Sanofi Presents Research Advances in Rare Genetic Diseases at 15th Annual WORLDSymposium™ 2019

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Continued research and development is critical to advancing care for people with rare diseases who have a significant need for earlier diagnosis, better disease management opportunities, and new treatment options. Sanofi Genzyme, the specialty care global business unit of Sanofi, has been a pioneer in rare diseases with a focus on lysosomal diseases, a group of extremely rare genetic diseases, for more than three decades.

New research on the company's approved treatments for a number of lysosomal diseases, along with ongoing clinical studies of treatments in development for a broader range of rare diseases, will be presented at an upcoming key scientific meeting, the 15th Annual WORLD Symposium™ 2019, being held February 4 – 8, 2019, in Orlando, FL.

"Expanding the depth and breadth of knowledge related to rare genetic diseases, particularly lysosomal diseases, are focus areas for Sanofi," said Sébastien Martel, Global Head of Rare Diseases, Sanofi Genzyme. "We are committed to bringing new therapeutic options to patients living with rare genetic diseases and look forward to sharing our progress at WORLD Symposium this year."

"We made significant progress this year advancing innovative therapies in clinical development for several rare diseases," said Gianluca Pirozzi, Head of Development for Rare Disease and Translational Gene Therapy, Sanofi. "We are on track with the enrollment of our clinical studies and we are excited to share our results."

Olipudase Alfa Development Program

Data presented at WORLD Symposium include the latest research regarding olipudase alfa, an investigational enzyme replacement therapy being studied for patients with non-neurological manifestations of acid sphingomyelinase deficiency (ASMD), also known as Niemann-Pick disease type B and A/B. Both the Phase 2/3 clinical trial in adults (ASCEND) and the Phase 1/2 clinical trial in children (ASCEND-Peds) have successfully completed enrollment for the targeted number of patients for each of these trials.

Olipudase Alfa Poster at WORLD Symposium:

- Long-term efficacy of olipudase alfa in adults with acid sphingomyelinase deficiency (ASMD): further clearance of hepatic sphingomyelin is associated with additional improvements in pro- and anti-atherogenic lipid profiles after 3.5 years of treatment (P351; Poster Session II; February 6; 4:30 – 6:30 p.m. ET)

Venglustat Development Program

Venglustat is an investigational oral therapy in development for multiple rare disorders associated with mutations in the glycosphingolipid metabolic pathway including Gaucher disease type 3, GBA-related Parkinson's disease, Fabry disease and autosomal dominant polycystic kidney disease (ADPKD). Preliminary data from the Phase 2 clinical trial in Gaucher disease type 3 (LEAP) will be presented at WORLD Symposium along with data from the Phase 2 clinical trial in Parkinson's disease patients with a GBA mutation (MOVES-PD):

- Venglustat in adult Gaucher disease type 3: Preliminary safety, pharmacology, and exploratory efficacy from a phase 2 trial in combination with imiglucerase (LEAP) (P320; Oral Presentation; February 7; 8:00 a.m. ET)
- Safety, tolerability and pharmacokinetics of oral venglustat in Parkinson's disease patients with a GBA mutation (P282; Poster Session II; February 6; 4:30 - 6:30 p.m. ET)
Avalglucosidase Alfa Development Program

Avalglucosidase alfa is an investigational enzyme replacement therapy that has been designed for selective receptor targeting and increased enzyme uptake in muscles, with the aim of enhancing glycogen clearance in patients with both late-onset Pompe disease and infantile-onset Pompe disease. Data from both the late-onset and infantile-onset studies will be shared at WORLD Symposium:

- NEOI and NEO-EXT studies: Long-term safety of repeat avalglucosidase alfa dosing for 4.5 years in late-onset Pompe disease patients (P278; Oral Presentation; February 7; 9:30 a.m. ET)
- Mini-COMET study: Safety data and immunogenicity for repeat avalglucosidase alfa dosing in patients with infantile-onset Pompe disease (IOPD) who were previously treated with alglucosidase alfa and demonstrated clinical decline (LB-15; Poster Session II; February 6; 4:30 – 6:30 p.m. ET)

Olipudase alfa, venglustat and avalglucosidase alfa are investigational agents and have not been approved by the US Food and Drug Administration (FDA) or any other regulatory agency worldwide for the uses under investigation.

Sanofi Genzyme Rare Disease Registries

A disease registry is an observational database where clinical information is collected per routine clinical practice and voluntarily reported on patients with a specific condition. This information can be retrospectively analyzed and the results can help healthcare professionals better understand the disease and its management. The Sanofi Genzyme Rare Disease Registries program started in 1991 for patients with Gaucher disease and since that time additional registries have been added for Fabry, MPS I, and Pompe diseases. Multiple analyses of registry data will be presented at WORLD Symposium:

- Renal and cardiac outcomes in female patients with Fabry disease treated with agalsidase beta: A Fabry Registry analysis of pre- versus post-treatment comparison (P372; Poster Session II; February 6; 4:30 – 6:30 p.m. ET)
- Renal and cardiac outcomes of young male patients with Fabry disease initiated on agalsidase beta treatment before age 30: A Fabry Registry analysis (P161; Poster Session I; February 5; 4:30 – 6:30 p.m. ET)
- Significant abdominal and acute pain improvements in young patients with Fabry disease initiated on agalsidase beta treatment before age 30: A Fabry Registry analysis (P162; Poster Session I; February 5; 4:30 – 6:30 p.m. ET)
- The MPS I Registry - 15 years of service to the community (P125; Poster Session I; February 5; 4:30 – 6:30 p.m. ET)
- Head circumference in individuals with MPS I compared to CDC standard charts (P34; Poster Session I; February 5; 4:30 – 6:30 p.m. ET)
- Impact of Time from Diagnosis to Treatment on Lung Function among Patients with Late-Onset Pompe Disease: Data from the Pompe Registry (P344; Poster Session II; February 6; 4:30 – 6:30 p.m. ET)
- Two years of efficacy of oral eliglustat in treatment-naïve and switch patients enrolled in the International Collaborative Gaucher Group Gaucher Registry (P236; Poster Session II; February 6; 4:30 – 6:30 p.m. ET)
- A composite fracture risk score for assessing adult fracture risk in imiglucerase-treated type 1 Gaucher disease patients using data from the International Collaborative Gaucher Group (ICGG) Gaucher Registry Pregnancy Sub-Registry maintained by Sanofi Genzyme (P295; Poster Session I; February 5; 4:30 – 6:30 p.m. ET)

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CERDELGA® (eliglustat)

Indication

CERDELGA is a prescription medicine used for the long-term treatment of Gaucher disease type 1 (GD1) in adults who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test. Your doctor will perform a test to make sure that CERDELGA is right for you.

Limitations of Use:

- CYP2D6 ultra-rapid metabolizers may not achieve adequate concentrations of CERDELGA to achieve a therapeutic effect.
- A specific dose cannot be recommended for CYP2D6 indeterminate metabolizers.

Important Safety Information

Certain patients should not use CERDELGA based on their CYP2D6 metabolizer status due to an increased risk of side effects, including heart problems. Do not use CERDELGA if you are:

- An Extensive Metabolizer (EM) taking a medicine that is a strong or moderate CYP2D6 inhibitor along with another medicine that is a strong or moderate CYP3A inhibitor, an EM with moderate or severe liver problems, or an EM with mild liver problems and taking a medicine that is a strong or moderate CYP2D6 inhibitor.
- An Intermediate Metabolizer (IM) taking a medicine that is a strong or moderate CYP2D6 inhibitor along with another medicine that is a strong or moderate CYP3A inhibitor, an IM taking a medicine that is a strong CYP3A inhibitor, or an IM with any degree of liver problems.
- A Poor Metabolizer (PM) taking a medicine that is a strong CYP3A inhibitor, or a PM with any degree of liver problems.

Your doctor will perform a test to help determine if CERDELGA is right for you.

CERDELGA can affect the way other medicines work and other medicines can affect how CERDELGA works. Using CERDELGA with other medicines or herbal supplements may cause an increased risk of side effects, including changes in electrical activity of your heart (ECG changes) and irregular heart beat (arrhythmias). Especially tell your doctor if you take St. John's Wort, or medicines for fungal infections, tuberculosis, seizures, heart conditions, high blood pressure, or depression or other mental health problems. Your doctor may need to prescribe a different medicine, change your dose of other medicines, or change your dose of CERDELGA. Tell your doctor about all of the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements before you start taking them.

Before taking CERDELGA, tell your doctor about all of your medical conditions, including heart problems (including a condition called long QT syndrome), a history of heart attack, kidney or liver problems. If you are pregnant or plan to become pregnant or breastfeed, talk to your doctor.
It is not known if CERDELGA will harm your unborn baby. Talk to your doctor if you are breastfeeding or planning to breastfeed. It is not known if CERDELGA passes into your breast milk. You and your doctor will decide if you should take CERDELGA or breastfeed. You should not do both.

CERDELGA, used with certain other medicines, may cause changes in the electrical activity of your heart (ECG changes) and irregular heart beat (arrhythmias). Tell your doctor if you have new symptoms such as palpitations, fainting, or dizziness.

The most common side effects (≥10%) of CERDELGA include: tiredness, headache, nausea, diarrhea, back pain, pain in extremities, and upper abdominal pain. Call your doctor for medical advice about adverse effects.

Tell your doctor if you have any side effect that bothers you or that does not go away. These are not all the possible side effects of CERDELGA. Call your doctor for medical advice about side effects.

It is not known if CERDELGA is safe and effective in children.

Please see the full Prescribing Information, including the Patient Medication Guide, for CERDELGA.

Cerezyme® (imiglucerase for injection) Indication & Usage

Cerezyme® (imiglucerase for injection) is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease that results in one or more of the following conditions:

1. anemia (low red blood cell count)
2. thrombocytopenia (low blood platelet count)
3. bone disease
4. hepatomegaly or splenomegaly (enlarged liver or spleen)

Important Safety Information

Approximately 15% of patients have developed immune responses (antibodies) to Cerezyme during the first year of therapy. These patients have a higher risk of an allergic reaction (hypersensitivity). Your doctor may periodically test for the presence of antibodies. Serious allergic reactions (anaphylaxis) have been reported in less than 1% of patients. Symptoms suggestive of allergic reaction happened in approximately 7% of patients, and include itching, flushing, hives, swelling, chest discomfort, shortness of breath, coughing, cyanosis (a bluish discoloration of the skin due to diminished oxygen), and low blood pressure. If you have had an allergic reaction to Cerezyme, you and your doctor should use caution if you continue to receive treatment with Cerezyme.

High blood pressure in the arteries of the lungs (pulmonary hypertension) and pneumonia have been observed in less than 1% of patients during treatment with Cerezyme. These are also known complications of Gaucher disease regardless of treatment. If you experience symptoms such as shortness of breath or chest pain, with or without fever, contact your doctor.

Approximately 14% of patients have experienced side effects related to treatment with Cerezyme. Some of these reactions occur at the site of injection such as discomfort, itching, burning, swelling or uninfected abscess. Other side effects, each of which was reported by less than 2% of patients, include nausea, abdominal pain, vomiting, diarrhea, rash, fatigue, headache, fever, dizziness, chills, backache, and rapid heart rate. Temporary swelling in the legs has also been observed with drugs like Cerezyme.

Please see Full Prescribing Information (PDF).

Fabrazyme® (agalsidase beta)

Indication and Usage

Fabrazyme® (agalsidase beta) is used to treat patients with Fabry disease. Fabrazyme lowers the amount of a substance called globotriaosylceramide (GL-3), which builds up in cells lining the blood vessels of the kidney and certain other cells.

The lowering of GL-3 suggests that Fabrazyme may improve how Fabry disease affects your body; however a relationship of lower GL-3 to specific signs and symptoms of Fabry disease has not been proven.

Important Safety Information

Fabrazyme can cause serious side effects, including:

**Severe Allergic Reactions (anaphylaxis):** Life-threatening severe allergic (anaphylactic) reactions have been seen in patients during Fabrazyme infusions. Approximately 1% of patients who have received Fabrazyme either during a clinical study or after Fabrazyme was approved have experienced anaphylactic or severe allergic reactions during their infusion.

- These reactions have included: localized swelling of the face, mouth and throat, narrowing of breathing airways, low blood pressure, hives, difficulty swallowing, rash, trouble breathing, flushing, chest discomfort, itching and nasal congestion.
- People who have experienced these reactions have required treatment including heart/lung resuscitation, oxygen, fluids given through the vein, hospitalization, and have needed treatment with inhaled drugs called beta-adrenergic agonists to help open the breathing airways, antihistamines, epinephrine (also known as adrenalin), and a medication given through the vein called a corticosteroid (or steroid) which helps to decrease the body's allergic reaction by decreasing inflammation. Because of the possibility that severe allergic reactions may occur, appropriate medical support should be available during your Fabrazyme infusion.

**Infusion-Associated Reactions:** In clinical studies with Fabrazyme, 59% of patients experienced infusion-associated reactions during Fabrazyme administration, some of which were severe.

- For patients who have had reactions to their infusions, it is recommended that they be given anti-fever and antihistamine medications right before their next infusions. Infusion-associated reactions have happened in some patients even after taking these medications before their infusions.
- If an infusion-associated reaction occurs, slowing the infusion rate, stopping the infusion for a short time and/or giving more anti-fever and antihistamine medications and or steroids may improve the symptoms.
- If severe infusion-associated reactions happen, your healthcare professional should consider stopping the Fabrazyme infusion right away and should provide medical care for your condition. Severe reactions are generally managed by giving antihistamine medications, corticosteroids, fluids through the vein, and/or oxygen when needed. Because severe infusion-associated reactions may happen, medical treatment should be readily available during your Fabrazyme infusion.

**Pre-existing Heart Problems:** People with advanced Fabry disease may have heart problems, which may put them at a higher risk for severe complications from infusion-associated reactions. These patients should be watched closely during their infusion if the decision is made to give them Fabrazyme.
Immune Response and Continued Treatment After Allergic Reaction: In the clinical studies, a few patients developed IgE antibodies or a reaction to an allergy skin test specific to Fabrazyme. IgE antibodies are usually produced by the body's immune system during an allergic reaction. Your doctor should consider testing for IgE antibodies if you experience suspected allergic reactions. Providing Fabrazyme to patients who have experienced severe or serious allergic reactions to Fabrazyme should only be done after carefully considering the risks and benefits of continuing the treatment, and only under the direct supervision of a qualified healthcare professional and with appropriate medical support readily available.

**Common and Other Possible Side Effects:**

- Common side effects reported in 20% or more of Fabrazyme treated patients in clinical studies compared to placebo were upper respiratory tract infection, headache, cough, burning and/or tingling sensation, fatigue, dizziness, swelling in the legs, and rash.
- Serious and/or frequently occurring side effects (occurring in 5% or more of the patients) thought to be related to Fabrazyme in placebo-controlled and open-label clinical studies have included: chills, fever, feeling hot or cold, trouble breathing, nausea, flushing of the skin, headache, vomiting, burning and/or tingling sensation, fatigue, itching, pain in the hands and feet, high blood pressure, chest pain, throat tightness, abdominal pain, dizziness, rapid heart rate, nasal congestion, diarrhea, swelling in the legs, muscle pain, back pain, paleness of the skin, slow heart rate, hives, low blood pressure, face swelling, rash and sleepiness.
- Other serious side effects that were seen in the clinical studies included stroke, pain, lack of muscle coordination, slow or irregular heartbeat, stopping of the heartbeat, decreased blood pumped by the heart, dizziness, and kidney problems resulting in too much protein leaving the body in the urine (nephrotic syndrome). These side effects also occur as part of Fabry disease.
- Since Fabrazyme has been approved, there have been side effects that resulted in death that may or may not be related to the use of Fabrazyme. These included: the heart and/or lungs stop working (known as cardiorespiratory arrest, respiratory failure, and/or cardiac failure), life-threatening infection in the blood stream (known as sepsis), stroke, heart attack, kidney failure, and pneumonia. Some of these side effects were reported in Fabry disease patients with significant underlying disease.

The safety and effectiveness of Fabrazyme in patients younger than 8 years of age have not been studied.

Please see full prescribing information for Fabrazyme.

**Language:**

English