



July 16, 2018

***By Electronic Delivery***

Aloysius B. Cuyjet, MD, MPH  
Vice Chair, Medicare Evidence Development & Coverage Advisory Committee  
Centers for Medicare and Medicaid Services  
7500 Security Boulevard  
Baltimore, MD 21244

**RE: Medicare Program; Meeting of the Medicare Evidence Development and Coverage Advisory Committee – August 22, 2018**

Dear Dr. Cuyjet,

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to provide comments to the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC or the Committee) in advance of the August 22<sup>nd</sup>, 2018 meeting on the state of evidence on Chimeric Antigen Receptor (CAR) T-cell therapies approved by the Food and Drug Administration (FDA) (MEDCAC meeting).

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics not only have improved health outcomes, but also have reduced healthcare expenditures due to fewer physician office visits, hospitalizations, and surgical interventions. BIO membership includes biologics and vaccine manufacturers and developers who have worked closely with stakeholders across the spectrum, including the public health and advocacy communities, to support policies that help ensure access to innovative and life-saving medicines and vaccines for all individuals.

CAR T therapies fall into a group of new, innovative treatments that represent a significant benefit and value for patient health outcomes and overall delivery of care – Transformative Therapies. These therapies generally address very serious diseases with high unmet medical need; can serve small patient populations, including rare and orphan diseases; and provide a substantial, durable health benefit. These therapies include cellular or gene therapies that are truly personalized medicines targeting treatment to specific patient populations or subsets of patient populations. CAR T therapies are a type of treatment in which a patient's own T-cells are reengineered to attack cancer cells through a highly-specific manufacturing process producing a single patient dose for a subset of incredibly vulnerable and sick cancer patients.

As new innovations in treatment are developed that have substantial benefit for patient health - providing new treatment options where previously there were no or limited options or serving patients with high unmet medical need – it is BIO's goal to ensure timely and appropriate patient access to these FDA-approved medicines, and development of coverage and reimbursement policies that facilitate such access and do not hinder patient/provider decision-making around the most appropriate and effective course of treatment for each patient's given condition.

We appreciate the Committee's focus on developing better understanding of patient experience and patient-reported outcomes (PROs) in cancer clinical studies and care during the course of the upcoming meeting. BIO strongly believes that patients must be involved in decision-making regarding their care, and that patients and patient advocacy organizations play a vital role throughout the drug development process, as they know what desired outcomes, risks, and other considerations are most appropriate for their diseases states/the disease states they serve. To this end, BIO has been supportive and engaged on FDA's activities related to patient-focused drug development (PFDD) to systematically obtain patient perspectives of specific diseases and their treatments. We believe that understanding patient perspectives is critical to the development of new and innovative treatment options for patients.

However, as it relates to the development of coverage policies, we have concerns where activities related to patient experience and outcomes may extend beyond the reach of CMS and into an area that is the jurisdiction of the FDA. As noted in our comments to the Centers for Medicare and Medicaid Services (CMS) on the proposed National Coverage Analysis (NCA) for CAR T,<sup>1</sup> we believe that FDA is the appropriate federal agency to consider any modifications for approval and subsequent coverage of therapies as it relates to patient outcomes – particularly for safety and efficacy. We reiterate to the Committee that FDA is the Agency that should be making such determinations around outcomes assessments, study design characteristics, study duration and use of PROs (validity, reliability, and generalizability).

Further, while we know MEDCAC does not make coverage determinations, BIO reiterates our continued caution to CMS against impeding patient access to therapy in opening up this NCA and as a result of the potential decisions made in a National Coverage Determination (NCD). We had cautioned the Agency against moving forward with such a coverage determination for this class of FDA-approved therapies. We believe an open stakeholder dialogue on PROs is an important and useful exercise across many therapy areas, but have concerns around this specific MEDCAC discussion and its potential to negatively impact patient access to newly approved CAR T therapies. We urge the Committee to move forward with caution and to allow the FDA to continue to collect inputs through its PFDD process on PROs, and to further assess outcomes for CAR Ts through the Risk Evaluation and Mitigation Strategies (REMS) that are in place for these products. Through REMS and long-term patient registry follow up, FDA can appropriately establish new requirements for therapies as ongoing post-marketing surveillance occurs.

BIO appreciates the opportunity to provide comments to MEDCAC in advance of the August 22nd meeting on PROs as they relate to CAR T therapies. We continue to believe that development of an NCD for CAR T therapies is inappropriate at this time, and note that potential discussions at the MEDCAC meeting could have significant impacts for patient access to these new therapies in Medicare. We look forward to continuing to work with the

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<sup>1</sup> See BIO Comments RE: National Coverage Analysis (NCA) Tracking Sheet for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancers (CAG-00451N). June 15, 2018.

Committee to ensure the most appropriate care and access to innovative treatment is delivered to cancer patients. Please do not hesitate to reach out with any questions at (202) 962-9200.

Sincerely,

/S/

Crystal Kuntz  
Vice President, Healthcare Policy and Research  
Biotechnology Innovation Organization

/S/

Mallory O'Connor  
Director, Healthcare Policy and Federal Programs  
Biotechnology Innovation Organization