



Safety and Efficacy of ELELYSO® (taliglucerase alfa) for Injection in Pediatric Patients with Type 1 Gaucher Disease in Long-term Outcome Study Presented at the WORLDSymposium 2015

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Pfizer Inc. (NYSE: PFE) today announced that researchers presented new data which expand on the existing body of data for ELELYSO (taliglucerase alfa) for injection in pediatric patients with Type 1 Gaucher disease. These data, which were released today in an oral presentation at the 11th Annual *WORLDSymposium* in Orlando, include results from a Phase 3, multi-center, extension trial evaluating the long-term efficacy and safety of ELELYSO in pediatric patients with Type 1 Gaucher disease who were treatment-naïve or previously treated with imiglucerase.

This pediatric-specific extension trial included 15 patients aged two to 18 years from two study cohorts. The first study cohort included 10 previously treatment-naïve pediatric patients treated with ELELYSO (9 who completed) for up to 36 months (the first 24 months of which were conducted in a double-blind fashion) at 30 units per kg or 60 units per kg. The second study cohort included five pediatric patients who were previously switched from imiglucerase (2 who completed) and administered ELELYSO for a total of 33 months at the same dose as each patient's previous imiglucerase dose. At the end of the study, treatment-naïve patients treated with ELELYSO for 36 months demonstrated

clinical improvements, as measured by a decrease in spleen and liver volume and an increase in platelet count. In patients previously switched from imiglucerase to ELELYSO, mean spleen and liver volume, platelet count and hemoglobin value remained stable through 33 months of ELELYSO treatment.

The most common adverse events reported were upper respiratory tract infection, cough, headache, abdominal pain, diarrhea, nasopharyngitis, pain in extremity, swollen or enlarged lymph nodes and fever.

“The data presented at the WORLDSymposium contribute to our understanding of ELELYSO as a long-term enzyme replacement therapy for patients with a confirmed diagnosis of Type 1 Gaucher disease,” said lead trial investigator Ari Zimran, MD, Gaucher Clinic, Sha’are Zedek Medical Center, Jerusalem, Israel. “This study further underscores the clinical benefit of ongoing treatment with ELELYSO in pediatric patients with Type 1 Gaucher disease.”

ELELYSO is approved in the United States for long-term enzyme replacement therapy (ERT) for adult and pediatric patients with a confirmed diagnosis of Type 1 Gaucher disease, an inherited lysosomal storage disorder in humans that affects an estimated 10,000 people worldwide. The recommended dosage of ELELYSO for treatment-naïve adult and pediatric patients four years of age and older is 60 units per kg of body weight administered every other week as a 60 to 120 minute intravenous infusion.

INDICATION

ELELYSO® (taliglucerase alfa) for injection is indicated for long-term enzyme replacement therapy (ERT) for adults and children with a confirmed diagnosis of Type 1 Gaucher disease.

IMPORTANT SAFETY INFORMATION

Serious hypersensitivity reactions including anaphylaxis have occurred in some patients treated with ELELYSO® (taliglucerase alfa) for injection. When treated with ELELYSO your doctor should monitor you before and after infusion for reactions.

Medical support should be readily available when ELELYSO is given. Discontinue ELELYSO immediately if you show signs or symptoms of anaphylaxis during infusion and get immediate medical care. Signs and symptoms of anaphylaxis include hives, low blood pressure, flushing, wheezing, chest tightness, nausea, vomiting, and dizziness.

Signs and symptoms of hypersensitivity include itching, swelling under the skin, flushing, redness, rash, nausea, vomiting, cough, chest tightness, and throat irritation. These reactions occurred up to 3 hours after the start of infusion.

Management of hypersensitivity reactions is based on the severity of the reaction. Your doctor may manage the reactions by slowing or temporarily stopping the infusion, and/or treating with medications such as an antihistamine, a fever reducer, and/or corticosteroids for mild reactions. Treatment with antihistamines and/or corticosteroids prior to infusion with ELELYSO may prevent these reactions from reoccurring. If severe hypersensitivity reactions occur, immediately stop the infusion of ELELYSO and get immediate medical care.

You should be carefully re-evaluated for treatment with ELELYSO if serious or hypersensitivity reactions including anaphylaxis occur.

The most common adverse reactions for ELELYSO are itching, flushing, headache, joint pain, pain in extremity, abdominal pain, vomiting, fatigue, back pain, dizziness, nausea, and rash. Vomiting occurred more often in children than adults and this may be due to a hypersensitivity reaction. The occurrence of other adverse reactions was similar between children and adults.

The recommended dosage of ELELYSO for adults and children who are 4 years of age and older and not taking another enzyme replacement therapy (ERT) is 60 Units per kg of body weight given every other week as a 60- to 120-minute intravenous infusion.

As with all therapeutic proteins, including ERTs, there is a possibility of developing antibodies to ELELYSO. The relationship between developing antibodies and hypersensitivity reactions is not clear. Your doctor should monitor you for antibodies to ELELYSO if you have developed antibodies or if you have experienced hypersensitivity reactions to ELELYSO or other ERTs.

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

ELELYSO is supplied as 200 Units per vial and is available by prescription only.

For full prescribing information for Elelyso click [here](#).

The health information contained herein is provided for educational purposes only and is not intended to replace discussions with a health care provider. All decisions regarding patient care must be made with a health care provider, considering the unique characteristics of the patient. This product information is intended only for residents of the United States.

About Gaucher Disease

Gaucher disease is an inherited lysosomal storage disorder in humans that affects an estimated 10,000 people worldwide and can cause severe and debilitating symptoms, including: enlargement of the liver and spleen, various forms of bone disease, easy bruising, and anemia (a low number of red blood cells).

Gaucher disease consists of varying degrees of severity; it has been sub-divided into three subtypes - Types 1, 2, and 3 - according to the presence or absence of neurological involvement. Type 1, the most common, is found at a higher frequency among individuals who are of Ashkenazi Jewish ancestry.

Pfizer and Rare Diseases

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than a decade of experience and a global portfolio of 22 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

Pfizer Inc.: Working together for a healthier world®

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at www.pfizer.com.

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