AMERICAN SOCIETY OF HEMATOLOGY
2021 L Street, NW, Suite 900, Washington, DC 20036 ph 202.776.0544 fax 202.776.0545 e-mail ASH@hematology.org

September 25, 2020

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Fred Upton
U.S. House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

Representatives DeGette and Upton,

I write on behalf of the American Society of Hematology (ASH) in reaction to the concept paper for 21st Century Cures 2.0 (Cures 2.0). Thank you for your efforts on this legislation. After reviewing the concept paper, the Society believes that many of these proposals have the potential to have a significant positive impact on the field of hematology – both in research and clinical practice.

ASH represents more than 18,000 clinicians and scientists worldwide who are committed to the study and treatment of blood and blood-related diseases. These disorders encompass malignant hematologic disorders such as leukemia, lymphoma, and multiple myeloma, as well as non-malignant conditions such as sickle cell anemia, thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. In addition, hematologists are pioneers in demonstrating the potential of treating various hematologic diseases and continue to be innovators in the field of stem cell biology, regenerative medicine, transfusion medicine, and gene therapy.

ASH respectively provides comments on the following areas:

1. Diversity in Clinical Trials
2. Increasing Use of Real-World Data/Evidence
3. Improve FDA-CMS Communication Regarding Transformative New Therapies
4. CMS Modernization

Diversity in Clinical Trials

ASH strongly supports including policies in the Cures 2.0 legislation that would promote diversity in clinical trials, including ensuring that Medicaid covers the routine care costs of clinical trial participation for enrollees with life-threatening conditions. ASH recommends that this not be limited to enrollees with life-threatening conditions but rather that this apply to all enrollees interested in participating in clinical trials under Medicaid. Unlike Medicare and private and commercial payers, Medicaid is not currently federally required to cover routine care costs associated with clinical trials, severely limiting who is able to participate and impeding efforts to ensure that trial participants reflect the real-world patient populations who will use a treatment. Additionally, the Society would like to highlight that insurance coverage beyond the clinical trial is equally as important. ASH encourages inclusion of policies that would require all states to consistently cover clinical trials for individuals on Medicaid, as well as ensure coverage following the trial.

Lack of awareness of and diversity of participants in clinical trials is a problem for many hematologic diseases, including multiple myeloma and sickle cell disease (SCD). The U.S.

Food and Drug Administration (FDA) hosted a workshop in February 2020 to examine under-representation of African Americans in multiple myeloma clinical trials. Incidence rates for multiple myeloma are higher in African Americans than whites, but there are lower rates of African American patient enrollment in pivotal and international trials. African Americans account for 20 percent of multiple myeloma patients, but clinical trials to date have not characterized either efficacy or safety in these patients.[1] Additionally, a recent clinical trial to compare bone marrow transplantation to standard of care in adolescents and young adults with severe SCD had trouble enrolling an appropriate number of patients because of Medicaid denials.

The Society also wants to make you aware of the ASH Research Collaborative's (ASH RC) Sickle Cell Disease Clinical Trials Network (SCD CTN). The ASH RC is a non-profit organization established by ASH in 2018 to improve the lives of people affected by blood diseases by fostering collaborative partnerships to accelerate progress in hematology. The SCD CTN aims to improve outcomes for individuals with SCD by expediting the development of treatments and facilitating innovation in clinical trial research. While there are only four FDA-approved therapies to treat SCD, there is a robust SCD therapy development pipeline; however, many challenges exist when it comes to conducting clinical trials for SCD, including a shortage of primary investigators, clinical trial sites, and enrolled participants. The SCD CTN is committed to forging new relationships with the SCD community to increase the understanding of clinical trials and trust in research; in fact, last year, the SCD CTN hosted a series of SCD community engagement workshops across the country to do just that. Insights from the workshops helped inform a SCD Community-Oriented Research Priority Report, which will establish a common understanding of goals for community-focused SCD research. Medicaid coverage of routine care costs of clinical trial participation would help advance research into this devastating disease.

Increasing Use of Real-World Data/Evidence

ASH supports the use of real-world evidence, and as such, supports the policies outlined in this section of the legislation. These proposals are complementary to the ASH RC Data Hub, which was created to facilitate the sharing of real-world data on benign and malignant hematologic conditions to support scientific inquiry and discovery. The Data Hub ingests a wide variety of information, including electronic health record (EHR) data, clinical and laboratory data, genomic or molecular correlates, patient-reported outcomes, and aggregated population data. Data is currently collected on individuals living with sickle cell disease, multiple myeloma, and those with a positive COVID-19 diagnosis and a hematologic condition or complication. The Data Hub is a reputable and dependable quality improvement resource that continues to evolve to meet the demands of the changing health care environment. Clinical registries, such as the Data Hub, support the FDA's ability to learn about safety signals in the post-market space while encouraging innovation by allowing data to be collected more efficiently than traditional clinical studies. As a major initiative within the ASH RC, the Data Hub aims to develop the largest shared information resource within the global hematology community. The ASH RC believes supporting registries is a cost-effective way to generate evidence to guide medical practice, expand indications, improve outcomes, and help drive innovation.

Improve FDA-CMS Communication Regarding Transformative New Therapies

ASH strongly supports establishment of an automatic communication requirement between the FDA and the Centers for Medicare and Medicaid Services (CMS) for products granted Breakthrough Therapy designations and believes that this sort of coordination could benefit access to treatments more broadly than just those with Breakthrough Therapy designations. In fact, in order to encourage innovation, the Society previously requested that the National Institutes of Health (NIH), the FDA, and CMS work together to ensure that research, approval, and reimbursement policies respectively support appropriate patient access. ASH anticipates that the gene and cellular therapies currently in development have the potential to significantly improve outcomes or potentially cure individuals with hematologic diseases or disorders, but the experience with chimeric antigen receptor T-cell (CAR-T) therapy raises the concern that innovative therapies currently in development will not be available to patients because the Medicare and Medicaid reimbursement systems are not equipped to equitably reimburse for these therapies, as explained in more detail below.

---

[1] U.S. Food and Drug Administration and American Association for Cancer Research. FDA-AACR Workshop to Examine Under-representation of African Americans in Multiple Myeloma Clinical Trials. https://www.aacr.org/wp-content/uploads/2020/03/FDA-AACR-MMCT-Slide-Deck.pdf

CMS Modernization

General Coverage Modernization of Cell and Gene Therapies

Current coverage and reimbursement rules for new medical products under federally-financed health programs are not equipped to adequately cover and pay for already approved and soon-to-be-approved gene and cell therapies – the majority of which will be potentially curative, one-time treatments but are also likely to be very high cost. ASH appreciates that the Cures 2.0 concept paper aims to address this issue. The drug pipeline currently has numerous gene and cell therapies in development, including many for hematologic diseases and disorders, such as SCD, hemophilia, as well as the blood cancers, lymphoma, myeloma, and leukemia. CAR-T therapy, currently approved for certain indications of leukemia and lymphoma, is an example of how current coverage and reimbursement rules were not ready for a high-cost, potentially curative new therapy. When the first two CAR-T products were approved in 2017, both public and private insurers had to scramble to determine coverage and payment for these products. Much of it was determined on a case-by-case basis, putting both patients and providers in a difficult position and likely delaying access to what may have been a last option for many patients.

CMS has recently taken significant steps to address some of the pitfalls in coverage and reimbursement and ASH believes that some of these policies should be considered for future high-cost therapies. ASH was pleased with the steps CMS took in the fiscal year (FY) 2021 Inpatient Prospective Payment System (IPPS) final rule to address reimbursement for CAR-T therapy – specifically, that CMS will exclude clinical trial claims and claims showing a pharmacy charge of less than $373,000 (the list price for CAR-T therapy) when calculating the weight/payment of the new MS-DRG for CAR-T. Knowing that forthcoming cell and gene therapies will also likely be high cost, ASH urged CMS to consider continuing to exclude clinical trial claims when calculating the weight of other new MS-DRGs. Furthermore, while CAR-T therapies are expensive, with a list price of $373,000 and $475,000, some future cell and gene therapies are estimated to cost $1million or more. It is imperative for CMS to further its work on how these therapies will be covered and reimbursed so that institutions are able to provide patient access to them. The Cures 2.0 legislation should include guidance on this.

Additionally, ASH was supportive of the proposed rule on Medicaid and value-based payment (VBP) arrangements released in June of this year. ASH encourages the development of VBP arrangements, especially for innovative therapies, as long as they are evidence-based and clinically appropriate. These arrangements must recognize the physician services associated with the provision of the treatment, including the cost of obtaining, storing, and direct administration of these therapies. Additionally, the Society only supports VBP arrangements that are patient centric – the only goal cannot be to reduce costs, it must include striving for and achieving superior patient outcomes. While the proposed rule addressed some barriers to implementing VBP arrangements, such as best price, others exist, including policies around average manufacturer price (AMP). The Cures 2.0 legislation could focus on removing those remaining barriers.

Thank you for your work on these important issues. We appreciate your review of these comments and we welcome the opportunity to discuss them with you and your staff. Please reach out if ASH can ever serve as a resource on the topics outlined above or on any matter related to hematology. For questions or to schedule a meeting, please reach out to Leslie Brady, ASH's Policy and Practice Manager, at lbrady@hematology.org.

Sincerely,

Stephanie J. Lee, MD, MPH
President