AMERICAN SOCIETY of GENE & CELL THERAPY

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Dear Ms. Syrek Jensen, Dr. Szarama, and Dr. Paserchia:

The American Society of Gene & Cell Therapy (ASGCT) appreciates the opportunity to provide comments to the Centers for Medicare & Medicaid Services (CMS) regarding the Agency's national coverage analysis for chimeric antigen receptor T-cell (CAR T-cell) therapy. ASGCT is the premier membership organization consisting of scientists, physicians, and other professionals involved in gene and cell therapy. Optimal patient access to approved gene and cell therapies aligns strongly with the mission of ASGCT, which is to advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease.

CAR T-cell therapy is a genetically-modified cell therapy in which a gene is added to a patient's T-cells (a type of immune cell), enabling these cells to recognize and attack cancer cells when multiplied and infused back into the patient.[1] Two CAR T-cell therapies initially approved in 2017 are now providing effective and lifesaving treatment options for patients with certain types of acute lymphoblastic leukemia (ALL) and lymphoma that are resistant to other treatment or have had two or more relapses.[2,3]

ASGCT questions the need for a National Coverage Determination (NCD) at this juncture. Currently, CAR T-cell therapies approved by the Food & Drug Administration (FDA) are covered by CMS for medically accepted use consistent with their labeling[4,5] ASGCT is unaware of any instances in which CAR T-cell therapies for medically accepted use for FDA approved indications (on-label use) have been denied coverage. The Society applauds CMS for its efforts to make these lifesaving therapies available to patients and encourages the Agency to clarify to providers and, subsequently their patients, that on-label use of CAR T-cell therapy is currently covered. Moreover, ASGCT encourages the Agency to ensure

that the NCA process and any resulting determination has no limiting impact on coverage for CAR T-cell therapies for FDA approved indications now and in the future.

ASGCT is concerned that the establishment of an NCD for CAR T-cell therapy is premature and may ultimately limit patient access. As stated by UnitedHealthcare in its NCD request, ongoing clinical trials are likely to identify new patient populations that may benefit from CAR T-cell therapy. According to clinicaltrials.gov, there are nearly 100 active clinical trials involving CARs in the United States, many of which are targeting indications beyond ALL and non-Hodgkin lymphoma.[6] The Society is concerned that the NCA and a potential NCD may lack sufficient scope or flexibility to allow expeditious patient access to future products or new indications. ASGCT therefore encourages CMS to consider this rapid development in the field as new evidence emerges supporting CAR T-cell therapy for additional indications when determining whether the NCA and a potential NCD are necessary or prudent at time.

ASGCT and its members sincerely appreciate the opportunity to provide these comments to CMS and are thankful to the Agency for its efforts to ensure patient access to potentially lifesaving CAR T-cell therapies.

Sincerely,

Michele P. Calos, PhD

ASGCT President

References

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