneficiaries.”⁴ CMS has similarly deemed medical devices that are part of FDA’s Breakthrough Devices Program to satisfy “newness” and “substantial clinical improvement” criteria.⁵ IGT respectfully requests that CMS advance a proposal to permit approved gene therapies with FDA designation of Breakthrough Therapy designation, Priority Review designation, Accelerated Approval, or 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).”⁶ 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.”⁷ 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.⁸ 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.⁹ 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.¹⁰ 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.”¹¹ 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.”¹² As emphasized in the outset of this letter, bringing gene therapy to fruition has taken decades of work for scientists, with an aim of revolutionizing how diseases are treated by addressing the cause of the disease itself.¹³ With the potential to cure and prevent rare and often life-threatening disorders, a gene therapy NTAP pathway is critical for providing a predictable and streamlined process for ensuring rapid access to qualifying gene therapies.

⁴ *Id.*

⁵ *Id.* at 58,606.

⁶ *Breakthrough Therapy*, FDA (Jan. 4, 2018), <https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy>.

⁷ *Priority Review*, FDA (Jan. 4, 2018), <https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review>.

⁸ FDA, *New Drug, Antibiotic, and Biological Drug Product Regulations; Accelerated Approval*, 57 Fed. Reg. 58,942 (Dec. 11, 1992).

⁹ *Accelerated Approval*, FDA (Jan. 1, 2018), <https://www.fda.gov/forpatients/approvals/fast/ucm405447.htm>.

¹⁰ *Regenerative Medicine Advanced Therapy Designation*, FDA (Oct. 5, 2020), <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/regenerative-medicine-advanced-therapy-designation>.

¹¹ 84 Fed. Reg. 42,044, 42,047 (Aug. 16, 2019).

¹² *What Is Gene Therapy? How Does It Work?*, FDA (Dec. 12, 2017), <https://www.fda.gov/consumers/consumer-updates/what-gene-therapy-how-does-it-work>.

¹³ *Gene Therapy Basics*, American Society of Cell and Gene Therapy (Oct. 22, 2020), <http://patienteducation.asgct.org/gene-therapy-101/gene-therapy-basics>.

2. Approval Timing Flexibility 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.¹⁴ Instead, CMS provides “conditional” NTAP for products through these pathways that do not receive approval by this deadline but otherwise satisfy NTAP criteria.¹⁵ 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 FY for which the applicant sought NTAP.¹⁶ IGT urges CMS to provide this same timing flexibility as part of a gene therapy NTAP pathway. 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 deadline is not met. These delays would be particularly 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, to the benefit of the patient’s short- and long-term health, their caregivers, the healthcare system (in the form of avoided costs), and society (in the form of increased productivity and reduced absenteeism).

3. 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. IGT 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. For purposes of this letter, however, we specifically request that CMS increase the add-on payment for qualifying gene therapies to seventy-five percent, as it has done through the QIDP and LPAD NTAP alternative pathways.¹⁷ More specifically, the add-on payment amount for qualifying QIDPs and LPADs is the lesser of: (1) seventy-five percent of the costs of the new medical service or technology; or (2) 75 percent of the amount by which the costs of the case exceed the standard Diagnosis Related Group (DRG) payment.¹⁸

In relation to the new LPAD pathway, CMS noted that “[the] LPAD pathway is used to treat a serious or life-threatening infection in a limited population of patients with unmet needs.”¹⁹ This same rationale applies strongly in the case of gene therapy, as many gene therapies are focused on rare diseases for limited patient populations with unmet needs. As such, IGT urges CMS to develop a similar construct to provide additional payment for gene therapies through an NTAP pathway. This enhanced add-on 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 maintains 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.

¹⁴ 85 Fed. Reg. at 58,436.

¹⁵ *Id.*

¹⁶ *Id.*

¹⁷ 85 Fed. Reg. at 58,739.

¹⁸ 42 C.F.R. § 412.88(a)(2)(ii)(B).

¹⁹ 85 Fed. Reg. at 58,737.

Conclusion

IGT appreciates the opportunity to submit this policy proposal for CMS consideration for the FY 2022 IPPS rulemaking cycle. The Institute respectfully requests that CMS take action to establish an NTAP pathway specific to gene therapies to ensure rapid eligibility upon approval and enhanced provider and patient access. IGT would be pleased to serve as a resource on gene therapy issues and answer any questions regarding these recommendations.

Sincerely,

A handwritten signature in black ink that reads "Erik Paulsen". The signature is written in a cursive, slightly slanted style.

The Honorable Erik Paulsen
Chairman
Institute for Gene Therapies

cc: Don Thompson, Director, Division of Acute Care

Jason Bennett, Acting Director, Technology Coding and Pricing Group