

Sanofi Genzyme Commitment to Patients with Rare Diseases Highlighted at WORLDSymposium 2017

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Support for 31 data presentations helps advance understanding of lysosomal storage disorders

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sanofi Genzyme, the specialty care global business unit of Sanofi, today announced that new investigational data on its marketed treatments for Fabry disease, Gaucher disease, MPS I and Pompe disease along with data on its rare diseases drug development pipeline will be presented at the 13th Annual WORLDSymposium. The symposium, which will be held in San Diego, CA, February 13 – 17, is focused on basic science, translational research and clinical trials for lysosomal storage disorders. Sanofi Genzyme is supporting 31 presentations including nine platform presentations.

“Sanofi Genzyme has a long-standing commitment to patients with lysosomal storage disorders, complex rare diseases that can be progressive, severely debilitating and life-threatening,” said North America Head of Rare Diseases, Yann Mazabraud. *“We are dedicated to empowering the lives of these patients by supporting research and development that advances the understanding of these disorders and supports the people we serve throughout the continuum of care.”*

Sanofi Genzyme-sponsored and grant-funded investigator study data presentations are as follows. Grant funded investigator studies are noted with an asterisk (*). All others are Sanofi Genzyme-sponsored presentations.

Acid Sphingomyelinase Deficiency (ASMD), historically referred to as Niemann-Pick disease types A and B (NPD A and B)

- Quantitative systems pharmacology model of acid sphingomyelinase deficiency and the enzyme replacement therapy olipudase alfa is an innovative tool for linking pathophysiology and pharmacology (Translational Research II, Platform Presentation; February 15; 2:00 p.m. PT)
- Olipudase alfa for the treatment of acid sphingomyelinase deficiency (ASMD): safety and efficacy in adults treated for 30 months (Clinical Trials I, Platform Presentation; February 16; 9:15 a.m. PT)
- Consensus recommendation on a diagnostic guideline for acid sphingomyelinase deficiency (P357; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)
- Autopsy pathology of infantile neurovisceral ASMD (Niemann-Pick disease type A): clinicopathologic correlations of a case report and review of the literature (P336; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)

Fabry Disease

- A randomized, phase 3B, open-label, parallel-group study of agalsidase beta in treatment-naive male pediatric patients with Fabry disease without severe symptoms (FIELD study): GL-3 clearance from kidney cells (Clinical Trials II, Platform Presentation; February 16; 2:30 p.m. PT)
- A Fabry genotype-phenotype working group initiative: classifying GLA mutations for male patients in the Fabry Registry (P100; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- The phenotypic characteristics of the p.N215S Fabry disease genotype in male and female patients: a multi-center Fabry Registry study (P101; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Burden of Fabry disease in young patients (≤ 30 years of age) who were initiated on enzyme replacement therapy with agalsidase beta: a Fabry Registry analysis (P142; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Sudden pronounced height increase for adolescent and young adult males with Fabry disease (P183; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Natural history data from 182 female patients with Fabry disease in Latin America: A Fabry Registry analysis of disease burden (P217; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)
- A randomized, phase 3B, open-label, parallel-group study of agalsidase beta in treatment-naive male pediatric patients with Fabry disease without severe symptoms (FIELD study): GL-3 clearance from superficial skin capillary endothelium (P281; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)

Gaucher Disease

- Long-term results of ENGAGE: a phase 3, randomized, double-blind, placebo-controlled, multi-center study investigating the efficacy and safety of eliglustat in adults with Gaucher disease type 1 (Clinical Trials I, Platform

Presentation; February 16, 8:15 a.m. PT)

- Maintenance of quality of life in adults with type 1 Gaucher disease previously stabilized on enzyme therapy who were switched to oral eliglustat: 4 year results of the ENCORE trial (Clinical Trials I, Platform Presentation; February 16, 8:30 a.m. PT)
- A conceptual framework of patient-reported outcomes for Gaucher disease type 3 (P130; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Two-year post marketing safety experience with oral eliglustat in adults with Gaucher disease type 1 (P146; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Effect of two different therapeutic interventions: SRT in comparison to ERT on immune aspects and bone involvement in Gaucher disease* (P197; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Transformation in pre-treatment presentations of Gaucher disease during the first two decades of imiglucerase enzyme replacement therapy: a report from the International Collaborative Gaucher Group Gaucher Registry (P359; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)

MPS I

- Triple therapy for MPS IH: intrathecal iduronidase, IV-ERT, and hematopoietic cell transplant* (Clinical Trials I, Platform Presentation; February 16, 10:45 a.m. PT)
- A randomized open-label clinical trial of intrathecal recombinant human alpha-L-iduronidase for cognitive decline in mucopolysaccharidosis type I* (Clinical Trials I, Platform Presentation; February 16, 11:00 a.m. PT)
- MPS I and carpal tunnel syndrome: analysis using the MPS I registry (P124; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Post-transplant intravenous laronidase augmentation for Hurler syndrome: anti-drug antibody, biochemical and donor hematopoietic graft response* (P233; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)

Pompe Disease

- Beneficial effects of carvedilol with enzyme replacement therapy in Pompe disease* (Basic Science II, Platform Presentation; February 14; 3:45 p.m. PT)
- Long-term efficacy of alglucosidase alfa in late-onset Pompe disease (Clinical Trials II, Platform Presentation; February 16; 3:15 p.m. PT)
- PRO instrument development for late-onset Pompe disease (P131; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Modeling of changes in forced vital capacity and late-onset Pompe disease related outcomes (P132; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- The effects of sub-maximal aerobic exercise in adults with late-onset Pompe disease (LOPD)* (P163; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Sequence variants and genotypes among 898 patients with Pompe disease: data from the Pompe registry (P170; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)
- Impact of earlier treatment on respiratory function in patients with late-onset Pompe disease: data from the Pompe Registry (P326; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)
- Quantitative muscle MRI is useful to follow-up late onset Pompe patients treated with enzyme replacement therapy* (LB-16; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)
- Enzymatic assays for alfa-glucosidase in patients with Pompe disease, results from ten years of high risk screening in Colombia (LB-43; Poster Session II; February 15; 4:30 – 6:30 p.m. PT)

Cross-LSD

- Evolving observational registries: an evidence-based approach to understanding data availability in the “Sanofi Genzyme Rare Disease Registries” (P77; Poster Session I; February 14; 4:30 – 6:30 p.m. PT)

About Lysosomal Storage Disorders

Lysosomal storage disorders (LSDs) are a group of more than 40 diseases. Each is caused by a genetic mutation that results in the deficiency or malfunction of a particular enzyme needed to remove waste material from cells. These waste molecules then accumulate, or build up, in cell lysosomes (smaller compartments within cells), disrupting cell function and causing a variety of symptoms. LSDs can be progressive, life-threatening and severely debilitating. Because these disorders are extremely rare, it can be difficult to find information about them. In the case of the most common of these disorders, Gaucher disease, it is estimated that only 10,000 people have been diagnosed worldwide. It is thought that many more people are affected by rare diseases than have been diagnosed. This is why access to information about LSDs is so important.

About FABRAZYME® (agalsidase beta)

Fabrazyme (agalsidase beta) is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types. The reduction of GL-3 inclusions suggests that Fabrazyme may ameliorate disease expression; however, the relationship of GL-3 inclusion reduction to specific clinical manifestations of Fabry disease has not been established.

Important Safety Information About FABRAZYME® (agalsidase beta)

Life-threatening anaphylactic and severe allergic reactions have been observed in patients during Fabrazyme infusions. In clinical trials and postmarketing safety experience, approximately 1% of patients developed anaphylactic or severe allergic reactions during Fabrazyme infusions. Reactions have included localized angioedema (including swelling of the face, mouth, and throat), bronchospasm, hypotension, generalized urticaria, dysphagia, rash, dyspnea, flushing, chest discomfort, pruritus, and nasal congestion. Interventions have included cardiopulmonary resuscitation, oxygen supplementation, IV fluids,

hospitalization, and treatment with inhaled beta-adrenergic agonists, antihistamines, epinephrine, and IV corticosteroids. If severe allergic or anaphylactic reactions occur, immediately discontinue administration of Fabrazyme and provide necessary emergency treatment. Because of the potential for severe allergic reactions, appropriate medical support measures should be readily available when Fabrazyme is administered.

In patients experiencing infusion reactions, pretreatment with an antipyretic and antihistamine is recommended. Infusion reactions occurred in some patients after receiving pretreatment with antipyretics, antihistamines, and oral steroids. If an infusion reaction occurs, decreasing the infusion rate, temporarily stopping the infusion, and/or administering additional antipyretics, antihistamines, and/or steroids may ameliorate the symptoms. If severe infusion reactions occur, immediate discontinuation of the administration of Fabrazyme should be considered, and appropriate medical treatment should be initiated. Severe reactions are generally managed with administration of antihistamines, corticosteroids, intravenous fluids, and/or oxygen when clinically indicated. Because of the potential for severe infusion reactions, appropriate medical support measures should be readily available when Fabrazyme is administered.

Re-administration of Fabrazyme to patients who have previously experienced severe or serious allergic reactions to Fabrazyme should be done only after careful consideration of the risks and benefits of continued treatment, and only under the direct supervision of qualified personnel and with appropriate medical support measures readily available.

The most common adverse reactions reported are infusion reactions, some of which were severe. Infusion reactions occurred in approximately 50-55% of patients during Fabrazyme administration in clinical trials. Serious and/or frequently occurring ($\geq 5\%$ incidence) related adverse reactions consisted of one or more of the following: chills, fever, feeling hot or cold, dyspnea, nausea, flushing, headache, vomiting, paresthesia, fatigue, pruritus, pain in extremity, hypertension, chest pain, throat tightness, abdominal pain, dizziness, tachycardia, nasal congestion, diarrhea, edema peripheral, myalgia, back pain, pallor, bradycardia, urticaria, hypotension, face edema, rash, and somnolence.

Patients with advanced Fabry disease may have compromised cardiac function, which may predispose them to a higher risk of severe complications from infusion reactions. Patients with compromised cardiac function should be monitored closely if the decision is made to administer Fabrazyme.

Other serious adverse events reported in clinical studies included stroke, pain, ataxia, bradycardia, cardiac arrhythmia, cardiac arrest, decreased cardiac output, vertigo, hypoacusia, and nephrotic syndrome. These adverse events also occur as manifestations of Fabry disease; an alteration in frequency or severity cannot be determined from the small numbers of patients studied.

Severe and serious infusion related reactions have been reported in postmarketing experience, some of which were life threatening including anaphylactic shock. In addition to the above adverse reactions, the following have been reported during post marketing use of Fabrazyme: arthralgia, asthenia, erythema, hyperhidrosis, infusion site reaction, lacrimation increased, leukocytoclastic vasculitis, lymphadenopathy, hypoesthesia, oral hypoesthesia, palpitations, rhinorrhea, oxygen saturation decreased and hypoxia.

Adverse reactions (regardless of relationship) resulting in death reported in the postmarketing setting with Fabrazyme treatment included cardiorespiratory arrest, respiratory failure, cardiac failure, sepsis, cerebrovascular accident, myocardial infarction, renal failure, and pneumonia. Some of these reactions were reported in Fabry disease patients with significant underlying disease.

The safety and efficacy in patients younger than 8 years of age have not been evaluated.

Most patients who develop IgG antibodies do so within the first three months of exposure. IgG seroconversion in pediatric patients was associated with prolonged half-life of Fabrazyme, a phenomenon rarely observed in adult patients.

In clinical trials, a few patients developed IgE or skin test reactivity specific to Fabrazyme. Physicians should consider testing for IgE in patients who experienced suspected allergic reactions and consider the risks and benefits of continued treatment in patients with anti-Fabrazyme IgE antibodies.

Please [click here](#) for full U.S. Prescribing Information for Fabrazyme.

About CERZYME[®] (imiglucerase for injection)

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: anemia, thrombocytopenia, bone disease, and hepatomegaly or splenomegaly.

Important Safety Information About CERZYME[®] (imiglucerase for injection)

Approximately 15% of patients have developed IgG antibodies to Cerezyme during the first year of therapy. Approximately 46% of patients with detectable IgG antibodies experienced symptoms of hypersensitivity, and these patients have a higher risk of hypersensitivity. It is suggested that patients be monitored periodically for IgG antibody formation during the first year of treatment.

Hypersensitivity has also been observed in patients without detectable IgG antibodies. Symptoms suggestive of hypersensitivity have been noted in approximately 6.6% of all patients, and anaphylactoid reactions in less than 1%. Treatment with Cerezyme should be approached with caution in patients who have exhibited hypersensitivity symptoms such as pruritus, flushing, urticarial, angioedema, chest discomfort, dyspnea, coughing, cyanosis, and hypotension. Pretreatment with antihistamines and/or corticosteroids and a reduced rate of infusion may allow continued treatment in most patients.

In less than 1% of patients, pulmonary hypertension and pneumonia have been observed during treatment with Cerezyme. These are known complications of Gaucher disease regardless of treatment. Patients with respiratory symptoms in the absence of fever should be evaluated for the presence of pulmonary hypertension.

Approximately 13.8% of patients have experienced adverse events related to treatment with Cerezyme. Some of these are injection site reactions such as discomfort, pruritus, burning, swelling or sterile abscess at the site at the site of venipuncture. Additional adverse reactions that have been reported include nausea, abdominal pain, vomiting, diarrhea, rash, fatigue, headache, fever, dizziness, chills, backache, and tachycardia. Transient peripheral edema has also been reported for

this therapeutic class of drug.

Please [click here](#) for full U.S. Prescribing Information for Cerezyme.

About CERDELGA® (eliglustat)

CERDELGA (eliglustat) capsules are indicated for the long-term treatment of adults with Gaucher disease type 1 (GD1) who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test. Patients who are CYP2D6 ultra-rapid metabolizers (URMs) may not achieve adequate concentrations of CERDELGA to achieve a therapeutic effect. A specific dose cannot be recommended for those patients whose CYP2D6 genotype cannot be determined (indeterminate metabolizers)

Important Safety Information for CERDELGA® (eliglustat)

CERDELGA is contraindicated in the following patients due to the risk of significantly increased eliglustat plasma concentrations, which may result in prolongation of the PR, QTc, and/or QRS cardiac intervals that could result in cardiac arrhythmias: EMs or IMs taking a strong or moderate CYP2D6 inhibitor concomitantly with a strong or moderate CYP3A inhibitor and IMs or PMs taking a strong CYP3A inhibitor.

Drugs that inhibit CYP2D6 and CYP3A may significantly increase the exposure to eliglustat; CERDELGA dose adjustment may be needed, depending on metabolizer status. See section 7 of the full Prescribing Information for more details and other potentially significant drug interactions.

Because CERDELGA is predicted to cause increases in ECG intervals at substantially elevated plasma concentrations, use is not recommended in patients with pre-existing cardiac disease, long QT syndrome, or in combination with Class IA and Class III antiarrhythmic medications.

The most common adverse reactions ($\geq 10\%$) for CERDELGA are: fatigue, headache, nausea, diarrhea, back pain, pain in extremities, and upper abdominal pain.

Only administer CERDELGA during pregnancy if the potential benefit justifies the potential risk; based on animal data, CERDELGA may cause fetal harm. Discontinue drug or nursing based on importance of drug to mother. CERDELGA is not recommended in patients with moderate to severe renal impairment, end-stage renal disease or in patients with hepatic impairment or cirrhosis.

To report SUSPECTED ADVERSE REACTIONS, contact Sanofi Genzyme Corporation at (1-800-745-4447) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see [full Prescribing Information](#), including patient Medication Guide, for additional important safety information.

About ALDURAZYME® (laronidase)

ALDURAZYME® (laronidase) is indicated for patients with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form who have moderate to severe symptoms. The risks and benefits of treating mildly affected patients with the Scheie form have not been established.

ALDURAZYME has been shown to improve pulmonary function and walking capacity. ALDURAZYME has not been evaluated for effects on the central nervous system manifestations of the disorder.

Important Safety Information for ALDURAZYME® (laronidase)

WARNING: Risk of anaphylaxis. Life-threatening anaphylactic reactions have been observed in some patients during ALDURAZYME® infusions. Therefore, appropriate medical support should be readily available when ALDURAZYME is administered. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions, and require additional monitoring.

Anaphylaxis and severe allergic reactions have been observed in patients during or up to 3 hours after ALDURAZYME infusions. Some of these reactions were life-threatening and included respiratory failure, respiratory distress, stridor, tachypnea, bronchospasm, obstructive airways disorder, hypoxia, hypotension, bradycardia, and urticaria. If anaphylactic or other severe allergic reactions occur, immediately discontinue the infusion of ALDURAZYME and initiate appropriate treatment. Caution should be exercised if epinephrine is being considered for use in patients with MPS I due to the increased prevalence of coronary artery disease in these patients. Interventions have included resuscitation, mechanical ventilatory support, emergency tracheotomy, hospitalization, and treatment with inhaled beta-adrenergic agonists, epinephrine, and IV corticosteroids.

In clinical studies and postmarketing safety experience with ALDURAZYME, approximately 1% of patients experienced severe or serious allergic reactions. In patients with MPS I, pre-existing upper airway obstruction may have contributed to the severity of some reactions. Due to the potential for severe allergic reactions, appropriate medical support should be readily available when ALDURAZYME is administered. Because of the potential for recurrent reactions, some patients who experience initial severe reactions may require prolonged observation.

The risks and benefits of re-administering ALDURAZYME following an anaphylactic or severe allergic reaction should be considered. Extreme care should be exercised, with appropriate resuscitation measures available, if the decision is made to re-administer the product.

Patients with an acute febrile or respiratory illness at the time of ALDURAZYME infusion may be at greater risk for infusion reactions. Careful consideration should be given to the patient's clinical status prior to administration of ALDURAZYME and consider delaying ALDURAZYME infusion.

Sleep apnea is common in MPS I patients. Evaluation of airway patency should be considered prior to initiation of treatment with ALDURAZYME. Patients using supplemental oxygen or continuous positive airway pressure (CPAP) during sleep should have these treatments readily available during infusion in the event of an infusion reaction or extreme drowsiness/sleep induced by antihistamine use.

Caution should be exercised when administering ALDURAZYME to patients susceptible to fluid overload or patients with an acute underlying respiratory illness or compromised cardiac and/or respiratory function for whom fluid restriction is indicated. These patients may be at risk of serious exacerbation of their cardiac or respiratory status during infusions. Appropriate medical support and monitoring measures should be readily available during ALDURAZYME infusion, and some patients may require prolonged observation times that should be based on the individual needs of the patient.

Because of the potential for infusion reactions, patients should receive antipyretics and/or antihistamines prior to infusion. If an infusion-related reaction occurs, regardless of pre-treatment, decreasing the infusion rate, temporarily stopping the infusion, or administering additional antipyretics and/or antihistamines may ameliorate the symptoms.

The most serious adverse reactions reported with ALDURAZYME treatment during clinical trials were anaphylactic and allergic reactions.

In a 26-week, placebo-controlled clinical trial in patients 6 years and older, the most commonly reported infusion reactions regardless of treatment group were flushing, pyrexia, headache, and rash. Flushing occurred in 5 patients (23%) receiving ALDURAZYME; the other reactions were less frequent. Less common infusion reactions included angioedema (including face edema), hypotension, paresthesia, feeling hot, hyperhidrosis, tachycardia, vomiting, back pain, and cough. Other reported adverse reactions included bronchospasm, dyspnea, urticaria, and pruritus. In the open-label, uncontrolled extension phase of this clinical trial, the infusion reactions were similar, but also included abdominal pain or discomfort and injection site reaction. Less commonly reported infusion reactions included nausea, diarrhea, feeling hot or cold, vomiting, pruritus, arthralgia and urticaria. Additional common adverse reactions included, back pain and musculoskeletal pain.

In an open-label, uncontrolled clinical trial in patients 6 years and younger who received ALDURAZYME treatment for up to 52 weeks, the most commonly reported serious adverse events (regardless of relationship) in patients 6 years and younger, were otitis media (20%), and central venous catheterization required for ALDURAZYME infusion (15%). The most commonly reported adverse reactions in patients 6 years and younger were infusion reactions reported in 35% (7 of 20) of patients and included pyrexia (30%), chills (20%), blood pressure increased (10%), tachycardia (10%), and oxygen saturation decreased (10%). Other commonly reported infusion reactions occurring in $\geq 5\%$ of patients were pallor, tremor, respiratory distress, wheezing, crepitations (pulmonary), pruritus, and rash.

In postmarketing experience with ALDURAZYME, severe and serious infusion reactions have been reported, some of which were life-threatening, including anaphylactic shock. Adverse reactions resulting in death reported in the postmarketing setting with ALDURAZYME treatment included cardio-respiratory arrest, respiratory failure, cardiac failure, and pneumonia. These events have been reported in MPS I patients with significant underlying disease. Additional common adverse reactions included erythema and cyanosis. There have been a small number of reports of extravasation in patients treated with ALDURAZYME. There have been no reports of tissue necrosis associated with extravasation. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to drug exposure.

In clinical trials, 99 of 102 patients (97%) treated with ALDURAZYME were positive for IgG antibodies to ALDURAZYME. In the 2 trials of patients 6 years and older, 9 patients who experienced severe infusion reactions were tested for ALDURAZYME-specific IgE antibodies and complement activation. One of the nine patients had an anaphylactic reaction consisting of urticaria and airway obstruction and tested positive for both ALDURAZYME-specific IgE binding antibodies and complement activation. In the postmarketing setting, approximately 1% of patients experienced severe or serious infusion-allergic reactions and tested positive for IgE. Of these IgE-positive patients, some have discontinued treatment, but some have been successfully re-challenged. The clinical significance of antibodies to ALDURAZYME, including the potential for product neutralization, is not known.

Please [click here](#) for full U.S. Prescribing Information for Aldurazyme including Boxed **WARNING**.

About LUMIZYME® (alglucosidase alfa)

INDICATION

LUMIZYME® (alglucosidase alfa) is a hydrolytic lysosomal glycogen-specific enzyme indicated for patients with Pompe disease (GAA deficiency).

IMPORTANT SAFETY INFORMATION

WARNING: RISK OF ANAPHYLAXIS, HYPERSENSITIVITY AND IMMUNEMEDIATED REACTIONS, and RISK OF CARDIORESPIRATORY FAILURE

- **Life-threatening anaphylactic reactions and severe hypersensitivity reactions, presenting as respiratory distress, hypoxia, apnea, dyspnea, bradycardia, tachycardia, bronchospasm, throat tightness, hypotension, angioedema (including tongue or lip swelling, periorbital edema, and face edema), and urticaria, have occurred in some patients during and after alglucosidase alfa infusions. Immune-mediated reactions presenting as proteinuria, nephrotic syndrome, and necrotizing skin lesions have occurred in some patients following alglucosidase alfa treatment. Closely observe patients during and after alglucosidase alfa administration and be prepared to manage anaphylaxis and hypersensitivity reactions. Inform patients of the signs and symptoms of anaphylaxis, hypersensitivity reactions, and immune-mediated-reactions and have them seek immediate medical care should signs and symptoms occur.**
- **Infantile-onset Pompe disease patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to fluid overload, and require additional monitoring.**

WARNINGS AND PRECAUTIONS

Anaphylaxis and Hypersensitivity Reactions: Life-threatening anaphylaxis and hypersensitivity reactions have been observed in some patients during and after treatment with alglucosidase alfa. If anaphylaxis or severe hypersensitivity reactions occur, immediately discontinue infusion and institute appropriate medical treatment. Appropriate medical support and monitoring measures should be available during infusion.

Immune-Mediated Reactions: Monitor patients for the development of systemic immunemediated reactions involving skin and other organs.

Risk of Acute Cardiorespiratory Failure: Patients with acute underlying respiratory illness and compromised cardiac and/or respiratory function may be at risk of acute cardiorespiratory failure. Caution should be exercised when administering alglucosidase alfa to patients susceptible to fluid volume overload. Appropriate medical support and monitoring measures should be available during infusion and some patients may require longer observation times.

Risk of Cardiac Arrhythmia and Sudden Cardiac Death during General Anesthesia for Central Venous Catheter Placement: Caution should be used when administering general anesthesia for the placement of a central venous catheter intended for alglucosidase alfa infusion.

Risk of Antibody Development: As with all therapeutic proteins, there is potential for immunogenicity. There is some evidence to suggest that some patients who develop high and sustained IgG antibody titers may experience reduced clinical efficacy. Patients should be monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter.

ADVERSE REACTIONS

The most frequently reported adverse reactions ($\geq 5\%$) in clinical trials were hypersensitivity reactions and included: anaphylaxis, rash, pyrexia, flushing/feeling hot, urticaria, headache, hyperhidrosis, nausea, cough, decreased oxygen saturation, tachycardia, tachypnea, chest discomfort, dizziness, muscle twitching, agitation, cyanosis, erythema, hypertension/increased blood pressure, pallor, rigors, tremor, vomiting, fatigue, and myalgia.

USE IN SPECIFIC POPULATIONS

Pregnancy: Based on animal data, alglucosidase alfa may cause fetal harm.

To report SUSPECTED ADVERSE REACTIONS, contact Sanofi Genzyme at 1-800-745-4447 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see the **Full Prescribing Information** for complete details, including **boxed WARNING**.

About Sanofi

Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi is organized into five global business units: Diabetes and Cardiovascular, General Medicines and Emerging Markets, Sanofi Genzyme, Sanofi Pasteur and Consumer Healthcare.

Sanofi Genzyme focuses on developing specialty treatments for debilitating diseases that are often difficult to diagnose and treat, providing hope to patients and their families.

Sanofi Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans" and similar expressions. Although Sanofi's management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, Sanofi's ability to benefit from external growth opportunities and/or obtain regulatory clearances, risks associated with intellectual property and any related pending or future litigation and the ultimate outcome of such litigation, trends in exchange rates and prevailing interest rates, volatile economic conditions, the impact of cost containment initiatives and subsequent changes thereto, the average number of shares outstanding as well as those discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under "Risk Factors" and "Cautionary Statement Regarding Forward-Looking Statements" in Sanofi's annual report on Form 20-F for the year ended December 31, 2015. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

□

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English

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