



Advancing Transfusion and
Cellular Therapies Worldwide

July 16, 2021

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, D.C. 20515-4329

The Honorable Fred Upton
U.S. House of Representatives
2183 Rayburn House Office Building
Washington, D.C. 20515-4329

Submitted Electronically Via: cures2@mail.house.gov

RE: 21st Century Cures 2.0 Discussion Draft and Advanced Research Projects Agency for Health (ARPA-H) Request for Information (RFI)

Dear Ms. DeGette and Mr. Upton:

AABB appreciates the opportunity to provide feedback on the Cures 2.0 discussion draft and Advanced Research Projects Agency for Health (ARPA-H) request for information (RFI). AABB is an international, not-for-profit association representing institutions and individuals involved in transfusion medicine and biotherapies. The association is committed to “improving lives by making transfusion medicine and biotherapies safe, available and effective worldwide.” AABB works toward this vision by developing and delivering standards, accreditation, and educational programs that focus on optimizing patient and donor care and safety. AABB’s institutional members include blood collection establishments, transfusion medicine services, cellular therapy laboratories and testing facilities. The Association’s individual membership includes physicians, nurses, scientists, researchers, administrators, medical technologists, and other health care providers.

AABB has an established history of ensuring patients’ access to safe, effective blood and biotherapies through standards and accreditation. AABB’s Accreditation Program started in 1958 and has been a key driver in the adoption of quality management systems by clinical laboratories to improve patient care. The AABB Standards and Accreditation Program for Cellular Therapy covers all elements of collecting, manufacturing, and administering cells, including the collection, storage, transport, testing, processing, administration and patient outcomes for these products. The Standards contain comprehensive requirements that focus on the clinical, technical, and regulatory processes necessary to safely deliver blood, cellular therapies and biotherapies to patients and include controls for the facilities administering the cells.

AABB commends you for your bipartisan leadership that resulted in the enactment of the 21st Century Cures Act (Cures Act, P.L. 114-255). This law assisted in accelerating medical product development, research, and the delivery of innovative biotherapies to patients who need them faster and more efficiently. AABB is proud that its members - including blood collection establishments, transfusion medicine services and cellular therapy laboratories - are involved in the full spectrum of biotherapies including the collection of cellular starting materials, research, and providing patient care. A variety of biotherapies are used to treat a broad range of diseases and chronic health conditions. For instance, hematopoietic stem cells are used to treat blood cancers, such as leukemia and lymphoma, and sickle cell disease and chimeric antigen receptor (CAR) T-cell therapies are used to treat certain types of lymphomas and leukemias as well as multiple myeloma, a cancer of the bone marrow.

In addition to being integral to manufacturing and making available novel biotherapies, AABB’s community includes the full spectrum of the U.S. blood system – from donor to patient or from vein to vein.

Blood collection establishments collect, test, process and distribute blood components to hospitals and other settings of care where blood is transfused to patients. Blood transfusions are medically necessary because there is no alternative treatment option. They are routine treatments for patients with chronic health conditions, life-saving therapies for patients who experience blood loss from trauma or surgery and must be available in emergencies. Thus, continued investments in innovation related to blood and biotherapies will benefit a wide variety of patients, and can improve access to these important treatment options.

AABB believes that Cures 2.0 has the potential to drive transformational changes that can improve the safety and availability of blood and biotherapies through policies such as:

- Supporting a harmonized approach to cellular starting materials that are used to manufacture novel biotherapies;
- Integrating the blood and biotherapies community into policies that advance a national testing and response strategy for future pandemics and public health responses;
- Encouraging improved communication and coordination between the Food and Drug Administration (FDA) and the Centers for Medicare and Medicaid Services (CMS); and
- Ensuring that the Advanced Research Projects Agency for Health (ARPA-H) can support research and development as well as projects that focus on the implementation of innovative technologies, processes and services related to blood, transfusion medicine and biotherapies.

I. Cellular Starting Materials Used to Manufacture Novel Biotherapies

AABB encourages Congress to ensure that patients have access to safe and effective biotherapies by (1) supporting a harmonized approach to the collection of cellular starting materials that are used to manufacture biotherapies and (2) strengthening the laboratory workforce.

Unlike most other life-sustaining medications, blood and biotherapies originate from donors. Therefore, it is important that collectors of cellular starting materials have quality management systems in place to ensure patient and donor care and safety, process controls, and continuous improvement. The absence of a consistent approach to the collection of cellular starting materials results in inefficiencies that slow advances in biotherapies, and have the potential to compromise intended patient outcomes, degrade effectiveness, and drive-up costs. We encourage Congress to advance a harmonized approach to the collection, characterization, and safe handling of cellular starting materials by:

- Recognizing related standard-setting and accreditation organizations, such as AABB;
- Providing support for related education and technical assistance for academic and research institutions, industry innovators, and practitioners developing novel therapies;
- Leveraging ARPA-H to explore a platform for data, protocols, and reference materials; and
- Facilitating multiple organizations working together to develop, produce and deliver promising biotherapies.

Additionally, Congress can support patients' access to safe biotherapies by strengthening the blood banking and transfusion medicine workforce. Unfortunately, a variety of blood banking and transfusion medicine positions are impacted by laboratory workforce shortages, including phlebotomists, medical laboratory technologists (also referred to as medical laboratory scientists), medical laboratory technicians, and supervisory staff roles in blood banking. The workforce shortages of qualified personnel for blood banking and

transfusion medicine limit the capacity of these facilities, presents risks to patient safety and blood availability, and reduces the nation's preparedness and response capabilities.^{1,2}

II. Discussion Draft Sec. 102. National Strategy to Prevent and Respond to Pandemics

AABB supports Congress' proposal to establish a strategy to address future pandemics, which includes testing strategies as well as a data sharing infrastructure to inform surveillance, monitoring, and response efforts. As part of this approach, we encourage Congress to:

- **Implement the [September 2020 recommendations](#) of the Department of Health and Human Services' (HHS) Advisory Committee on Blood and Tissue Safety and Availability (ACBTSA)³ and the [recommendations](#) in the HHS Report to Congress on the adequacy of the national blood supply,⁴ which include a recommendation to establish, implement and fund a comprehensive, sustainable, minimally burdensome infrastructure that monitors and makes available real-time data on blood availability and utilization.**

The absence of data has been a significant challenge throughout the pandemic due to significant fluctuations in blood availability and utilization. For example, at the beginning of the pandemic, blood donation centers experienced a sharp decline in blood donation due to travel restrictions and social distancing efforts. As the pandemic progressed, hospitals stopped performing non-emergent procedures, which resulted in a steep reduction of blood utilization. As hospitals resumed non-emergent and elective services, utilization increased and the blood supply was again strained. A data infrastructure can provide visibility into the status of the blood supply, which can help predict demand, understand availability, and ensure that patients have access to this life-saving therapy.

- **Fund and enhance the national surveillance infrastructure so that it tracks vector and human activities, including the extent and activities of the agents, their vectors, and human infections throughout the United States, in near real-time.**

Such surveillance should also include programs to monitor non-vector borne transmissions including via blood transfusion and therapies involving human cells, tissues, and cellular and tissue-based products (HCT/Ps). Comprehensive, timely surveillance is key to developing and adopting evidence-based policies and procedures that are proportional to documented risk, mitigating the risks of vector-borne diseases, and ensuring the availability of safe blood and HCT/Ps.

- **Consider policies and dedicate resources that support the screening, testing and surveillance activities conducted by the blood community, which possess unique, well-established expertise that is critical to the nation's public health infrastructure. These efforts can benefit donors, patients, local communities, and the nation, and often support other sectors.**

¹ The Clinical Laboratory Workforce: Understanding the Challenges to Meeting Current and Future Needs, April 2021, available at

https://ascpcdn.s3.amazonaws.com/static/ISTP/Siemens_Clinical+Laboratory+Workforce_Brochure_042721.pdf.

² Edna Garcia, MPH, Iman Kundu, MPH, Melissa Kelly, PhD, Ryan Soles, MS, The American Society for Clinical Pathology's 2018 Vacancy Survey of Medical Laboratories in the United States, American Journal of Clinical Pathology, Volume 152, Issue 2, August 2019, Pages 155–168, <https://doi.org/10.1093/ajcp/aqz046>.

³ Recommendations of the 52nd ACBTSA Meeting, September 25, 2020, available at <https://www.hhs.gov/oidp/advisory-committee/blood-tissue-safety-availability/recommendations/2020-09-25/index.html> (last visited July 16, 2021).

⁴ U.S. Department of Health and Human Services, Adequacy of the National Blood Supply: Report to Congress, 2020, available at <https://www.hhs.gov/sites/default/files/hhs-adequacy-national-blood-supply-report-congress-2020.pdf> (last visited July 16, 2021).

The lack of reliable, community-based national surveillance data is historically quite problematic. Despite the current lack of funding, the blood community has been instrumental in early detection through surveillance, screening, and testing activities related to multiple infectious disease agents, such as Zika virus (ZIKV).

For example, in 2016, FDA classified ZIKV as a relevant transfusion-transmitted infection and issued guidance that required blood collection establishments to test blood donations for the ZIKV. Blood collectors immediately implemented the new testing requirements. Additionally, in the absence of a national surveillance infrastructure, AABB created a Zika Virus Biovigilance Network that enabled blood collection establishments to voluntarily report data on the number of blood donations in the United States from donors with suspected and confirmed ZIKV infection, and then mapped the information to geographic locations. While AABB established this surveillance tool to support the blood community, many state and local public health departments contacted AABB and used the information in the Zika Virus Biovigilance Network as a proxy for determining the incidence and prevalence of ZIKV in their geographic regions.⁵

In May 2021, FDA eliminated the requirement that blood establishments test blood donations for the ZIKV. FDA relied on data from CDC as well as the AABB Zika Biovigilance Network to confirm the absence of ZIKV infection in the donor population. We strongly support this decision and FDA's evidence-based approach to updating the Zika policy for blood establishments. However, both the requirements that blood collectors test blood for vector-borne agents and the establishment of associated Biovigilance Networks are unfunded initiatives that cannot be sustained.

III. Discussion Draft Sec. 305. Improving FDA-CMS Communication Regarding Transformative New Therapies

AABB is supportive of inter-agency communication and its potential to improve patients' access to novel biotherapies. However, we believe that improved communication must be coupled with greater coordination between the two agencies so that CMS' coverage and reimbursement decisions occur in parallel to FDA's approval. Additionally, we encourage Congress to ensure that FDA and CMS consider all items and services required throughout a novel biotherapies treatment protocol, starting with the collection of the cellular starting material. Each step is resource intensive and can take place at different institutions. For example, cellular starting material can be harvested at blood collection establishments, manipulated in a different laboratory, and then administered to a hospital inpatient. Similarly, we urge Congress to ensure that FDA and CMS engage in early dialogue and coordination to ensure that reimbursement policies are timely and include costs associated with innovation, such as implementing new regulatory blood safety requirements, conducting new screening tests, developing improved blood components, and utilizing new blood therapies.

IV. Discussion Draft Sec. 501. Advanced Research Projects Agency for Health

AABB strongly supports Congress' proposal to expedite transformational research and implementation in healthcare through the creation of the Advanced Research Projects Agency for Health (ARPA-H). We encourage Congress to ensure that ARPA-H invests in efforts intended to advance research, development and

⁵ AABB modeled the ZIKV Biovigilance Network after that used for West Nile Virus (WNV) (established in 2003) and a similar Biovigilance Network that was used for Chagas (established in 2007). The AABB Biovigilance Networks for infectious agents transmitted by blood and HCT/Ps have been used as public health tools in the absence of other real-time networks for reporting.

the implementation of innovative technologies, processes and services related to blood, transfusion medicine and biotherapies.

For instance, we urge Congress to structure ARPA-H in a manner that enables the agency to invest in a public-private partnership that can strengthen the blood system through efforts such as: (1) identifying barriers that limit innovation or interfere with patient care and exploring alternative policies that support novel blood products, technologies, processes and procedures; (2) exploring policy solutions that support better care for individuals, better health for populations and lower costs; (3) proactively identifying efficiencies and opportunities to improve clinical trials conducted during public health emergencies; and (4) using information from the COVID-19 response to update preparedness plans for a future pandemic involving an emerging transfusion-transmissible virus.

As another example, we encourage Congress to ensure that ARPA-H can drive progress related to biotherapies by investing in an innovative infrastructure that captures data, protocols, and information on reference materials for the purpose of facilitating collaboration and harmonization. Investments in research, development, and implementation of innovative manufacturing processes, beginning with the collection of cellular starting materials, has the potential to expedite the development of novel biotherapies and ultimately contribute to expedited reviews of new treatment options. ARPA-H can also be used to support research, development, and implementation of novel biotherapy treatments, such as immunotherapies and regenerative medicine.

Finally, we recommend that Congress ensure that ARPA-H is nimble and able to address a wide variety of challenges that limit research, development, and implementation of innovation in healthcare. For example, continued growth and advances in biotherapies relies on partnerships between academic medical centers and biopharmaceutical companies. While these critical relationships facilitate the development and clinical application of novel immune effector cell therapies and other biotherapies, they can be strained by the rapidly increasing number of research studies as well as the diverse requirements of each study. ARPA-H should be structured in a manner that supports investments in efforts intended to address different challenges that can thwart innovation.

V. Conclusion

AABB commends both your previous and current efforts in increasing access to innovative biotherapies to improve patient care and outcomes. We appreciate the opportunity to provide comments in response to the discussion draft and would welcome the opportunity to further support your work on this effort. If you or your staff have any questions, please contact Leah Mendelsohn Stone, vice-president of public policy & advocacy at 301-215-6554 or lmstone@aabb.org.

Sincerely,

Debra BenAvram
Chief Executive Officer
AABB