New Gene Therapy for β-Thalassemia

The FDA recently approved the first cell-based gene therapy for patients with β-thalassemia who require red blood cell transfusions.

Thalassemia reduces the production of hemoglobin in the blood. Many people with the condition have such severe anemia that they require red blood cell transfusions, typically every 2 to 5 weeks, for their entire life. Worldwide, thousands of infants each year are born with thalassemia, which is an autosomal recessive inherited condition. It occurs most frequently in people from Mediterranean countries or their descendants.

bluebird bio, the Somerville, Massachusetts, company that is marketing the new gene therapy, estimates that 1300 to 1500 people in the US have transfusion-dependent thalassemia. These transfusions lead to iron overload, which must be treated with chelation therapy to prevent an early death, according to the Cooley’s Anemia (another name for β-thalassemia) Foundation.

The new treatment, betibeglogene autotemcel (Zynteglo), uses a patient’s own bone marrow stem cells that have been genetically modified to produce a hemoglobin component, the FDA noted. In 2 clinical trials involving 41 patients who received the gene therapy, 89% didn’t need a red blood cell transfusion for at least 12 months.

“While advances in treatment have been of enormous benefit to those with beta-thalassemia, a potentially curative therapy may offer a true life-changing experience,” Craig Butler, national executive director of the Cooley’s Anemia Foundation, said in a statement from the bluebird bio news release.

Because of its complexity, the new gene therapy will be available only at qualified treatment centers selected based on their expertise in stem cell transplant, cell and gene therapy, β-thalassemia, and other relevant areas, according to bluebird bio. The centers are receiving specialized training in administering the therapy, and bluebird bio has set up a website, https://www.mybluebirdsupport.com/ for more information.

Some Hearing Aids Will Soon Be Sold Over the Counter

Over-the-counter hearing aids could be available in US retail stores by mid-October as a result of a new FDA final rule.

The rule enables adults 18 years or older with mild to moderate hearing impairment to buy certain air-conduction hearing aids without a medical examination, prescription, or fitting by an audiologist. Hearing aids for adolescents and children younger than 18 years or for people with severe hearing impairment remain available only by prescription.

Hearing loss affects an estimated 30 million US adults, but only about 20% of those who could benefit from hearing aids try to get one, according to the final rule. Some of them might have been unable to afford prescription hearing aids that require professional services such as fitting, adjustment, or maintenance.

The FDA received and reviewed more than 1000 public comments on the proposed rule, which it issued in October 2021. Some commenters said hearing aids are medical devices, not consumer electronics, so they shouldn’t be available without a prescription, according to the rule.

In response to the comments and to ensure the safety and effectiveness of over-the-counter hearing aids, the FDA made several changes from the proposed rule. Among them: lowering the maximum sound output to reduce the risk to hearing, requiring adjustable volume control, and simplifying device labeling.

Along with the final rule about over-the-counter hearing aids, the FDA issued a final guidance to clarify the difference between hearing aids, which are medical devices, and personal sound amplification products, which are consumer products that amplify sound for people with normal hearing.

Congress had passed the bipartisan Over-the-Counter Hearing Aid Act of 2017, signed into law by then-President Donald Trump, but it wasn’t fully implemented until now.

The new final rule follows President Joe Biden’s executive order in 2021 to increase competition among key businesses and lower costs. In a statement, Biden said the new final rule will reduce the cost of a pair of hearing aids by nearly $3000.

Blood Donors Need Not Be Screened for Monkeypox

The FDA recently recommended against blood banks asking donors specific questions about possible exposure to the monkeypox virus or using laboratory diagnostic tests to screen them for it.

There have been no reports of transmission of monkeypox virus through blood transfusion, and existing safeguards for blood safety, which include requiring that donors be in good health and have a normal temperature on the day they donate, are robust, according to the FDA. In addition, donors are usually asked to report if they become ill after donating blood so that the blood bank can determine whether it’s safe to transfuse.

Meanwhile, the agency said it will continue to monitor US monkeypox cases and available information about the risk of transmitting the virus in blood, which remains theoretical.

As of August 26, more than 17 000 cases of monkeypox had been reported in the US, and nearly 48 000 had been reported worldwide, according to the US Centers for Disease Control and Prevention. – Rita Rubin, MA

Note: Source references are available through embedded hyperlinks in the article text online.