AMERICAN SOCIETY OF HEMATOLOGY
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July 16, 2021

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

Dear Representatives DeGette and Upton:

I write on behalf of the American Society of Hematology (ASH) in response to the Cures 2.0 discussion draft that you released on June 22, 2021. Thank you for your efforts and for the opportunity to submit feedback. After reviewing the draft legislation, the Society believes that many of these proposals have the potential to positively impact the field of hematology and the patients we care for – 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 disease, 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 of the discussion draft:

1. Diversity in Clinical Trials
2. Increasing Use of Real-World Data/Evidence
3. Improving FDA-CMS Communication Regarding Transformative New Therapies
4. CMS Modernization
5. Extending Medicare Telehealth Flexibilities
6. Advanced Research Projects Agency for Health (ARPA-H)
7. Research Investment to Spark the Economy

Diversity in Clinical Trials

Lack of awareness 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 and mortality 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]

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, in 2019, 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, as is now required, will also undoubtedly help advance research into this devastating disease. However, ASH believes it is also critically important to ensure that patients continue to have health coverage after completion of a clinical trial. A patient covered by Medicaid who participates in a clinical trial and then moves to a different state could find themselves without medical coverage and financially unable to secure the care they need. ASH encourages you to include language requiring Medicaid to provide necessary care for clinical trial participants following completion of the trial.

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 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. We applaud the

[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

inclusion in the discussion draft of language seeking to increase the use of real-world evidence and supporting the use of patient registries to fulfill post-approval study requirements for products regulated by the Food and Drug Administration. However, additional, more specific reforms are needed to ensure that clinician-led clinical data registries have meaningful access to Medicare, Medicaid, and State Children's Health Insurance Program claims data to better track patient outcomes over time, expand their ability to assess the safety and effectiveness of medical treatments, and provide them with the information necessary to assess the cost-effectiveness of therapies. Gaining meaningful access to claims data would enable clinician-led clinical data registries to provide greater insight into the value of emerging therapies, particularly in underrepresented and underserved patient populations. We urge you to include language in Cures 2.0 to ensure that clinician-led clinical data registries are afforded meaningful access to claims data.

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, such as gene therapy for sickle cell disease, 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.

CMS Modernization

General Coverage Modernization of Cell and Gene Therapies

Current coverage and reimbursement rules for new medical products under federally financed health programs do not have a well-defined strategy on how already approved and soon-to-be-approved gene and cell therapies will be covered and paid. Many of these products are 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 sickle cell disease, hemophilia, as well as the blood cancers, lymphoma, myeloma, and leukemia. 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 what may have been a last option for many patients.

CMS has recently taken significant steps to address some of the pitfalls in reimbursement for CAR-T provided to hospital inpatients. ASH was pleased with the steps CMS took in the fiscal year (FY) 2021 Inpatient Prospective Payment System (IPPS) proposed rule to address reimbursement for CAR-T therapy – specifically, that CMS proposed to exclude clinical trial claims (when drugs are provided at no cost to patients) 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 proposed MS-DRG for CAR-T. Knowing that forthcoming cell and gene therapies will also likely be high cost, ASH urges 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, some future cell and gene therapies are estimated to cost $1million or more. The Cures 2.0 legislation should include guidance more generally on how CMS should reimburse for high-cost drugs provided on an outpatient basis.

Additionally, ASH was supportive of the proposed rule on Medicaid and value-based payment (VBP) arrangements released in June of last 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. We recommend that the Cures 2.0 legislation focus on ensuring any future VBP arrangements meet the criteria outlined above.

Extending Medicare Telehealth Flexibilities
The COVID-19 public health emergency (PHE) has highlighted the need for and importance of telehealth in enabling physicians to care for their patients. Making permanent the changes to Medicare and Medicaid telehealth services implemented during the PHE will improve and expand patient access to care.

Many hematologic diseases are rare and complex to manage. Patients may have to travel great distances to see specialists, including hematologists, particularly for follow-up visits and oral chemotherapies. Telehealth services increase access to care and reduce the use of resources for travel to receive specialty care. Permanently expanding telehealth services would ease the burden on patients, allow them to continue care management remotely, and help them receive appropriate care regardless of where they live.

To allow patients and physicians to continue to benefit from telehealth services, ASH supports permanently eliminating originating site requirements. Prior to the PHE, Medicare beneficiaries had to travel to designated locations in order to utilize telehealth services and only Medicare beneficiaries living in rural areas or with specific conditions were allowed to utilize telehealth services from their home. These requirements were waived during the PHE, allowing beneficiaries, regardless of geographic location or condition, to utilize telehealth from the safety and comfort of their own home.

ASH also supports maintaining payment parity between telehealth and in-person visits. While there will always be situations that require a patient to be seen in-person by a physician, when care is provided via telehealth, physicians provide the same level of service as they would for an in-person visit. Physicians should receive the same level of reimbursement for telehealth services as they do for the comparable level of an in-person office or outpatient visit.

Payment for audio-only telehealth services must also be made permanent. Although codes existed for audio-only telehealth services prior to the PHE, they were not covered or reimbursed under Medicare. During the PHE, not only have those services been covered, but the reimbursement was also increased to align with the comparable in-person office/outpatient evaluation and management codes. Video is not always an option for many patients – technology fails, bandwidth is not strong enough, elderly patients do not know how to access/utilize it. Additionally, many times, the oral conversation between a physician and a patient is the key component (rather than visually seeing the patient), especially for patients with blood diseases.

Advanced Research Projects Agency for Health (ARPA-H)
ASH applauds President Biden's and Congress' commitment to help drive transformational innovation in health research and speed application and implementation of health breakthroughs. However, in order to be able to fully realize this goal, the Society believes that ARPA-H will need to be truly unique and different from the status quo and must not duplicate efforts already underway at NIH or any of the other federal research agencies. Large-scale, innovative research focusing on areas of need, including research to reduce health disparities and efforts to make existing therapies more affordable and more widely available, as well as projects not able to be accomplished within existing infrastructure or processes, should form the basis of the agency's research portfolio.

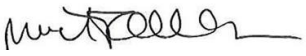
Additionally, the Society joins with others in the research community in stressing that robust growth in the foundational research that NIH supports will be key to this vision, and we strongly believe that any additional funds for ARPA-H or other targeted initiatives must supplement, rather than supplant, this core investment.

Research Investment to Spark the Economy
ASH is extremely grateful for Congress's ongoing commitment to NIH as a top national priority through the regular appropriations process. However, the Society also believes there is a need for additional emergency supplemental investments for to enable NIH to mitigate COVID-19 pandemic-related disruptions without foregoing promising new science. ASH strongly supports emergency funding for federal research agencies – including $10 billion for NIH—as outlined in the bipartisan Research Investment to Spark the Economy (RISE) Act that you first introduced last year, reintroduced in the 117th Congress, and have included in the Cures 2.0 discussion draft.

The pandemic's impact on biomedical research has been serious and far-reaching. Researchers in every state were forced to suspend many laboratory activities for their own personal safety and to comply with physical distancing guidelines. The closure of many research facilities impacted trainees, technicians, early-stage investigators, and established investigators alike, preventing the research workforce from maintaining momentum toward better prevention, treatments, diagnostics, and cures for diseases such as blood cancers, sickle cell disease, and other hematologic diseases and conditions. While many institutions have been implementing plans to ramp this work back up again as safely as possible, challenges associated with the disruptions continue to linger. For example, certain types of research – such as clinical trials and other research projects with human participants – have been slower to recover. Additionally, as a result of the lags, we risk undoing progress we have made in recent years in strengthening the research workforce, including among women, underrepresented minorities, and early-career investigators and others at a pivotal point in their career trajectories.

Thank you, again, for your work on these important issues. We appreciate the opportunity to submit 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 contact Tracy Roades, ASH Senior Manager, Legislative Advocacy at *troades@hematology.org*, 202-292-0256, or, Suzanne Leous, MPA, ASH Chief Policy Officer at *sleous@hematology.org*, 202-412-7531.

Sincerely,



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