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ABC NEWSLETTER

CURRENT EVENTS AND TRENDS IN BLOOD SERVICES

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2021 #23 **June 25, 2021**

INSIDE:

Please Note: The *ABC Newsletter* will not be published on July 2nd. We will resume regular publication on July 9th. Thank you for your continued interest.

Data from the 2019 NBCUS Published

Findings from the 2019 National Blood Collection and Utilization Survey (NBCUS) have been published in *Transfusion*. The 2019 survey report includes "estimates" regarding the total number of blood components collected, distributed, and transfused in the U.S. The reported data is based on the answers provided by blood collection facilities (18 questions) and transfusing hospitals (26 questions). According to the authors, close to 95 percent of community-based blood collection facilities participated in the survey (50/53) with 84 percent of hospital-based blood collection facilities and 76 percent of transfusing hospitals.

Data from the report showed that, "[b]etween 2017 and 2019, the number of whole blood units collected decreased by 6.1 percent, while the number of apheresis units increased by 0.7 percent. Among all whole blood units collected during 2019, 99.9 percent were collected for allogenic, nondirected transfusions. During 2019, 10,852,000 units of whole blood-derived and apheresis red blood cell (RBC) units were transfused in the United States (95 percent CI, 10,444,000–11,259,000 units), a 2.5 percent increase compared with 2017, when 10,654,000 units were transfused. Between 2017 and 2019, the decline in the number of RBC units distributed from blood centers to hospitals continued. However, although the number of RBC units transfused had decreased during 2008–2017, no significant decrease occurred between 2017 and 2019. Similarly, the number of whole blood and RBC units collected per 1000 population has decreased from a maximum of 86.4 in 2008 to 55.3 in 2019. However, RBC units transfused per 1000 population were highest in 2008 (49.4), decreased until 2017 (32.8), and increased in 2019 (33.1)." The authors also explained that "[a]mong all whole blood and RBC units rejected after collection, the proportion of units rejected because of abnormal disease markers has continued to increase from 9.4 percent in 2015 to 11.7 percent in 2017 and 14.5 percent in 2019. After accounting for rejections, the total available supply of whole blood and RBC units in 2019 was 10,879,000 (95 percent CI, 10,484,000–11,274,000) units, a 5.8 percent decrease from 2017. During 2019, 349,000 whole blood and RBC units were outdated (95 percent CI, 321,000–377,000), a 21.7 percent decrease compared with 2017."

For hospitals that participated in both the 2017 and 2019 NBCUS, the authors discovered that, "the median percent difference in the number of RBC units transfused between 2017 and 2019 was 2.2 percent. The median percent difference differed

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2019 NBCUS Data Published (continued from page 1)

by surgical volume category. Hospitals with lower surgical volumes were associated with a larger decrease in RBC transfusion volume between 2017 and 2019. The median percent difference of RBC transfusions between 2017 and 2019 among hospitals with 100–999 surgical operations per year was 7.9 percent, while transfusions increased by 1.9 percent among hospitals performing ≥8000 surgical operations per year. In 2019, among locations within a healthcare facility, the highest volume of RBC units were transfused in inpatient medicine settings (3,909,000 units; 95 percent CI, 3,593,000–4,226,000 units), followed by critical care (1,810,000 units; 95 percent CI, 1,645,000–1,975,000 units), outpatient and nonacute inpatient settings (1,512,000 units; 95 percent CI, 1,335,000–1,689,000 units), surgery (1,380,000 units; 95 percent CI 1,236,000–1,525,000 units), and emergency departments (1,277,000 units; 1,163,000–1,391,000 units)."

The reported data "[i]n 2019 [showed that] 2,359,000 units (95 percent CI, 2,240,000–2,477,000 units) of apheresis PLT units were distributed, a 0.9 percent increase compared with the 2,338,000 units distributed in 2017. Approximately 149,000 units (in apheresis unit equivalents; 95 percent CI, 105,000–193,000 units) of whole blood derived platelets (PLTs) were distributed in 2019, a 33.1 percent decrease compared with the 223,000 units distributed in 2017. Among all PLT units distributed, the proportion that were whole blood-derived decreased from 8.7 percent in 2017 to 5.9 percent in 2019… 2,243,000 apheresis and whole blood-derived PLT units (95 percent CI, 1,930,000–2,555,000 units) were transfused, a 15.8 percent increase from the 1,937,000 units transfused in 2017. Approximately 1,996,000 apheresis PLT units (95 percent CI, 1,846,000–2,147,000 units) were transfused in 2019, an 8.0 percent increase from the 1,848,000 units transfused in 2017 and a 10.5 percent increase from the 1,807,000 units transfused in 2015. Approximately 243,000 whole blood-derived platelet units (in apheresis equivalents; 95 percent CI, 0–503,000 units) were transfused in 2019, a 197 percent increase from 2017 and 42.1 percent increase from 2015."

Plasma distributions decreased by 16.5 percent in 2019 to 2,679,000 units down from 3,209,000 in 2017. "This value includes all types of plasma, including fresh-frozen plasma, plasma frozen within 24 h of collection, cryoprecipitate-reduced plasma, and liquid plasma. A decrease was also seen in the number of plasma units transfused. Approximately 2,185,000 plasma units (95 percent CI, 2,068,000– 2,301,000 units) were transfused in 2019, an 8.0 percent decrease compared to the 2,374,000 plasma units transfused in 2017."

The authors conclude by explaining that "the decline in utilization rates of RBC in the U.S. might have ended. Between 2017 and 2019, RBC collections declined while RBC transfusions did not significantly change, suggesting a narrowing between blood supply and demand. However, apheresis PLT collection and utilization have increased since 2015, and additional strategies may be needed to ensure a sufficient PLT supply. CDC and OASH will continue to monitor national blood collection and utilization data to support efforts to ensure the safety and availability of the blood supply." They also state that "hospitals with lower

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ABC advocates for and advances policies that promote the role of independent blood centers in providing life-saving blood products and recognize the continuous need for a safe and robust blood supply. ABC exists to advocate for laws and regulations recognizing the essential role that independent blood centers play in the healthcare system; promote partnerships, policies and programs that increase awareness about the need for blood donation; and serve as a thought-leader in the advancement of evidence-based medical and scientific solutions related to health and safety.

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surgical volumes reported greater decreases in red blood cell transfusion than hospitals with larger surgical volumes. However, a greater median percent difference between 2017 and 2019 compared to [the] difference between 2015 and 2017 was noted across all surgical volume categories. This suggests that larger hospitals are, on average, no longer experiencing a decrease in RBC utilization, and the decline in utilization among smaller hospitals continues to slow…Between 2017 and 2019, RBC collections continued to decrease while transfusions increased slightly, indicating a narrowing between blood supply and demand. This suggests the potential for reduced elasticity in blood supply, which may impact blood sufficiency if blood demand increases or blood supply decreases during public health emergencies The potential for reduced elasticity has resulted in both governmental and non-governmental stakeholders to express concerns about the adequacy of the blood supply…The increasing demand for PLTs may pose future challenges to maintaining an adequate PLT supply. The average age of apheresis PLT donors is increasing, with blood centers reporting difficulties with recruiting and retaining younger apheresis PLT donors." Several limitations regarding the reported data are enumerated including "potential for novel pandemic-related data quality issues that cannot be easily quantified and would not be encapsulated by the confidence intervals presented."

Citation: Jones, J., Sapiano, M., Mowla, S., Bota, D., Berger, J, Basavaraju, S. "Has the trend of declining blood transfusions in the United States ended? Findings of the 2019 National Blood Collection and Utilization Survey." *Transfusion*. 2021.

BRIEFLY NOTED

The Pediatric Acute Lung Injury and Sepsis Investigators (PALISI), BloodNET (The Pediatric Critical Care Blood Research Network), and Trauma Hemostasis and Oxygenation Research (THOR) are hosting a free webinar on July 21st from 3-5 p.m. EDT titled "Why do you split blood apart and put it all back together again? – Current data and experience with whole blood for severe bleeding in children." Registration is open. The webinar aims to:

- [d]iscuss the pathophysiology of life-threatening bleeding in children and the biologic rationale for low titer type O whole blood (LTOWB);
- [d]escribe current data on the use and outcomes of LTOWB in children with traumatic injury and undergoing cardiac surgery;
- [d]iscuss the relative risks of LTOWB administration in children; and
- [l]earn how LTOWB is supplied with experiences from the American Red Cross and the South Texas Blood & Tissue Center.

Speakers include:

- Phil Spinella, MD;
- Christine Leeper, MD;
- Marie Steiner, MD, MS;
- Mark Yazer, MD;
- Pampee Young, MD, PhD;
- Elizabeth Waltman; and
- Jennifer Muszynski, MD.

(Source: PALISI, BloodNET, and THOR Announcement, 6/21/21)

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INSIDE ABC

The programs and services described in the Inside ABC section are available to ABC member blood centers and their staff only, unless otherwise specified.

Register Today for 2021 ABC Medical Directors Workshop & Summer Summit

Register today for America's Blood Centers (ABC) 2021 Medical Directors Workshop and Summer Summit. These events will take place in-person in Cleveland, Ohio August 4th-6th. Virtual registration options also exist for those unable to attend including group discounts for virtual registration to allow as many of blood center staff members to participate as possible. A preliminary program is available. ABC is working with its event location partner to ensure the safety and well-being of all attendees is prioritized in accordance with local, state, and national guidelines. Secure hotel reservations now before the July 12th deadline. More information and updates will be provided as they become available.

June ABC *Blood Bulletin* Now Available

ABC's Scientific, Medical, and Technical (SMT) Publications Committee has published the June 2021 Issue of the *Blood Bulletin*, titled "Transfusing O-Negative Blood: Good Stewardship of a Precious Resource." The article was written by Chris Gresens, MD, Senior Chief Medical Officer, Mountain & West Divisions at Vitalant, Nanci Fredrich, RN, BSN, MM, Transfusion Safety & Blood Management Officer for Versiti, Richard Gammon, MD, Medical Director at OneBlood, and Nancy Van Buren, MD, Medical Director at Innovative Blood Resources, a division of New York Blood Center. The *Blood Bulletin* is reviewed and edited by ABC's SMT Publications Committee. ABC publishes the *Blood Bulletin* for use by member blood centers in their educational programs as a value-added service for hospital customers. Current and previous issues can be accessed at any time on the ABC member portal. Please contact Member Services for trouble accessing the publication.

Please note: The MS Word version may not display properly for users with older versions of MS Word. For those individuals, we recommend viewing and using the PDF version of this publication instead.

(Source: MCN 21-045, 6/11/21)

WORD IN WASHINGTON

The U.S>Senate has confirmed Dawn O'Connell as the Assistant Secretary for Preparedness and Response (ASPR) at the U.S. Department of Health and Human Services Secretary. Ms. O'Connell currently serves as senior counsel for COVID-19 to the HHS Secretary. According to a news release from her nomination in March, "[in the role of senior counsel], she coordinates the [d]epartment-wide response to the pandemic. Prior to assuming this role, [she] served on the[n] [President-elect] Biden['s] Transition Team as the health cluster lead for the Nominations Team. She was also the Director of the Coalition for Epidemic Preparedness and Innovation's (CEPI) U.S. Office…[Ms. O'Connell] also served as the executive director for CEPI's Joint Coordination Group—a roundtable of institutional partners who all have a vested interest in the successful development and deployment of epidemic vaccines. Prior to joining CEPI in June 2017, she was a Senior Counselor to Secretary Sylvia Burwell at [HHS] during the Obama Administration. As Senior

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WORD IN WASHINGTON (continued from page 4)

Counselor, Ms. O'Connell advised Secretary Burwell on high-priority global health and humanitarian issues, including infectious diseases, unaccompanied children, and refugees. She worked with HHS leaders, the White House, and other federal and international partners, to resolve key policy challenges, lead implementation, and drive progress toward Administration goals. She received her undergraduate degree in literature from Vanderbilt University and her law degree from Tulane University School of Law."

(Sources: *Bloomberg*, 2 High-Ranking HHS Nominees Sail Through Senate Confirmation, 6/24/21; White House News Release, 3/19/21)

RESEARCH IN BRIEF

Machine Learning Predicts Red Cell Demand. Authors of an article published in *Transfusion Medicine* describe the process of using machine learning to forecast demand for red blood cells (RBC). "Considering the lack of feasible and unified standards in the existing industry regulations and guidelines, clinicians rely on the empirical decision-making model of preoperative hemoglobin (Hb) + surgery type" stated the authors. "[H]ow to effectively restrict the volume of unnecessary RBC transfusion while meeting the minimum oxygen demand are urgent matters that need to be resolved…This can be accomplished retrospectively by analyzing the medical records of surgical patients, using preoperative parameters such as patient vital signs and surgical information, and adopting machine learning (ML) methods to establish mathematical models." "[The article] hypothesize[d] that [the authors'] method can effectively assist clinicians in making individualized and precise preoperative RBC preparations…During 2011–2017, electronic medical records of [surgical] patients [who were transfused] were collected." The authors "constructed a model for predicting the volume of RBCs required by patients based on preoperative data." They "employed the ML algorithm[s] to construct an accurate prediction model of RBC transfusion volume. Of "the 130,996 patients [a] total of 40,609 patients (31 percent) received RBC transfusion during surgery…Among the seven ML algorithms, the light gradient boosting machine (Lightgbm) algorithm was the best…Within the actual blood transfusion volume 0–10 units range, the artificial intelligence (AI) model was more accurate than doctors in predicting RBC of 0, 2, and 4 units, but not better for 1, 3, and 5–10 units…However, [the latter group] account[s] for only 15 percent of all data." The authors explained that, "[u]sing [this] model, [the study] predicted and prepared the required perioperative RBC transfusion volume based on the individual differences and dynamic changes in physiological conditions of each patient, which breaks the existing empirical model of "preoperative Hb + surgery type…This provides a strong reference for doctors with insufficient experience in blood transfusion, and provides more accurate predictions for doctors with experience…The prediction model will reduce the use of insufficient RBC blood reserve before operation," according to authors. "[I]t can reduce the excess blood reserve, save the cost of blood resources and compatibility testing, and improve blood resources for more operations."

Citation: Feng, Y., Xu, Z., Sun, X., Wang, D., Yu, Y. "Machine learning for predicting preoperative red blood cell demand." *Transfusion Medicine*. 2021.

Contributed by Richard Gammon, MD, Medical Director at OneBlood

INFECTIOUS DISEASES UPDATE

EBOLA

The World Health Organization (WHO) has declared the outbreak of Ebola in Guinea officially over. This outbreak began in February and resulted in 16 confirmed cases and seven probable cases with 12 deaths. "I commend the affected communities, the government and people of Guinea, health workers, partners and everyone else whose dedicated efforts made it possible to contain this Ebola outbreak," said Tedros Adhanom Ghebreyesus, PhD, WHO Director-General in a statement from the organization. "Based on the lessons learned from the 2014–16 outbreak and through rapid, coordinated response efforts, community engagement, effective public health measures and the equitable use of vaccines, Guinea managed to control the outbreak and prevent its spread beyond its borders. Our work in Guinea continues, including supporting survivors to access post-illness care." Approximately 24,000 Ebola vaccine doses were provided to Guinea by the WHO. The U.S. Centers for Disease Control and Prevention Director Rochelle P. Walensky, MD, MPH added in an agency news release, "I commend the government and first responders in Guinea for ending the country's Ebola outbreak. Our heartfelt sympathies are with the people who lost loved ones to this disease. CDC remains committed to supporting survivor programs and helping strengthen global preparedness and response capacities that can prevent or extinguish future Ebola outbreaks."

(Sources: WHO Announcement, 6/19/21; CDC News Release, 6/19/21)

MEMBER NEWS

LifeStream Blood Bank (San Bernardino, Calif.) celebrated its 70th anniversary of community service the week of June 13th. Formerly known as the Blood Bank of San Bernardino and Riverside Counties, before rebranding to LifeStream in 2008, the organization was founded June 19th, 1951 by the medical societies of San Bernardino and Riverside Counties. Today, those two counties have grown to a population of 4.4 million as LifeStream Blood Bank has extended its service area to include hospitals in Los Angeles, Orange, Imperial, and San Diego counties. Today, the blood bank provides blood products and services to more than 80 Southern California hospitals. "We were humbled and excited to mark this milestone and happy to share the celebration with our community and our generous, life-saving blood donors," said Rick Axelrod, MD, president, chief executive officer (CEO), and medical director of LifeStream Blood Bank, in an announcement from the organization. To celebrate "anniversary week," blood donors received a coupon for a sweet treat at Nothing Bundt Cakes, while local stores also generously donated a complimentary "bundlet" to all LifeStream employees. The blood bank's team members also took part in a colorful photo montage celebrating the occasion, a 70-year anniversary pin, and two face coverings that included the 70-year anniversary logo.

(Source: LifeStream Blood Bank Announcement, 6/22/21)

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The Blood Center (New Orleans, La.) has partnered with biolog-id LLC to use the company's platform to support "enhanced visibility, traceability," and management of platelets and antigen-negative red blood cells. "We are firm believers in leveraging technology to improve the efficiency, quality, and safety of the blood supply," said Billy Weales, president and CEO of The Blood Center, in the news release. "Our co-operation with biolog-id allows us to achieve these goals with two of our most critical blood components – platelets and rare red blood cells." Amit Mayer, vice president of Innovation and Analytics at biolog-id, added in the news release, "[o]ur cooperation with The Blood Center of New Orleans is a direct result of our efforts to develop solutions tailored to the specific needs of community blood centers. The complexity associated with the collection, manufacturing and distribution of platelets is only increasing, and we take great pride in the growing impact of our platelet lifecycle management tools on our growing customer base nationwide."

(Source: biolog-id, LLC News Release, 6/21/21) ♦

GLOBAL NEWS

Canadian Blood Services recently indicated that the organization will request by the end of 2021 that Canada's regulatory authority, Health Canada, replace the country's blood donor deferral policy for men who have sex with other men (MSM) with an individual risk-based policy, according to a report from *CTV News*. "We plan to make a submission to Health Canada by the end of the year and work is underway to build the submission," said Canadian Blood Services Spokesperson Catherine Lewis to the news outlet. A statement titled, "Evolving eligibility criteria for gay, bisexual and other men who have sex with men" on the Canadian Blood Services website reads, "[our] goal is to remove the current waiting period for men who have sex with men and use sexual behavi[o]r-based screening for all donors instead. To this end, we intend to make a submission recommending this change to Health Canada, our regulator, by the end of 2021. Currently, men are eligible to give blood if it has been more than three months since their last sexual contact with a man. As an incremental step, in 2019 we started the process of making a change in our source plasma eligibility criteria. In May 2021 we submitted the request for changes to Health Canada. If approved, some gay, bisexual, and other men who have sex with men will be able to donate plasma at select cente[r]s beginning this fall. This page describes where we've been, where we're going, and the next steps we're taking to get there. It also describes the history of Canadian Blood Services and how we work with our regulator, Health Canada, to protect Canada's blood supply."

(Sources: *CTV News*, Canadian Blood Services aiming to make submission in 2021 to remove blood ban, 6/18/21; Canadian Blood Services Statement, 6/18/21)

England, Scotland, and Wales have implemented changes, previously announced, to blood donation regulations. As of the week of June 14th, "[e]ligibility will be based on individual circumstances surrounding health, travel and sexual behavi[o]rs evidenced to be at a higher risk of sexual infection. Donors will no longer be asked if they are a [MSM], removing the element of assessment that is based on the previous population-based risks. Instead, any individual who attends to give blood — regardless of gender — will be asked if they have had sex and, if so, about recent sexual behavi[o]rs. Anyone who has had the same sexual partner for the last three months will be eligible to donate. The changes to the renamed Donation Safety Check form will affect blood, plasma, and platelet donors. The process of giving blood will not change…[P]eople can donate if they have had the same sexual partner for the last three months, or if they have a new sexual partner with whom they have not had anal sex, and there is no known recent exposure to a sexually transmitted infection or recent use of PrEP or PEP. This will mean more [MSM] will be eligible to donate. Anyone who has had anal sex with a new partner or with multiple partners in the last three months will not be able to give blood right now but may be eligible in the future. Donors who have

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been recently treated for [gonorrhea] will be deferred. Anyone who has ever received treatment for syphilis will not be able to give blood. The changes follow an evidence-based review into individuali[z]ed criteria by the FAIR (For the Assessment of Individuali[z]ed Risk) steering group." NHS Blood and Transplant (NHSBT), the national blood provider for England and transplant services for the United Kingdom (UK), Chief Nurse Ella Poppitt stated in a NHSBT announcement, "We screen all donations for evidence of significant infections, which goes hand-in-hand with donor selection to maintain the safety of blood sent to hospitals. All donors will now be asked about sexual behavi[o]rs which might have increased their risk of infection, particularly recently acquired infections. This means some donors might not be eligible on the day but may be in the future. Our priority is to make sure that donors are able to answer the pre-donation questions in a setting that makes them feel comfortable and safe and donation is something that continues to make people feel amazing. Our staff have been trained to make sure these more personal conversations are conducted with care and sensitivity and accurate information is captured. We are asking all blood, plasma, and platelet donors to please consider the new questions alongside the existing health and travel questions before their appointment, and to re-schedule if they do not meet the changed criteria to donate right now. We want donation to be a positive experience and we are looking forward to welcoming donors as we move forward with these changes."

(Source: NHSBT Announcement, 6/14/21)

COMPANY NEWS

Updated results from a phase III trial for **uniQure N.V.**'s investigational hemophilia b gene therapy candidate have been announced. The company provided "positive" 52-week clinical data" for all patients from its HOPE-B gene therapy trial. "Data [showed] that participants continued to demonstrate durable, sustained increases in Factor IX (FIX) activity at 52-weeks post-infusion with a mean FIX activity of 41.5 percent of normal, as measured by a one-stage APTT-based clotting assay, compared to a mean FIX activity of 39.0 percent of normal at 26-weeks of follow-up. There continued to be no clinically significant correlation between pre-existing neutralizing antibodies to [adeno-associated virus five] (AAV5) (NAbs) and FIX activity in patients with NAb titers up to 678.2, a range expected to include more than 93 percent of the general population. During the 52-week period, a single dose of [the gene therapy] significantly reduced the annualized rate of bleeding requiring treatment by 80 percent from a prospectively collected 3.39 at baseline to 0.68 bleeding episodes per year (p-value <0.0001). The annualized rate of spontaneous bleeding requiring treatment was also significantly reduced by 85 percent from a prospectively collected 1.16 at baseline to 0.18 bleeds per year during the 52-week period (p-value <0.0001). Usage of FIX replacement therapy (IU/year and infusions/year) in all patients declined 96 percent during the 52-week period, with 52 of 54 patients (96 percent) successfully discontinuing their prophylactic infusions," according to a company news release. The company also noted that the gene therapy, Etranacogene dezaparvovec, "continues to be generally well-tolerated with no treatment-related serious adverse events. No inhibitors to FIX have been reported and no consistent relationship between safety and pre-existing NAb titers has been observed." uniQure and its partner CSL Behring remain in communication with the U.S. Food and Drug Administration (FDA) and have held a pre-biologics licensing application (BLA) submission meeting with the agency. "We continue to be very encouraged by the data generated from the HOPE-B pivotal study of etranacogene dezaparvovec," stated Ricardo Dolmetch, Ph.D., president of Research and Development at uniQure, in the news release. "The 52-week data show mean FIX activity in the normal range and increase our confidence in the potential durability and long-term benefits of etranacogene dezaparvovec, bringing us one step closer to our goal of delivering this groundbreaking therapy to fulfill an unmet medical need for patients living with hemophilia B." In April, **uniQure** shared that FDA has "removed the clinical hold" on its gene therapy

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program to treat hemophilia B. The hold had been in place since December when a patient in a clinical trial for the company's gene therapy candidate was diagnosed with epatocellular carcinoma (HCC).

(Source: uniQure N.V. News Release, 6/22/21)

Blood has published results from the phase III clinical trial of ***Gamida* Cell Ltd.**'s investigational advanced cell therapy, omidubicel, as an allogeneic hematopoietic stem cell transplant solution for patients with hematologic malignancies. According to the company news release, "omidubicel demonstrated a statistically significant reduction in time to neutrophil engraftment, a measure of how quickly the stem cells a patient receives in a transplant are established and begin to make healthy new cells and a key milestone in a patient's recovery from a bone marrow transplant. The median time to neutrophil engraftment was 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p<0.001$). All three secondary endpoints [demonstrated] a statistically significant improvement among patients who were randomized to omidubicel compared to patients randomized to standard cord blood graft. Platelet engraftment was significantly accelerated with omidubicel, with 55 percent of patients randomized to omidubicel achieving platelet engraftment at day 42, compared to 35 percent for the comparator ($p = 0.028$). Hospitalization in the first 100 days after transplant was also reduced in patients randomized to omidubicel, with a median number of days alive and out of hospital for patients randomized to omidubicel of 61 days, compared to 48 days for the comparator ($p=0.005$). The rate of infection was significantly reduced for patients randomized to omidubicel, with the cumulative incidence of first grade 2 or grade 3 bacterial or invasive fungal infection for patients randomized to omidubicel of 37 percent, compared to 57 percent for the comparator ($p=0.027$). Additional data reported in the manuscript included a comparison of infection density, or the number of infections during the first year following transplantation, which showed that the risk for grade 2 and grade 3 infections was significantly lower among recipients of omidubicel compared to control (risk ratio 0.5, $p<0.001$). Data from the study relating to exploratory endpoints also support the clinical benefit demonstrated by the study's primary and secondary endpoints. There was no statistically significant difference between the two patient groups in incidence of grade 3/4 acute GvHD (14 percent for omidubicel, 21 percent for the comparator) or all grades chronic GvHD at one year (35 percent for omidubicel, 29 percent for the comparator). Non-relapse mortality was shown to be 11 percent for patients randomized to omidubicel and 24 percent for patients randomized to the comparator ($p=0.09$)." Gamida Cell intends to submit a BLA to the FDA later this year. "We are pleased that the data from this well-conducted international Phase III trial have been published in *Blood*, the highly respected, peer-reviewed journal of the American Society of Hematology," said Ronit Simantov, MD, chief medical officer of Gamida Cell, in the news release. "The robust results of this clinical trial have demonstrated that omidubicel could provide an important new option for patients with hematologic malignancies in need of a bone marrow transplant."

(Source: Gamida Cell Ltd. News Release, 6/23/21)

bluebird bio, Inc. recently announced results from multiple ongoing clinical trials of its investigational gene therapy candidate, betibeglogene autotemcel (beti-cel) (licensed as ZYNTEGLO™ in the European Union and United Kingdom) to treat tranfusion-dependent beta thalessassemia (TDT). Results shared in the news release include, "[a]fter participating in and completing the two years of follow-up in any of the Phase I/II or Phase III studies (HGB-207, HGB-212), patients treated with beti-cel were invited to enroll in a 13-year long-term follow-up study, LTF-303. As of March 9, 2021, 51 of 63 beti-cel-treated patients across age groups and genotypes spanning a broad range of TDT severity have completed two years of follow-up in the parent study and were enrolled in LTF-303 (22 treated in Phase I/II studies, 29 treated in Phase 3 studies) with a median post-infusion follow-up of 44.2 months (min-max: 22.9 – 86.5). Of the 51 patients enrolled in LTF-303, 40 patients achieved transfusion independence (TI): 15/22 (68 percent) patients treated in Phase I/II and 25/29 (86 percent) patients treated in Phase 3. All patients achieved TI in the parent

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studies and maintained it through last follow-up in LTF-303. As of the data cut-off date, all patients who achieved TI remained free from transfusions through their last follow-up (n=40). Phase 1/2 patients had a median duration of ongoing TI of 57.1 months (min-max: 15.8 – 84.1) and Phase 3 patients had a median ongoing TI duration of 26.3 months (min-max: 13.1 – 39.4)…Prior to beti-cel infusion, all patients were on iron chelation, which is needed to reduce excess iron caused by chronic blood transfusions. Of the 40 patients who achieved TI following treatment with beti-cel, 73% (29/40) restarted iron chelation after beti-cel infusion and the majority (59%, 17/29) of patients who restarted iron chelation after infusion have since stopped; and 28% (11/40) were able to receive phlebotomy (blood removal), which is a preferred method for iron reduction. Of the 11 patients who were able to receive phlebotomy, 10 have not received phlebotomy in more than seven months and their total unsupported Hb at last study visit ranged from 10.5 to 14.0 g/dL. There were no deaths, and no vector-derived replication-competent lentivirus nor events of insertional oncogenesis or malignancy have been reported in patients enrolled in LTF-303. No drug-related adverse events (AEs) were reported in the long-term follow-up study…As of March 9, 2021, 41 patients were treated in the Phase 3 studies HBG-207 (Northstar-2; n=23; median follow-up 24.3 months [min-max: 13.0 - 27.5]); and HGB-212 (Northstar-3; n=18; median follow-up 23 months [min-max: 4.1 – 26.8]).Following treatment with beti-cel, 89% (32/36) of evaluable patients across ages and genotypes in both Phase 3 studies achieved transfusion independence (TI). As of the data cut-off date, these patients continue to be free of transfusions for a median duration of 25 months (min-max: 12.5 – 38.5), with median weighted average total hemoglobin levels during TI of 11.6 g/dL (min-max: 9.3 – 13.7). Median gene therapy-derived hemoglobin (HbAT87Q) was stable approximately six months post-infusion: 8.8 g/dL at Month 6 (n=33); 9.2 g/dL at Month 9 (n=34); 8.7 g/dL at Month 12 (n=36); 9.3 g/dL at Month 18 (n=29); and 8.9 g/dL at Month 24 (n=26). In exploratory analyses, biomarkers of ineffective erythropoiesis trended toward normal over time in patients who achieved TI, supporting the disease-modifying potential of beti-cel in patients with TDT; additionally, biomarkers of hemolysis normalized in patients who achieved TI…Grade ≥3 veno-occlusive liver disease in three patients was attributed to busulfan conditioning and resolved with defibrotide treatment. One patient developed serious, Grade 3 congestive heart failure unrelated to drug product, which was downgraded to Grade 1 at 5 months and resolved at 12 months. Adverse events (AEs) considered related or possibly related to the drug product included thrombocytopenia (n=3), abdominal pain (n=3), leukopenia (n=1), neutropenia (n=1), pain in extremity (n=1), tachycardia (n=1) and autoimmune disorder (n=1)…There were no deaths, no graft failures or graft-versus-host disease (GVHD), and no cases of replication-competent lentivirus, insertional oncogenesis, clonal predominance, or malignancy…As of March 9, 2021, 27 pediatric patients (<12 years: n=16; ≥12 to <18 years: n=11) were treated in the Phase 3 HGB-207 (Northstar-2) and HGB-212 (Northstar-3) studies and had a median follow-up of 25.5 months (min-max: 4.1 – 41.5 months). Following treatment with beti-cel, 91% (20/22) of evaluable patients under the age of 18 years (ages 4 to 17), including 10 patients under age 12, achieved transfusion independence (TI). These patients continue to be free of transfusions through their last follow-up, with median weighted average Hb levels during TI of 10.0 g/dL in patients under age 12 (n=10) and 11.7 g/dL in patients age 12-18 (n=10). The median baseline score for 18 patients who achieved TI was 79.90 (range, 47.83-97.83; n=18) on the PedsQL-4.0; healthy children reach scores of approximately 84. At 24 months, improvement in quality of life was approximately three-fold higher than the minimal clinically significant meaningful difference (MCMD, a change between 4.30 and 4.83 points) as measured by the PedsQL-4.0. Improvements were more pronounced in patients with more severe scores at baseline, showing greater than five-fold higher improvements over the MCMD. Adverse events (AEs) in pediatric patients during the HGB-207 and HGB-212 trials that were considered related or possibly related to beti-cel were non-serious and included tachycardia (Grade 1, n=1) and abdominal pain (Grade 1, n=2) on the day of infusion, and Grade 3 thrombocytopenia in one patient post-infusion. There were no deaths, no graft failures or GVHD, and no cases of replication-competent lentivirus or insertional oncogenesis. No clonal predominance has been observed. Grade 4 veno-occlusive liver disease occurred in two patients (ages ≥12

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to <18 years) and one Grade 2 event occurred in a patient age <12 years; all events resolved after treatment with defibrotide."

(Source: bluebird bio, Inc. News Release, 6/11/21)

Upcoming ABC Webinars – Don't Miss Out!

- **ABC SMT Journal Club Webinar** – July 26th from 3 – 4 p.m. (EDT). More details coming soon.
- **ABC QA Education Webinar: What Worked? What Didn't? Doing Business in a different Way During the Pandemic** – July 20th from 3 – 4:30 p.m. (EDT). More details coming soon.

ABC Calendar of Events

ABC offers a variety of meetings, workshops and virtual opportunities for education and networking as well as participation in ABC business. The calendar of events includes annual and summer meetings, board meetings, workshops, and webinars, and details will be updated as confirmed. We look forward to your support and participation!

CALENDAR

Note to subscribers: *Submissions for a free listing in this calendar (published weekly) are welcome. Send information to newsletter@americasblood.org or by fax to (202) 899-2621. (For a more detailed announcement in the weekly "Meetings" section of the newsletter, please include program information.)*

2021

July 19-23. **U.S Food and Drug Administration (FDA) Regulatory Education for Industry (REdI) Annual Conference 2021 (Virtual).** Registration is open.

Aug. 4. **ABC Medical Directors Workshop, Cleveland, Ohio**. Registration is open.

Aug. 5-6. **ABC Summer Summit, Cleveland, Ohio.** Registration is open.

Aug. 17-19. **2021 ADRP Conference, Kansas City, Mo.** Registration is open.

Sept. 15-17. **4th European Conference on Donor Health and Management, Hamburg, Germany.** Registration is open.

Sept. 22. **11th Annual Symposium on Red Cell Genotyping 2021: The New Normal, Bethesda, MD (Hybrid)**. For more information click here or contact Natasha Leon.

Oct. 17-19. **AABB Annual Meeting (Virtual).** More details available here.

Nov. 3-4. **The Biomedical Advanced Research and Development Authority (BARDA) Industry Day (Virtual).** More details available here.

CLASSIFIED ADVERTISING

Classified advertisements, including notices of positions available and wanted, are published free of charge for a maximum of three weeks per position per calendar year for ABC institutional members. There are charges for non-members: $139 per placement for *ABC Newsletter* subscribers and $279 for non-subscribers. A six (6) percent processing fee will be applied to all credit card payments. Notices ordinarily are limited to 150 words. To place an ad, e-mail: newsletter@americasblood.org

POSITIONS

VP/Chief Medical Officer. LifeStream Blood Bank, located in San Bernardino, California, is seeking a VP/Chief Medical Officer to provide leadership and direction for the medical programs needed to support the organization. This includes all laboratories, product management, hospital relations, donor collections, therapeutic and clinical services, clinical research, donor counseling, national marrow programs and quality departments. LifeStream distributes approximately 130,000 Red Cells and 30,000 Platelets to 80 hospitals in southern California. LifeStream has a vibrant and growing therapeutic apheresis program, active cellular collections program and a strong reference laboratory. This position reports to the President/CEO, is a member of the executive team and has administrative responsibility for the Reference Laboratory and an Assistant Medical Director. Qualifications for this position include a minimum of five years medical experience at a blood center or hospital-based transfusion medicine practice, California medical license eligibility, Board Certification in Clinical Pathology or other relevant medical specialty and Board Certification in Transfusion Medicine. The compensation package includes a strong competitive salary, relocation package and best in class benefits including 100% employer paid family medical and dental insurance. Interested candidates are encouraged to send their resume/CV to Judy Taylor, Director, Human Resources at taylorju@lstream.org.

Technical Manager. LifeSouth Community Blood Centers is currently seeking a skilled individual for a Technical Manager position in Gainesville, FL. This position is responsible for managing production through subordinate coordinators and staff. The selected candidate will also be fully accountable for costs, methods, personnel, quality, inventory and distribution within the Components Laboratory and Hospital Services departments. Bachelor's degree in a science related field required. Certified Medical Laboratory Technician (MLT) with experience in transfusion services required. Medical Technologist (MT) license preferred. Previous management experience preferred. Background check and drug test required. Equal Opportunity/Affirmative Action Employer/DFWP/Tobacco Free. VEVRAA Federal Contractor. Click here to apply.