March 11, 2021

Steven D. Pearson, MD
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

RE: Institute for Clinical and Economic Review — Multiple Myeloma Review Draft Evidence Report

Dear Dr. Pearson:

The undersigned organizations, who participate in a CAR T-cell therapy working group, appreciate the opportunity to respond to the draft evidence report on Anti B-Cell Maturation Antigen CAR T-cell and Antibody Drug Conjugate Therapy for Heavily Pre-Treated Relapsed and Refractory Multiple Myeloma.

CAR T-cell therapy (CAR T) is a transformative therapy that can substantially improve outcomes for patients with specific types of cancer.[1] While there are currently four approved CAR T therapies, there are over 630 active clinical trials, including those for Multiple Myeloma, two of which are being reviewed in this report.[2,3]

Both idecabtagene vicleucel and ciltacabtagene autoleucel have yet to be approved and studied in real world settings. We remain concerned that the clinical and financial data utilized are premature for the evaluation of CAR-T for Multiple Myeloma. The clinical benefits to patients receiving CAR T for Multiple Myeloma are still evolving, and new studies testing these treatments in earlier lines of care explore the possibility that they may be more effective.[4,5]

Below, we highlight several areas that we recommend ICER further consider.

CAR T Challenges & Patient Population

With the potential approval of CAR T for Multiple Myeloma approaching, there is significant excitement about the possibility to improve the lives of many patients impacted by the disease.

Multiple Myeloma patients eligible for CAR T are usually at the point where they have limited alternate treatment options and a very poor chance of survival, with data showing median overall survival without CAR T at 3.4 to 9.3 months.[6] CAR T for Multiple Myeloma have demonstrated an overall survival of over 19 months.[7] Research has also shown that the "cyclical nature" of Multiple Myeloma can result in higher levels of anxiety, depression and fatigue.[8] We have heard first-hand from patients about the value of hope, and that having another option can provide a mindset shift to those facing these circumstances.

Studies show that many Multiple Myeloma patients experience significant quality of life impacts, including physical symptoms of the disease and side effects of treatment.[9] The ongoing psychosocial impacts on patients, caregivers, and family members are also great.[10,11] Physical

ailments can include neurological damage such as peripheral neuropathy; pain management issues; kidney failure caused by Multiple Myeloma; and more, having a substantial impact on quality of life. Specifically, in a survey of approximately 200 multiple myeloma patients, 65% said that fatigue interferes with their daily life, 38% were at risk for clinically significant levels of anxiety, and 33% were at risk for clinically significant levels of depression.[12]

Health Disparities

Multiple Myeloma is twice as common in Black people.[13] ICER addresses concerns about health disparities in the draft evidence report. Specifically, ICER suggests that complex and higher-cost therapies have been underutilized by historically disadvantaged populations, suggesting that breakthrough treatments like CAR T may worsen health disparities.

We recognize the critical need to ensure that all therapies – including the most innovative – are available to all people living with multiple myeloma, particularly those from historically disadvantaged populations. We look forward to working with ICER and all relevant stakeholders to ensure equitable access.

Additional Patient Perspectives are Needed

We recognize and appreciate ICER's inclusion of patient and caregiver perspectives in the report. The significant physical, emotional, and financial burden on patients being treated for Multiple Myeloma should continue to be a focal point of these analyses.

ICER takes into account the impact that side effects have on patients, however it is critical that ICER understand the value of a "one and done" therapy. Numerous treatments and regular physician and hospital visits impose a financial burden on both patients and caregivers, including loss of work and/or societal contributions, in addition to direct costs of assuming the role of family caregiver.[14] These challenges can be significantly disruptive to the daily life of patients and caregivers.[15]

In conclusion, thank you for the opportunity to provide comments on this draft evidence report document. We believe that innovative treatments like CAR T represent hope for patients and caregivers. If you have any questions regarding our comments, please do not hesitate to reach out to our organizations.

Sincerely,

American Society for Gene and Cell Therapy

BMT InfoNet

Cancer Support Community

CLL Society

Myeloma Crowd

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