November 2, 2020

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Re: CMS-3372-P - Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary"

Dear Administrator Verma:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services (CMS) proposal to revise Medicare coverage policies in CMS-3372-P.

ASGCT is a nonprofit professional membership organization comprised of more than 4,400 scientists, physicians, and other professionals working in gene and cell therapy in settings such as universities, hospitals, and biotechnology companies. Many of our members have spent their careers in this field performing the underlying research that has led to today's robust pipeline of transformative therapies.

A core portion of the Society's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. Therefore, the accessibility of such therapies to patients is of paramount importance to ASGCT. The Society supports full coverage, and sufficient reimbursement of providers, for approved therapies, and payment models that foster patient access. These positions do not imply endorsement of any individual pricing decisions.

We are encouraged that CMS recognizes the significance of facilitating access to innovation by proposing immediate, predictable coverage concurrently with FDA market authorization for breakthrough-designated medical devices for Medicare beneficiaries. ASGCT appreciate CMS's request for public comment on whether this Medicare Coverage of Innovative Technology (MCIT) pathway should also include diagnostics, drugs and/or biologics that utilize breakthrough or expedited approaches or all diagnostics, drugs, and/or biologics. ASGCT recommends that a clear pathway to coverage be available for transformative potentially durable gene and cell therapies to ensure Medicare patient access as soon as possible after FDA approval. We recommend consideration of the specific provisions of such a pathway prior to application to gene and cell therapies, however, to ensure incorporation of procedures that provide maximal patient access throughout the coverage process, as indicated below.Gaining approval from the

Food and Drug Administration (FDA) is a critical first step to patient access that ensures that new drugs and biologics are safe and effective. This is a rigorous process that for novel gene and cell therapies continues after approval/licensure through post-market monitoring.[1] Upon FDA approval, current CMS processes for determining coverage of these products have resulted in limiting the accessibility of these impactful, sometimes life-saving treatments for Medicare beneficiaries.

Consider the example of chimeric antigen receptor T-cell (CAR T-cell) therapy. The FDA approved two CAR T-cell therapies—Kymriah and Yescarta—in 2017. In May 2018, CMS opened a National Coverage Analysis, creating uncertainty for providers regarding whether these therapies would continue to be covered as they had been under local MAC determinations post-approval. ASGCT appreciates that CMS concluded this process by creating a National Coverage Determination that provides coverage in all appropriate treatment sites for FDA-approved indications (according to the FDA-approved label for that product) or for other uses when the product has been FDA-approved and the use is supported in one or more CMS-approved compendia. Significantly, therapies that receive FDA approval in the future for use in earlier lines of treatment would be covered under this NCD. However, this process took over a year, and the original proposition in the proposed Decision Memo resulting from the National Coverage Analysis (NCA) would have resulted in patient access restrictions by placing excessive strain on providers who opted to provide the therapy.

Concurrently, the Inpatient Prospective Payment System (IPPS) provided insufficient reimbursement for CAR-T therapy which will continue for some providers, despite recent progress in reimbursement methodology, depending on application of adjustments, institutional cost-to-charge ratios, and charging practices. This combination of coverage and reimbursement challenges has contributed to the low number of beneficiaries who have accessed those therapies—fewer than 100, not including those participating in clinical trials—as CMS noted in the final FY 2021 IPPS rule. We are concerned from this high-profile case study in CMS treatment of transformational gene therapies that current practices could have a chilling effect on this pipeline unless there is clear action to modernize the system.

Given this, we urge CMS to develop a mechanism for rapid coverage of breakthrough- and regenerative medicine advanced therapy (RMAT)-designated gene and cell therapies coming to market. ASGCT believes that a coverage pathway should be aligned with FDA-approved indications, sites of care, and post-marketing obligations, as well as considering beneficiary access to providers and other needs of these populations. If such a coverage pathway were to include National Coverage Determinations, ASGCT encourages CMS to allow for stakeholder

[1]U.S. Department of Health and Human Services, Food and Drug Administration, Center for Biologics Evaluation and Research (2020, January). *Long Term Follow-up After Administration of Human Gene Therapy Products: Guidance for Industry*. Retrieved from https://www.fda.gov/media/113768/download.

input prior to finalization to ensure that coverage is sufficiently broad to include potential future indications, patient populations, sites of care, and lines of treatment.

By establishing a clear coverage pathway for these products, CMS could eliminate significant marketplace uncertainty for potentially durable treatment for patients suffering from blood cancers, sickle cell disease, and other devastating and costly conditions. By eliminating this uncertainty, CMS can help bridge the gap between Medicare beneficiaries and these new therapies.

We appreciate the opportunity to share our perspective on this proposal. Please do not hesitate to contact us with any questions.

Sincerely,

David Barrett, JD, MS
Chief Executive Office