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The Stress of Sickle Cell Disease

Those who care for patients with sickle cell disease know that the first 24 hours after they seek medical help for sickle cell-related complications is a particularly critical period. Close monitoring is needed to assess the severity of the situation, whether it is acute chest syndrome, worsening anemia, a painful vasoocclusive crisis, stroke or organ failure. Immediate and aggressive treatment is imperative.
Progress Toward a Cure for Sickle Cell Disease

ESTIMATES INDICATE THAT MORE THAN 100,000 PEOPLE HAVE SICKLE CELL DISEASE (SCD) IN THE U.S. ALONE, THOUGH MANY MORE CARRY THE SICKLE CELL GENE. WORLDWIDE, SCD IS THOUGHT TO AFFECT MILLIONS — MANY OF WHOM REMAIN UNDIAGNOSED.

Recent research has accelerated understanding of and, subsequently, treatments for SCD. Studies have also advanced our knowledge about optimal management of the condition, offered new treatment options and made progress on potential cures. However, SCD remains a significant public health issue. It leads to tens of thousands of hospitalizations in the U.S. each year and generates medical costs of hundreds of millions of dollars. Furthermore, life expectancy for patients with SCD remains lower than for the general population.

Because AABB members provide resources and services used by almost all patients with SCD, our association has been long committed to these patients and supported research on treatments and potential cures for this condition. Basic and clinical research both directly and indirectly linked to SCD has been published in Transfusion, AABB’s peer-reviewed scientific journal, and featured regularly at AABB’s annual meetings. AABB works actively to reach a cure through our membership in the Sickle Cell Disease Coalition and involvement in events to raise awareness and promote research for the condition, particularly during National Sickle Cell Awareness Month, held every September. In this issue of AABB News, we highlight SCD and the patients affected by this condition. Our feature article, beginning on page 10, examines the current state of treatments for patients with SCD and illustrates how researchers are making progress on finding a cure for the condition.

On another note, since my term as president of AABB ends with this month’s Annual Meeting, this will be my final President’s Message in these pages. I’ll be handing over the presidency, as well as this column, to my friend and longtime AABB member, Mary Beth Bassett, who will lead our association for the next year. I have worked with Mary Beth on AABB’s Board of Directors for many years; I am certain that AABB will be in good hands under her leadership.

And finally, it has been my honor to serve as president of our association during this past year. Having been blessed with the support of the superb Board of Directors, excellent AABB staff, and, most importantly, by your understanding of our actions accompanied by input from the many of you with whom I have worked throughout my tenure. The most sincere thank you to my fellow AABB members. 謹

Zbigniew M. Szczepiorkowski, MD, PhD, FCAP
AABB President
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In what it calls an “historic action,” the Food and Drug Administration approved a chimeric antigen receptor- (CAR-) T cell immunotherapy on Aug. 30. The therapy, tisagenlecleucel (Kymriah, Novartis Pharmaceuticals Corp.), is approved to treat patients who are younger than 25 years and have B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.

In July, FDA’s Oncologic Drugs Advisory Committee unanimously recommended approval of tisagenlecleucel (formerly known as CTL019) for this indication. The recommendation was based on the results of a phase II clinical trial of 63 patients. The overall remission rate among patients in the study was 83% at three months. The probability of being relapse-free at six and 12 months was 75% and 64%, respectively.

The approval requires a post-marketing long-term safety study and a risk evaluation and mitigation strategy. Specifically, FDA asks that hospitals and associated clinics that dispense tisagenlecleucel be certified, and staff trained to recognize and manage cytokine release syndrome (CRS), a life-threatening side effect, and other potentially life-threatening neurologic complications. In the phase II study, 78% of patients experienced CRS within eight weeks of infusion, with almost half at grade 3 or 4 severity. The approval of tisagenlecleucel also comes with an expanded approval for tocilizumab (Actemra, Genentech USA Inc.) to treat CRS. Certified hospitals and clinics are required to have protocols in place to ensure that tocilizumab is available for immediate administration.

Abnormalities in the function of plasma lipoproteins suggest that replacing only red blood cells to treat vasoocclusive crisis in patients with sickle cell disease (SCD) may not be as effective as commonly believed.

While it’s known that patients with SCD have decreased levels of cholesterol associated with abnormal cholesterol metabolism, researchers now better understand these abnormalities and their implications for treating the disease.

The researchers have identified alterations in the ratio of larger and smaller particles of high density lipoprotein (HDL), also known as good cholesterol, in the plasma of patients with SCD, along with altered function of those components that is proinflammatory. These alterations are exacerbated during acute vasoocclusive episodes, which are commonly treated with transfusion of healthy red blood cells to replace ‘sickled’ RBCs. According to the researchers, simply replacing RBCs without addressing alterations in plasma might not be optimal. Instead, they suggest that whole-blood transfusions could be a better treatment for these patients. The findings were published in the journal Experimental Biology and Medicine.

ENDNOTES: