food and drug administration Approves First-of-its-Kind Drug to Treat Rare Blood Disorder

http://www.firstlight.cn 2007-03-30

March 16, 2007， The U.S. Food and Drug Administration (FDA) today approved Soliris (eculizumab), the first product for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), a rare type of blood disorder that can lead to disability and premature death. Soliris is classified as an Orphan Drug and is a new molecular entity containing an ingredient not previously marketed in the United States.

"This product is important in that it offers a treatment other than blood transfusion that may help this small population of patients who are often very ill," said Steven Galson, M.D., M.P.H., director, Center for Drug Evaluation and Research, FDA. "This approval is one of multiple examples of how the orphan products program can benefit the public health with urgently needed products that would otherwise not be commercially available."

PNH, which usually develops in adults, is a disease characterized by red blood cells that develop abnormally. Once the abnormal cells are present in the bloodstream, naturally occurring proteins (called the complement system) designed to destroy bacteria and other infection-causing organisms break these cells down. This leads to abnormally darkened urine and, more importantly, causes anemia. Depending upon the severity of the disorder, patients with PNH may have pain, fatigue and debilitating weakness, the need for frequent blood transfusions, blood clots, and life-threatening or fatal strokes, heart attacks and intestinal disease.

Soliris does not cure PNH, but treats the breakdown of red blood cells, the most common characteristic of PNH. Soliris acts to block the complement system activity, including the destruction of PNH red blood cells.

FDA based its approval on the company's randomized, double-blind, placebo-controlled clinical study of 87 patients with PNH and a series of other clinical studies. The controlled study showed that over a 26-week period half of the participants receiving Soliris had stabilization of blood hemoglobin concentrations compared with no stabilization among placebo-treated patients. Soliris-treated patients also required significantly fewer blood transfusions.

Soliris' blockade of the body's natural immune system increases the patient's susceptibility to certain serious infections, particularly meningococcal infections that can cause bacterial meningitis, an infection of the tissue surrounding the spinal cord and brain. Serious meningococcal infection was the most important adverse reaction experienced by patients who received Soliris in clinical studies. Because of the high risk for serious meningococcal infections, all 196 PNH patients in the clinical studies were vaccinated with a meningococcal vaccine; two of them developed meningococcal sepsis (an infection of the bloodstream caused by meningococcal bacteria) during treatment with Soliris.

A special risk management plan for Soliris has been developed to address the risk of serious meningococcal infection. The labeling of the product contains a boxed warning and requires that patients receive meningococcal vaccination prior to receiving Soliris. The labeling also includes a Medication Guide for patients. In addition, the risk management plan includes an educational program for physicians.

Orphan products are developed to treat rare diseases or conditions that affect fewer than 200,000 people in the United States. The Orphan Drug Act provides a seven-year period of exclusive marketing to the first manufacturer who obtains marketing approval for a designated orphan product. About one person out of a million people will be diagnosed with PNH.

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