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January 22, 2024

The Honorable Bill Cassidy
Ranking Member of the Health, Education, Labor, and Pensions Committee
United States Senate
Washington DC, 20510

RE: RFI on Improving Americans' Access to Gene Therapies

Dear Ranking Member Cassidy,

The Institute for Gene Therapies (IGT or "the Institute") is grateful for the opportunity to respond to your Request for Information (RFI) on Improving Americans' Access to Gene Therapies. IGT supports Congressional efforts to realize the value of transformative therapies for patients, caregivers, the healthcare system, and society at large. IGT was launched in February 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access.

Through our Patient Advocacy Advisory Council, Corporate 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. Our response to this RFI indicates our perspective as a membership group and focuses on areas where our members have firsthand experience or knowledge.

A complete list of our members is available at https://www.gene-therapies.org/about-igt.

I. Executive Summary

In 2023, the Food and Drug Administration (FDA) approved four gene therapies and a gene-editing cell therapy[1]. This more than doubled the number of FDA-approved gene therapies on the U.S. market and increased the number of patients who could potentially benefit by an order of magnitude – most notably through the approval of two therapies for sickle cell disease (SCD). The RFI contemplates a scenario where the increasing pace of gene therapy approvals results in a fiscal breakdown in the commercial markets or with government payers, with accompanying patient affordability challenges due to the rare nature of the diseases these therapies treat.

IGT continues to encourage Congress to lean in on the benefits of innovative financing models, including value-based payment arrangements (VBAs). To date, IGT members have not experienced significant barriers when working with payers on coverage issues, including VBA development, in the commercial markets, and patients

[1] In chronological order, see: (1) https://www.fda.gov/vaccines-blood-biologics/vyjuvek; (2) https://www.fda.gov/vaccines-blood-biologics/tissue-tissue-products/elevidys; (3) https://www.fda.gov/vaccines-blood-biologics/roctavian; (4) https://www.fda.gov/vaccines-blood-biologics/lyfgenia and (5) https://www.fda.gov/vaccines-blood-biologics/casgevy.

have primarily been afforded access to these therapies through existing legal structures. On the other hand, barriers around VBAs in the Medicaid program continue to exist that can also indirectly hinder progress in the commercial markets.

Because these therapies are still new – with manufacturers, payers, intermediaries, and providers all working through the treatment journey for the first time with these patients - IGT recommends modest reforms in the commercial markets to align some coverage processes across different plan types. Moreover, IGT suggests additional reforms to address some patient affordability concerns and several Medicaid reforms that would enhance access and patient affordability. IGT currently does not support a "curative therapy" coverage requirement in the commercial / employer markets.

II. RFI Topic Responses

1. What treatments should be included?

IGT has consistently supported regulatory flexibility for coverage of curative therapies and does not believe a wholly new legislative framework is needed for now as commercial payers are largely covering these therapies. However, if payers began to widely exclude coverage to the point a new framework was appropriate, limiting eligibility to "ultra-rare" populations would be cumbersome, as no current definition for ultra-rare currently exists. Creating a new definition would likely be arbitrary and potentially discriminatory to particular patient groups. Moreover, many gene therapies in development target less rare diseases. For example, sickle cell disease, which now has an FDA-approved gene therapy and a gene editing technology, is not ultra-rare, but the newly approved therapies to cure the disease are ideal candidates for outcomes-based arrangements. Gene therapy developers should be incentivized to continue seeking cures for any condition with high unmet medical need.

This broad approach to eligibility is also consistent with the immense value curative therapies can bring to the system. Any FDA-approved curative therapy has the potential to decrease downstream healthcare costs significantly, even more so for larger patient populations and no less so for smaller ones.

2. What is the Current Practice for Patients with Ultra-Rare Diseases or Disorders?

Patients working to access gene therapies face a variety of potential challenges. The experience can be long, complicated, and daunting for these populations who can access therapies for their diseases often for the very first time. Many stakeholders, ranging from manufacturers to non-profit programs, provide patient assistance programs to help patients navigate their journey from diagnosis to receiving a gene therapy. These programs almost always begin with providing "know-how" resources such as information on a patient's available insurance benefits, explanations of treatment logistics, identification of financial assistance options, and ongoing educational support.

In the commercial market, many stakeholders can provide patient financial assistance, including manufacturers. For example, a child living in Maryland who is covered by insurance and then diagnosed with a genetic or rare disease could be helped by 103 different financial assistance programs, according to the Patient Advocate Foundation[2]. In practice, while the sheer number of these programs can be challenging to navigate alone, the unique nature of gene therapy has facilitated direct relationships between patients and disease groups or manufacturers offering legitimate expertise in arming patients to apply and access their eligible assistance programs.

[2] See here: https://www.patientadvocate.org/explore-our-resources/national-financial-resource-directory/

However, employers sometimes engage alternative funding programs, some of which work with the employer health plan to exclude coverage of certain treatments and specialty drugs, and in turn establish patient access to treatments via alternate routes, like manufacturer patient assistance programs. A manufacturer-sponsored patient assistance program is intended for uninsured or low-income patients, not beneficiaries who have employer-sponsored health insurance and pay premiums for that insurance. Nonetheless, when this practice occurs, the alternative funding program often receives a payment from the employer; while the manufacturer pays the full cost of the drug and the employer incurs no cost. This establishes a perverse incentive and does not allow the patients who most need assistance to take advantage. IGT would support reforms to curtail this type of behavior and ensure these programs are kept available for the patients who truly need it.

IGT also supports changes to the federal fraud and abuse laws so patients enrolled in Medicare and Medicaid can access patient assistance programs. For example, Congress should address existing barriers for patients who must travel to receive specialized care at a Center of Excellence or who require ancillary services as part of their treatment journey. Antikickback statute (AKS) currently hinders manufacturers from being able to help provide financial support for travel, lodging, and meals for patients and their families. Some gene therapies also require patients to undergo myeloablative conditioning before receiving a gene therapy. This process leaves many patients unable to reproduce, where adult patients will seek out cryopreservation and, in the future, fertilization services to bear offspring. Financial assistance is limited for patients in this area. IGT would welcome new AKS safe harbors for manufacturer support of ancillary services, or additional affordability barriers for these services will replace traditional deductible and copay considerations.

Many of the challenges surrounding patient travel are exacerbated in State Medicaid programs. Out-of-state provider enrollment is a significant cause of access delays, and legislation such as the Accelerating Kids' Access to Care Act (H.R. 4758 / S. 2372) would eliminate some of the challenges concerning enrollment. Moreover, Medicaid guidelines for patient travel and lodging per diem or remuneration vary significantly by state. As gene therapy treatment is likely to remain highly specialized within various Centers of Excellence, Congress should examine setting a reasonable floor for State Medicaid programs to provide covered financial assistance to its beneficiaries traveling out of state.

Finally, programs like copay accumulators remove the impact of any financial assistance that gene therapy patients can receive. IGT supports the passage of the Help Ensure Lower Patient (HELP) Copays Act (H.R. 830 / S. 1375), which would allow financial assistance to count towards that patient's deductible and copays, greatly enhancing the affordability of gene therapies.

3. How Do Plans and Payers Currently Manage Financial Risk?

IGT does not currently include health plans, benefit consultants, or plan brokers in our membership. Nonetheless, IGT is concerned about the potential proliferation of utilization management tools for gene therapies in the commercial market. These practices are currently rare, and commercial insurers are covering these therapies. There have, however, been a few instances where commercial payers have denied or delayed coverage of gene therapy to a patient via "lasering" out coverage of treatments for a given rare disease[3]. Most, if not all, of these denials have eventually been overturned via the appeals process. However, given the time-sensitive nature of delivering gene therapies, the ability of gene therapy patients to quickly bring in a peer-to-peer review of any coverage decision is critical.

[3] See here: https://www.statnews.com/2023/08/16/duchenne-muscular-dystrophy-sarepta-elevidys-gene-therapy-eligible-boys/

The Affordable Care Act (ACA) requires ACA-compliant plans to allow patients to seek external review, but these reviews can be problematic. In gene therapy, external reviews are more commonly limited due to a lack of meaningful expertise from a particular reviewer. In the case cited above, an emergency room doctor was tasked with reviewing an appeal for a patient seeking coverage for a recently approved gene therapy to treat a rare neuromuscular disease. This is clearly inappropriate, and it resulted in the denial of an on-label use of gene therapy for a Duchenne muscular dystrophy patient. Current law grants the Food & Drug Administration (FDA) significant authority to waive appropriate experts onto their Advisory Committee panels to ensure the agency receives adequate input. Seeking similar expertise for external reviews as part of the appeals process in the commercial markets should be considered as a requirement.

IGT is aware of the perceived threat of systemic financial risk posed by the increasing utilization of high-priced gene therapies in the commercial markets, particularly in the small-group plan or smaller self-funded plan markets. There have been concerns about these therapies being "lasered" out of contracts or how the presence of, or lack thereof, stop-loss policies could lead to access barriers for gene therapy patients. However, IGT members have yet to see these issues arise. They are instead optimistic about current trends and solutions in the commercial market via flexible financing models such as VBAs or pay-over-time arrangements.

The commercial market has the tools to continue fostering coverage of these therapies. As stated previously, IGT prioritizes the need for flexibility in the contract design so financing models can remain appropriately tailored to patients and the disease states they treat. For now, it does not appear there are any additional legal requirements, such as coverage mandates, that are needed to allow this kind of innovation.

IGT cannot speak to how plans and payers evaluate the financial benefit of covering gene therapy. However, IGT has laid out a core set of value principles that should be incorporated when discussing the value of gene therapy. These value principles can be found here: https://www.gene-therapies.org/value-of-gene-therapies.

IGT is also concerned about using quality-adjusted life-years (QALYs) as the baseline metric in value assessment. While it does allow for standard comparison exercises, the QALY has structural issues that devalue the life-years of certain patients and should be avoided or caveated when assessing the value of gene therapies, particularly when patients are likely to have significant differences in patient-reported quality of life outcomes than what the QALY could factor.

IGT members are concerned about recent decisions by commercial and government payers to restrict product coverage if approved via the FDA's Accelerated Approval pathway. It is not the job of a health plan, a State, or the Centers for Medicare and Medicaid Services (CMS) to relitigate the FDA's review. Efforts to label a drug as "experimental" based solely on the approval pathway are arbitrary and specifically detrimental to gene therapy access. As might be expected, assessment of surrogate endpoints is especially important for diseases that progress over prolonged periods or in diseases where functional outcome measures may be subject to much variability. Restricting coverage based on the use of surrogate endpoints is a rejection of the science underpinning gene therapy. IGT supports prohibiting coverage restrictions for products approved via the accelerated approval pathway, based solely on said accelerated approval, in private and government markets.

In Medicaid, patients face even more challenges when working to access gene therapies. Recent regulation, most notably the Medicaid "Multiple Best Price" Rule[4], has allowed manufacturers to begin offering gene therapies via VBAs without triggering problems in both the commercial market and Medicaid program without triggering significant problems with reporting Medicaid Best Price (BP). This regulation should be enshrined in

[4] Available here: https://www.regulations.gov/document/CMS-2020-0072-30223

statute to protect it from potentially poor future rulemaking, and IGT supports passage of the Medicaid VBPs for Patients (MVP) Act (H.R. 2666), which would codify this rule while making small but necessary changes to other programs that bolster and strengthen the VBA environment based on the multiple best price approach.

Nonetheless, states continue to need help understanding the perceived requirements placed on them when exploring their options to enter VBAs covering gene therapy. The rule appropriately lowers the burden for states, making the entire program voluntary for states while also requiring manufacturers to offer any effectuated VBA terms to all states, ensuring states can access the best VBA contracting terms available in other states or the commercial market. However, at the federal level, CMS should be more active in providing and disseminating detailed guidance to all states on the requirements they need to meet to engage in a VBA through this pathway. In the experience of IGT members, many states perceive the pathway of approvals and signoffs to be much longer and higher than CMS would tell them directly. This misperception directly delays access to these therapies as states grapple with trying to pay for these therapies upfront. Congress should urge CMS to provide greater clarity to States on the specific requirements states must complete to use a VBA based on the multiple best price approach.

Timely coding for gene therapies is another challenge in the Medicaid markets, as several states delay coverage until a product-specific J-code is available for physician administered drugs, such as gene therapies. IGT supports speeding the availability of code creation, including the ability for CMS to begin communicating with the FDA pre-approval to specifically address potential coding issues.

4. How do Manufacturers Price and Design Contracts for Treatment of Ultra-Rare Disease?

As stated in multiple other places in this document, IGT supports flexibility in contract design for financing gene therapies. Innovative payment arrangements often take years to develop, negotiate, and implement. Both sides need time to research the eligible patient population, create financial modeling tools to estimate budgets over time, and then agree to operational elements - starting with broad agreement terms and then contractual specifics such as payment timing, reconciliation, and adjudication. For value-based or outcomes-based arrangements, this process is also layered with contractual elements to identify agreed-upon patient outcomes and then how to track those outcomes over time.

This process currently affords manufacturers and payers adequate flexibility to design these contracts for their specific beneficiaries and disease states. Thus, while the process is becoming more typical, the agreements are often not. As more and more VBAs are adopted, there will be some significant overlap in the design of these contracts. However, we do not support the concept of templated contract designs, as this begins to erode the core principle of design flexibility. Experience in this process will benefit contract design more than new regulations or legislation to expedite the process through a “one size fits all” standardized contract approach.

The IGT value principles (https://www.gene-therapies.org/value-of-gene-therapies) again point to a multitude of factors involving the given disease state, treatment approach, system impact, and societal benefit that could and should be part of any pricing discussion.

5. How Do Supply Chain Intermediaries Price and Design Contracts?

IGT does not currently include any supply chain intermediaries among its membership and cannot speak to specific strategies in designing their contracts. However, IGT is wary of supply chain intermediaries applying practices for conventional drugs and biologics, namely administrative fees charged as a percentage of list price, into their gene therapy services. Congress should work to ensure transparency from these intermediaries to

avoid egregious percentage-based markups that ultimately hamper affordability and bring additional drains on the healthcare system.

6. How Do Physicians Provide Access to These Therapies?

IGT is not a physician or provider organization and does not wish to comment on the specific questions in this section. However, IGT is concerned about current and proposed policies that leave providers underwater when administering gene therapy, particularly in the Medicaid inpatient setting. Notably, CMS released a proposed rule[5] in May of 2023 that would change the definition of "Covered Outpatient Drug" to include instances where a therapy is merely "separately identified" on a claim form as a form of "direct reimbursement." This proposed change would essentially make all gene therapies administered in the inpatient setting a "covered outpatient drug," regardless of whether the treatment was actually paid separately. As a result, states would presumably be authorized to seek Medicaid rebates from manufacturers, irrespective of whether the therapy was separately paid for. The end effect is that hospitals can be reimbursed according to the standard inpatient bundled rate, which will not include any resources for providing gene therapy for years (if ever), while the state collects rebates on said therapy. Congress should seek to halt this proposed rule from being finalized as it threatens the ability of hospitals to provide gene therapies.

III. Conclusion

IGT greatly appreciates the Senator's interest in ensuring access to gene therapies. There are numerous challenges in the current commercial payer markets, but the sky is not falling. IGT members are experiencing adequate coverage of gene therapies and gene editing technologies. While problems may arise in the future regarding portability, affordability, or coverage, we do not believe any legislative changes are necessary at present, as we think interested stakeholders have adequate tools available to manage these concerns, absent foul play. Per the old proverb, "Where there is a will, there is a way," and for now, the transformative value that gene therapies can bring to the healthcare ecosystem continues to drive interest in solving these challenges. Nonetheless, Congress should seek to pass legislation such as the HELP Copays Act and contemplate additional AKS reforms to ensure patients receive the support they need to access these therapies.

In Medicaid, more challenges exist. Congress should enact the MVP Act, the Accelerating Kids' Access to Care Act, and halt regulatory overreach that would harm innovation and undermine inpatient hospitals that provide gene therapy. Thankfully, the recently effectuated "multiple best price" approach is likely to be a cornerstone regulatory policy that will foster the utilization of innovative financing models in both Medicaid and the commercial markets for years to come.

Thank you again for the opportunity to provide this information.

Sincerely,

Kenneth L. Hodge
Director, Federal Affairs
Institute for Gene Therapies

[5] Available here: https://www.regulations.gov/docket/CMS-2023-0092