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November 2, 2020

VIA ELECTRONIC DELIVERY

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS–3372–P
P.O. Box 8016
Baltimore, MD 21244–8010

RE: Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of ''Reasonable and Necessary'' Proposed Rule [CMS-3372-P]

Dear Administrator Verma,

The Institute for Gene Therapies (IGT or "the Institute") appreciates the opportunity to submit these comments to the Centers for Medicare and Medicaid Services (CMS or "the Agency") on the "Medicare Coverage of Innovative Technology (MCIT) and Definition of 'Reasonable and Necessary'" Proposed Rule ("Proposed Rule").[1] Our comments respond to CMS' request for feedback on whether to expand the MCIT pathway to include drugs and biologics, as well as the proposal to codify a regulatory definition of Medicare's "reasonable and necessary" coverage criteria. IGT recommends that CMS finalize its proposal to limit the MCIT pathway to medical devices, as Medicare's laws and guidance provide existing mechanisms to streamline coverage and facilitate patient access to drugs and biologics, including gene therapy, once approved by the Food and Drug Administration (FDA). IGT also respectfully recommends that CMS clarify that the proposed "reasonable and necessary" regulatory definition does not apply to drugs and biologics. If the Agency does not take this approach, then IGT strongly urges CMS to either: (1) refrain from applying the elements of the proposed "reasonable and necessary definition" that extend beyond the existing definition in the Medicare Program Integrity Manual (*e.g.*, using commercial insurance coverage policies for determining Medicare coverage policies) to drugs and biologics; or (2) enforce the new proposed definition of "reasonable and necessary" solely in the case where it would result in expanded Medicare coverage for drugs and biologics.

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

[1] 85 Fed. Reg. 54,327 (Sept. 1, 2020).

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.

I. Opening of the MCIT Pathway to Drugs and Biologics is Not Necessary or Recommended

CMS is proposing to establish a new coverage pathway under Medicare specifically for innovative medical devices with breakthrough designation from the FDA. The Agency states that the overall goal of the MCIT pathway is to increase Medicare beneficiary access to these devices by providing immediate coverage upon FDA approval. While CMS proposes to limit the pathway to breakthrough devices, it requests feedback on whether the MCIT pathway should also include diagnostics, drugs, and/or biologics that utilize breakthrough or expedited approaches through the FDA (*e.g.*, Breakthrough Therapy, Fast Track, Priority Review, Accelerated Approval) or all diagnostics, drugs, and/or biologics.[2]

IGT appreciates the Agency's focus on, and efforts to facilitate access to, technologies that the FDA designates as addressing an unmet medical need. IGT agrees that it is critical to ensure that CMS' coverage and reimbursement policies are modernized to keep pace with advancements in technologies across the medical field. The Institute also recognizes, however, that differences exist in the coverage standard applied to medical devices and the standard applied to drugs and biologics. Those differences drive our recommendation that CMS refrain from expanding the MCIT pathway to include drugs or biologics. Medicare coverage upon FDA approval of drugs and biologics is available for those products meeting the "reasonable and necessary" statutory definition in Section 1862(a)(1)(A) of the Social Security Act (SSA) and Chapter 13 of the Medicare Program Integrity Manual, along with Part B statutory criteria for drugs and biologics in Section 1861 of the SSA and implementing regulations and guidance. Where CMS applies these criteria, they have contributed to facilitating timely patient access to Part B drugs and biologics upon FDA approval. Moreover, the Agency has the discretion to institute more formal coverage parameters through National Coverage Determinations (NCDs) or to allow Medicare contractors to do so through Local Coverage Determinations (LCDs).

Opening the MCIT pathway to drugs and biologics, including gene therapies, would not result in meaningful improvements in Medicare Part B coverage and could have unintended, negative consequences for these therapies based on the focus of operationalizing MCIT in the context of medical device regulatory and reimbursement standards. IGT commends the Agency, however, for considering the need to facilitate beneficiary access in an efficient and effective manner to novel therapies that FDA has identified as likely to have substantial impact on diseases with significant unmet medical needs.

II. CMS Should Clarify that the Reasonable and Necessary Regulatory Definition is Limited to Medical Devices

CMS proposes to establish a new regulatory definition of "reasonable and necessary" in Section 405.201 of the Code of Federal Regulations. In addition to the definition currently included in the Program Integrity Manual, CMS indicates that it would add a separate basis under which an item or service is considered "appropriate" based on commercial health insurer coverage policies. This new commercial market analysis would apply in cases where an item or service does not meet the existing criteria for being considered "appropriate" for Medicare patients but is determined to be safe and effective and not experimental or investigational.[3]

[2] *Id.* at 54,331.
[3] *Id.* at 54,332.

IGT understands these proposals are focused on revising the "reasonable and necessary" criteria to provide flexibility for medical devices in obtaining coverage in Medicare, opening the door to permitting stakeholders to reference commercial insurance plan coverage as grounds for attaining Medicare coverage. In fact, CMS proposes to codify its proposed regulatory definition of "reasonable and necessary" in a portion of Medicare regulations focused specifically on medical device coverage issues. On these grounds, IGT finds it confusing that CMS states in the Fact Sheet and Press Release issued with the Proposed Rule that it intends for this new regulatory definition to apply to all Medicare items and services. In the Fact Sheet, CMS notes: "In this proposed rule, we are proposing to codify a definition of 'reasonable and necessary' for items and services that may be covered under Part A and Part B of the Medicare program. This proposed definition would apply to all Medicare items and services, not just those covered through the MCIT pathway."[4] Similarly, the Press Release states that "the MCIT proposed rule would clarify the standard CMS uses to determine whether Medicare should cover a product, like a drug, device, or biologic."[5] IGT recommends that CMS specify in the Final Rule that this proposed regulatory definition does not apply to drugs or biologics.

The Agency's proposal to apply a revised definition of "reasonable and necessary" to drugs and biologics in terms of appropriateness for Medicare beneficiaries is unnecessary and could result in unintended consequences. As indicated in the preceding section of these comments, the existing statutory and manual definition of "reasonable and necessary," along with the Part B drug and biologic criteria, have facilitated streamlined coverage for and beneficiary access to drugs and biologics, including gene therapies. IGT does not support instituting the new proposed regulatory definition, as it is not fit-for-purpose for the critical medicines that beneficiaries and their treating providers may seek to access under existing coverage standards. IGT is concerned that changes to this definition could create uncertainty regarding Medicare coverage and potentially result in unintended detrimental impacts for provider and patient access and, in turn, downstream negative impacts on program spending (*e.g.*, resulting from increased hospitalizations and emergency department use as a result of decreased access to needed therapies).[6]

IGT appreciates that the experience of medical devices in obtaining coverage is different from drugs and biologics and, thus, recommends that CMS make clear that any finalized version of this new definition does not apply to drugs and biologics. IGT emphasizes that it does not take a position on this definition as it applies to medical devices or whether it should be finalized in relation to medical devices. To the extent CMS finalizes this definition for medical devices, however, IGT recommends the following revision to the opening of the proposed regulatory definition of "reasonable and necessary"[7] (revisions underlined):

§ 405.201 Scope of subpart and definitions.

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(b) * * *

[4] Fact Sheet, Centers for Medicare and Medicaid Services (CMS), Proposed Medicare Coverage of Innovative Technology (CMS-3372-P) (Aug. 31, 2020).
[5] Press Release, CMS, CMS Acts to Spur Innovation for America's Seniors (Aug. 31, 2020).
[6] A 2012 Congressional Budget Office (CBO) review of the link between prescription drug spending and other categories of healthcare utilization costs is emblematic of this example: Congressional Budget Office (CBO), Offsetting Effects of Prescription Drug Use on Medicare's Spending for Medical Services (November 29, 2012), available at: https://www.cbo.gov/publication/43741 (last accessed October 18, 2020).
[7] 85 Fed. Reg. at 54,338.

Reasonable and necessary means that an item or service categorized as a medical device is considered—

If CMS does not adopt the Institute's recommendation, then IGT recommends that CMS only implement the proposed "reasonable and necessary" definition for drugs and biologics in one of two ways: (1) refrain from applying the elements of the proposed "reasonable and necessary definition" that extend beyond the existing definitions in the Program Integrity Manual (*e.g.*, using commercial insurance coverage policies for determining Medicare coverage policies) to drugs and biologics; or (2) enforce the new proposed definition of "reasonable and necessary" solely in the case where it would result in expanded Medicare coverage for drugs and biologics.

Conclusion

The Institute appreciates CMS' recognition of the need for establishing the MCIT pathway and revision to the "reasonable and necessary" requirements to facilitate patient access to medical devices. The inclusion of drugs and biologics, including gene therapies, in these proposals, however, is not necessary or warranted. Accordingly, IGT recommends that CMS exclude drugs and biologics from both the MCIT pathway and the proposed regulatory definition of "reasonable and necessary." IGT would be pleased to serve as a resource on gene therapy issues during this process and answer any questions regarding these comments.

Sincerely,

Lauren Randall Buckley, JD
Director, Health Policy & Advocacy