New Treatment Option For Patients With A Rare Genetic Disorder

(NAPSA)—There's encouraging news for patients who suffer from a rare disease. According to the National Institutes of Health, there are approximately 6,800 rare diseases that collectively affect nearly 30 million Americans. Patients with rare diseases and their loved ones struggle with the fact that there are very few treatments available for the vast majority of these diseases.

Fortunately, patients with Gaucher disease, a rare genetic disorder, now have a new treatment option available.

The U.S. Food and Drug Administration (FDA) recently approved Elelyso (taliglucerase alfa) for injection, an enzyme replacement therapy (ERT) to treat Type 1 Gaucher disease in adults. People with Gaucher disease lack an enzyme called glucocerebrosidase, which causes a fatty substance called glucocerebroside to accumulate in certain cells in the body. The accumulation of these fats can cause symptoms such as enlargement and malfunctioning of the liver, spleen and bone marrow.

Elelyso replaces the missing enzyme and allows for normal functioning and breakdown of the lipid molecules within the cells, leading to normal metabolism within the cell. In a clinical trial of 31 patients, Elelyso reduced spleen and liver size and improved hemoglobin levels and platelet counts.

Kim Rozzi has been living with Gaucher disease for over 20 years. "As a Gaucher patient, I am excited about the FDA approval of Elelyso because there is a new option available," said Kim.

With the approval of Elelyso, Pfizer has also developed the Supply Continuity Program. This program plans to maintain a continuously restocked 24 months of supply at various stages of production for U.S. patients.

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In addition to the Supply Continuity Program, Pfizer will also provide a specialized patient support service entitled Gaucher Personal Support (GPS), which staffs a dedicated team of health care specialists who are available to help Gaucher disease patients and their families with services including reimbursement assistance and locating infusion services.

For more information about Elelyso, including product and prescribing information, please see www.ELELYSO.com or call (855) ELELYSO (1-855-353-5976) for a free patient information kit about Elelyso and the GPS program.

Important Safety Information

As with any intravenous protein medicine, like enzyme replacement therapy (ERT), severe allergic reactions (including anaphylaxis) have been observed in patients treated with Elelyso. If this occurs, your doctor may immediately discontinue Elelyso. Patients who have experienced anaphylaxis to Elelyso or another ERT should proceed with caution upon retreatment.

In addition, infusion reactions (including allergic reactions)—defined as a reaction occurring within 24 hours of the infusion—were the most commonly observed reactions to Elelyso. The most commonly observed infusion reactions were headache, chest pain or discomfort, weakness, fatigue, hives, abnormal redness of the skin, increased blood pressure, back or joint pain, and flushing. Most of these reactions were mild and did not require treatment.

Management of infusion reactions is based on the type and severity of the reaction. Your doctor may manage infusion reactions by temporarily stopping the infusion, slowing the infusion rate, or treating with medications such as an antihistamine and/or a fever reducer. Treatment with antihistamines and/or corticosteroids prior to infusion with Elelyso may prevent these reactions.

Other common adverse reactions observed were upper respiratory tract infections, throat infection, flu, urinary tract infection, and pain in extremities.

As with all therapeutic proteins, including ERTs, there is a possibility of developing antibodies to Elelyso. However, it is currently unclear whether this has an impact on the clinical response or adverse reactions. Patients with an immune response to other ERTs who are switching to Elelyso should continue to be monitored for antibodies. Comparison of the frequency of antibodies across ERTs may be misleading.

If you are pregnant or plan to become pregnant, you should talk to your doctor about potential benefits and risks.