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January 26, 2021

VIA ELECTRONIC DELIVERY

The Honorable Elizabeth Richter
Acting Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS–5528–IFC
7500 Security Boulevard
Mail Stop C4–26–05
Baltimore, MD 21244-1850

RE: Comments on Most Favored Nation Model Interim Final Rule with Comment Period (CMS-5528-IFC)

Dear Acting Administrator Richter,

The Institute for Gene Therapies (IGT or "the Institute") submits these comments to the Centers for Medicare and Medicaid Services (CMS or "the Agency") on the Most Favored Nations (MFN) Model Interim Final Rule with Comment (MFN IFC).[1] The Institute is exceedingly concerned with the lack of proper procedure followed in finalizing the Model, the ways in which the MFN Model exceeds the Secretary's statutory authority, and the devastating impact it would have on patient access to medicines, particularly gene therapies. As delineated in these comments, IGT strongly supports the litigation efforts and arguments set forth against the IFC, which has culminated in the IFC being vacated in its entirety pending completion of the notice and comment process required under the Administrative Procedures Act (APA).[2] The Institute submits these comments to express our strong concerns regarding the Model contemplated in the IFC and urges CMS to refrain from future action to advance this or other such international price referencing demonstrations.

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 aims to inform the conversation regarding the value of transformative therapies and advocate for policies and practices to ensure

[1] 85 Fed. Reg. 76,180 (Nov. 27, 2020).
[2] Biotechnology Innovation Organization v. Azar, No. 20-cv-08603 (N.D. Cal. Dec. 28, 2020) (order granting preliminary injunction).

patient access to these treatments. A full list of our members is available at https://www.gene-therapies.org/advisory-councils.

IGT Supports the Litigation Challenging the Legality of the MFN Model IFC

Four primary lawsuits have been filed in United States (U.S.) District Courts challenging the IFC on procedural and substantive grounds.[3] IGT strongly supports these lawsuits and the decisions ordered by all four courts halting implementation of the MFN Model, which was slated to have begun on January 1, 2021.

In the U.S. District Court for the District of Maryland, the Association of Community Cancer Centers (ACCC), the Pharmaceutical Research and Manufacturers of America (PhRMA), the Global Colon Cancer Association, and the National Infusion Center Association assert that CMS exceeded the Agency's statutory authority in issuing the IFC. The ACCC lawsuit raises three significant areas of concern with the IFC: (1) substantive issues pertaining to the provisions contained in the IFC; (2) procedural issues regarding notice and solicitation of public comment; and (3) statutory authority issues concerning the Agency's use of the CMS Innovation Center to institute a mandatory nationwide payment model.[4]

IGT strongly supports the arguments and rationale set forth by plaintiffs in this litigation. More specifically, plaintiffs argue that, according to a CMS analysis of the IFC, Medicare beneficiaries could lose access to doctors and prescription drugs, and the Model would only lower out-of-pocket costs for approximately one percent of beneficiaries in Medicare Part B due to both supplemental coverage and the number of beneficiaries who do not take the medicines included in the demonstration. In addition, the lawsuit argues that CMS did not undertake proper administrative procedures when issuing the IFC. The lawsuit notes that by publishing an Interim Final Rule, CMS deprived stakeholders the opportunity for public comment. In addition, the litigation notes that while the Coronavirus (COVID-19) Public Health Emergency is cited as the reason for the Agency issuing an Interim Final Rule, the scope of the provisions contained in the MFN Interim Final Rule specifically carves out COVID-19 treatments and therapies. Finally, while Social Security Act Section 1115A authorizes CMS to conduct "tests" of new payment models under the Innovation Center, the MFN Model revises a significant portion of the Medicare Part B program on a mandatory and nationwide basis. Using the Innovation Center to facilitate these significant changes is beyond CMS' statutory authority.

On December 23, 2020, the U.S. District Court for the District of Maryland granted a temporary restraining order halting implementation of the MFN Model for fourteen days.[5] Subsequently, on January 6, 2021, the Court extended the temporary restraining order until January 20, 2021.[6] In the parallel case filed by the Biotechnology Innovation Organization (BIO), the California Life Sciences Association (CLSA), and Biocom California, the Court granted a preliminary injunction prohibiting CMS from implementing the IFC on the grounds that CMS did not follow notice and comment procedures required under the APA.[7] The order vacates the MFN in its entirety, pending completion of the notice and comment process. IGT supports the orders issued across these cases.

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[3] Assoc. Comm. Cancer Ctrs. v. Azar, No. 20-cv-03531 (D. Md. filed Dec. 4, 2020); Biotechnology Innovation Organization v. Azar, No. 20-cv-08603 (N.D. Cal. filed Dec. 4, 2020); Comm. Oncology Alliance v. Azar, No. 20-cv-03604 (D. D.C. filed Dec. 11, 2020); Regeneron Pharmaceuticals, Inc. v. United States Dept. of Health and Human Services, No. 20-cv-10488 (S.D. N.Y filed Dec. 11, 2020).

[4] Assoc. Comm. Cancer Ctrs. v. Azar, No. 20-cv-03531 (D. Md. filed Dec. 4, 2020).

[5] Assoc. Comm. Cancer Ctrs. v. Azar, No. 20-cv-03531 (D. Md. Dec. 23, 2020) (order granting preliminary injunction).

[6] Assoc. Comm. Cancer Ctrs. v. Azar, No. 20-cv-03531 (D. Md. Jan. 6, 2021) (order extending preliminary injunction).

[7] Biotechnology Innovation Organization v. Azar, No. 20-cv-08603 (N.D. Cal. Dec. 28, 2020) (order granting preliminary injunction).

IGT Urges CMS to Refrain from Advancing Any Future International Price Referencing Demonstration

The Institute is significantly concerned about the negative implications the IFC poses for drugs and biologicals covered under Medicare Part B, as set forth in the ongoing litigation. With the IFC now vacated, the Institute urges CMS to refrain from initiating any future international price referencing demonstrations for Part B drugs and biologics. While these types of price control demonstrations are harmful to provider and patient access to specialty drugs and biologicals, they would be particularly devastating if applied to gene therapy.

The science behind gene therapies has been decades in the making, and its arrival marks a high point in scientific innovation and shifts in patient treatment paradigms. Gene therapies have the potential to revolutionize our healthcare landscape, replacing life-long chronic treatment with therapies intended for one-time administration and providing potentially curative therapies for diseases for which no treatment options exist. Transformative therapies can not only extend but also enhance the quality of life for individuals afflicted by rare genetic diseases. The approval of several gene therapies, with more on the horizon, has increased the need to modernize payer systems to reflect the unique coverage, coding, and payment parameters necessary for facilitating long-term access to gene therapies while preserving healthcare system sustainability.

The institution of draconian price control measures, such as international price referencing, would be exceedingly harmful for provider and patient access to these transformative therapies. Of most concern, many of the reference countries use the quality adjusted life year (QALY) as a metric to assess a product's "value" and dictate an artificial price. Notably, the Affordable Care Act prohibited the use of evidence or findings from comparative clinical effectiveness research as a threshold to determine Medicare coverage and reimbursement in a manner that "treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill."[8] Importing foreign prices informed by this discriminatory metric counters the Agency's touted purpose of the MFN Model IFC of "preserving or enhancing quality of care furnished to Medicare beneficiaries[.]"[9] In addition, a report issued by the U.S. National Council on Disability (NCD) found that implementing price control measures, such as certain countries' use of QALYs to make benefit and coverage decisions, has led to a decline in patient access to medications.[10] When these types of price controls are enacted, subjective benchmarks assessing cost-effectiveness often take precedence over patient needs.

CMS itself has recognized that application of the MFN IFC to gene therapies would be problematic, noting that it is –

> considering whether we should exclude certain gene and cell therapies based on supply chain criteria, similar to our policy to exclude vaccines and compounded drugs. For future years, we seek comment on whether we should exclude certain gene and cell therapies or new drugs for the treatment of rare diseases and conditions from the MFN Model, and how CMS would identify such drugs for exclusions, particularly how we would define such drugs, identify rare diseases and conditions for purposes of the MFN Model, and determine the appropriate length of such exclusion, for example, all performance years or several years after the drug is first sold in the U.S.[11]

[8] Social Security Act § 1182(c)(1).
[9] 85 Fed. Reg. at 76,181.
[10] NATIONAL COUNCIL ON DISABILITY (NCD), QUALITY-ADJUSTED LIFE YEARS AND THE DEVALUATION OF LIFE WITH DISABILITY (2019).
[11] 85 Fed. Reg. at 76,191.

IGT urges CMS to refrain from moving forward with the MFN IFC in any fashion for any drugs or biologicals, including for gene therapies.

In addition to the concerns set forth in the litigation and the provider and patient access concerns described above, a variety of unique considerations apply to gene therapies. IGT is concerned that interference with these dynamics through the MFN Model or other demonstrations could result in provider and patient access issues and broader ramifications on the gene therapy industry. Several of these issues include, but are not limited to:

- Rare and Ultra-rare Patient Populations: Gene therapies for rare and ultra-rare genetic diseases may have very low volume across payers or very low volume in Medicare. The institution of severe price control measures, such as those in the MFN Model, would pose significant negative ramifications to beneficiaries by establishing barriers to provider and patient use of gene therapies. At a time when gene therapy stakeholders are working intensively to develop reimbursement solutions to facilitate appropriate reimbursement and access, these types of Models would substantially delay or effectively deny access for Medicare patients.

- Ex-U.S. Rights: Given the resources required to commercialize highly specialized technologies in the diverse ex-U.S. markets, small gene therapy companies often use out-licensing agreements with other companies for ex-U.S. gene therapy commercialization, including price setting in ex-US countries. As such, the U.S. companies do not control the pricing of the drug sold outside of the U.S. Comparing the cost of a drug sold in the U.S. to other countries in which, due to out-licensing agreements, U.S. manufacturers have no control over international pricing, would not be justified under the MFN Model or any future Part B drug payment demonstration.

- Supply Chain Considerations: In the IFC, CMS discusses the exclusion of certain drugs that are acquired outside of the typical supply chain.[12] Specialized supply chains are typically necessary for gene therapies, such as cold-/frozen-chain requirements for distribution, close coordination among supply chain stakeholders, just-in-time delivery due to self-life limitations, controlled operating conditions, and facilitation by digital platforms, patient services, or hubs.[13] In addition, the "bespoke" manufacturing processes for gene therapies are often outsourced due to complexity.[14]

- Drug Selection Logic: The review of annual allowed charges methodology for drugs to be included in the MFN IFC is not compatible with cost considerations for gene therapies. Transformative therapies intended for one-time administration have the potential to replace a lifetime of medical and non-medical costs and should not be assessed under the type of Model CMS has developed. Including these types of therapies in the MFN Model or any future Part B drug payment demonstration would be imprudent as gene therapies do not encompass the same types of recurring, stable costs as drugs that treat chronic conditions over a patient's lifetime.

[12] *Id.* at 76,190.
[13] *See*, *e.g.*, Sanjay Srivastava et al., *Transforming Next-Gen Therapy Supply Chains Into Patient-Connected Value Chains*, CELL & GENE (June 20, 2019), https://www.cellandgene.com/doc/transforming-next-gen-therapy-supply-chains-into-patient-connected-value-chains-0001.
[14] *Id.*

Conclusion

The Institute appreciates the opportunity to submit comments to CMS on the MFN Model IFC. The Institute strongly supports the ongoing litigation challenging the Model on procedural and substantive grounds. IGT urges CMS to refrain from any future action to advance a similar concept given the significant risks the Model poses to Medicare beneficiary and provider access to specialty drugs and biologicals. IGT would be pleased to answer any questions regarding the issues raised in these comments or to serve as a resource on gene therapy issues in 2021 and beyond.

Sincerely,

Lauren Randall Buckley, JD
Director, Health Policy & Advocacy