Keeping an eye on late-onset Pompe disease

NEXVIAZYME (avalglucosidase alfa-ngpt) is used for the treatment of patients 1 year of age and older with late-onset Pompe disease.

The answers you provide here are to help evaluate how you're doing and how to prepare for your next discussion with your healthcare provider.

How I'm feeling physically since my last checkup. (1 is same as last visit, and 10 is noticed a lot of change.)

1 2 3 4 5 6 7 8 9 10

Things I've noticed a difference in or have difficulty doing

BREATHING

- ◯ More breathless during and/or after exercise
- ◯ Waking up throughout the night feeling breathless
- ◯ Weak cough

Breathing issues can cause other symptoms as well. I've been experiencing more

- ◯ Morning headaches
- ◯ Daytime sleepiness

MOVING

- ◯ Harder to climb stairs
- ◯ More difficult to get up out of a chair
- ◯ Using a mobility aid or device while walking
- ◯ Walking slower or with more of a waddle
- ◯ Difficulty maintaining balance while walking or standing
- ◯ More trouble reaching over my head
- ◯ More difficulty doing everyday tasks, like washing or brushing my hair
- ◯ More difficulty beginning or continuing physical exercises

OTHER

- ◯ Difficulty chewing/swallowing
- ◯ Unexplained weight loss
- ◯ Tongue weakness
- ◯ Pain

Am I feeling more fatigued than usual? (1 is not more than usual, and 10 is feeling extreme fatigue.)

1 2 3 4 5 6 7 8 9 10

I feel my current management plan is still helping my late-onset Pompe disease.

◯ YES ◯ COULD BE BETTER ◯ NO ◯ UNSURE

IMPORTANT SAFETY INFORMATION

IMPORTANT SAFETY INFORMATION

WARNING: SEVERE HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, and RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

Hypersensitivity Reactions Including Anaphylaxis

If you are taking NEXVIAZYME, you should know that severe and potentially life-threatening allergic-type reactions known as anaphylaxis and severe hypersensitivity reactions may occur during and after NEXVIAZYME treatment. You should seek immediate medical care if signs and symptoms of anaphylaxis or hypersensitivity reactions occur. If such a reaction is severe enough, your doctor may decide to immediately discontinue the infusion and provide immediate medical care. Appropriate medical support measures may be administered during your infusion, and you may require close observation during and after NEXVIAZYME administration.

Please see additional Important Safety Information, including **Boxed WARNING**, and accompanying full Prescribing Information, on following pages.

Nexviazyme™ (avalglucosidase alfa-ngpt)

Keeping an eye on late-onset Pompe disease

Other things I'd like to discuss:

Together with your healthcare provider, you can discuss if NEXVIAZYME is a treatment option that is right for you.

NEXVIAZYME is the first new enzyme replacement therapy for late-onset Pompe disease in 15 years. So now, for the first time, you have a choice. And a new treatment to consider.

IMPORTANT SAFETY INFORMATION

IMPORTANT SAFETY INFORMATION CONT'D

Infusion-Associated Reactions (IARs)

If you are taking NEXVIAZYME, you should know that severe IARs may occur during and after NEXVIAZYME treatment. If severe IARs occur during your NEXVIAZYME infusion, your doctor may decide to immediately discontinue the infusion and provide appropriate medical care. If you have an acute underlying illness at the time of NEXVIAZYME infusion you may be at greater risk for IARs. If you have advanced Pompe disease you may have compromised heart and breathing function, which may put you at a higher risk of severe complications from IARs.

Please see additional Important Safety Information, including **Boxed WARNING**, and accompanying full Prescribing Information, on following pages.

Nexviazyme™ (avalglucosidase alfa-ngpt)

What is NEXVIAZYME?

NEXVIAZYME is the first new enzyme replacement therapy for late-onset Pompe Disease (LOPD) in 15 years. It's designed to bind to muscle cells with a high affinity (a strong attachment). When this happens, NEXVIAZYME enters cells, so it can help break down glycogen.

What does NEXVIAZYME do?

In a clinical study of 100 LOPD patients, NEXVIAZYME helped people who had not been on treatment before improve breathing and walking distance compared to when they started the study.

After 49 weeks on NEXVIAZYME, compared to when they began treatment:

People were able to improve their breathing capacity by an average of 2.9 percentage points in a breathing test.

- Those who took alglucosidase alfa improved their breathing by an average of 0.5 percentage points.
- This resulted in a measurable improvement of 2.4 percentage points for people treated with NEXVIAZYME compared with people taking alglucosidase alfa, although it was not statistically superior.

Those who took NEXVIAZYME improved their walking distance by an average of 106 feet during a 6-minute walk test (6MWT).

- People taking alglucosidase alfa improved their walking distance by an average of 7.2 feet.
- People taking NEXVIAZYME walked an average of 98 feet farther than those who were taking alglucosidase alfa. The study was not designed to test whether NEXVIAZYME was superior to alglucosidase alfa for this measure.

The tests were given at the beginning of the study and again at the end, after 49 weeks of treatment.

NEXVIAZYME and safety

In the clinical trial, serious adverse reactions were reported in 1 patient treated with NEXVIAZYME and in 3 patients treated with alglucosidase alfa.

The most common side effects reported by people receiving NEXVIAZYME were headache, fatigue, diarrhea, nausea, joint pain, dizziness, muscle pain, itching, vomiting, shortness of breath, rash, "pins-and-needles" sensation, and hives.

In addition, 13 (25%) of people receiving NEXVIAZYME experienced mild-to-moderate infusion-associated reactions. These reactions included headache, diarrhea, itching, hives, and rash. No one experienced a severe infusion-associated reaction.

These are not all the possible side effects. If you experience any side effects, it's important to tell your healthcare provider right away. You can also report side effects at 1-800-FDA-1088 or www.fda.gov/medwatch.

How does NEXVIAZYME work?

When you have LOPD, the body does not have enough of the enzyme that breaks down glycogen in your muscle cells, so the glycogen builds up and causes muscle damage.

NEXVIAZYME has something called "high binding affinity" for a receptor in your muscle cell called M6P receptor. That receptor is like a lock on a door. Residue called M6P is the key to open that lock. NEXVIAZYME has "keys" on its surface that fit in the "lock." In fact, NEXVIAZYME has 15 times the number of keys that alglucosidase alfa has. In a clinical study, NEXVIAZYME was not shown to be statistically superior to alglucosidase alfa.

How do I take NEXVIAZYME?

NEXVIAZYME is given every 2 weeks by intravenous (IV) infusion. The recommended dosage of NEXVIAZYME is either 20 mg or 40 mg for each kilogram of body weight—your healthcare provider will calculate the appropriate dosage for you. The infusion usually takes approximately 4-5 hours for those receiving 20 mg/kg and approximately 5-7 hours for those receiving 40 mg/kg. There could be additional time if you need any pretreatment. Also, infusion times may vary based on your response to therapy and comfort. Your healthcare provider will give you more details about what to expect during and after your infusion as well as how to prepare.

You'll most likely want to bring a book, work, and/or electronic devices to make the most of your time. If you're switching to NEXVIAZYME from alglucosidase alfa, your treatment schedule may stay the same.

For any other questions, please contact your Sanofi Genzyme CareConnectPSS® team 1-800-745-4447, option 3.

IMPORTANT SAFETY INFORMATION

IMPORTANT SAFETY INFORMATION CONT'D

Risk of Acute Cardiorespiratory Failure in Susceptible Patients

If you are likely to develop fluid volume overload, or have acute underlying breathing problems or compromised heart or breathing function that may require fluid restriction, there may be a risk of worsening of your heart or breathing status during NEXVIAZYME infusion. Your doctor may decide that close observation during NEXVIAZYME administration may be necessary.

Please see additional Important Safety Information, including Boxed WARNING, and accompanying full Prescribing Information, on following page.

Nexviazyme™
(avalglucosidase alfa-ngpt)

IMPORTANT SAFETY INFORMATION AND INDICATION

IMPORTANT SAFETY INFORMATION

WARNING: SEVERE HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, and RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

Hypersensitivity Reactions Including Anaphylaxis

If you are taking NEXVIAZYME, you should know that severe and potentially life-threatening allergic-type reactions known as anaphylaxis and severe hypersensitivity reactions may occur during and after NEXVIAZYME treatment. You should seek immediate medical care if signs and symptoms of anaphylaxis or hypersensitivity reactions occur. If such a reaction is severe enough, your doctor may decide to immediately discontinue the infusion and provide immediate medical care. Appropriate medical support measures may be administered during your infusion, and you may require close observation during and after NEXVIAZYME administration.

Infusion-Associated Reactions (IARs)

If you are taking NEXVIAZYME, you should know that severe IARs may occur during and after NEXVIAZYME treatment. If severe IARs occur during your NEXVIAZYME infusion, your doctor may decide to immediately discontinue the infusion and provide appropriate medical care. If you have an acute underlying illness at the time of NEXVIAZYME infusion you may be at greater risk for IARs. If you have advanced Pompe disease you may have compromised heart and breathing function, which may put you at a higher risk of severe complications from IARs.

Risk of Acute Cardiorespiratory Failure in Susceptible Patients

If you are likely to develop fluid volume overload, or have acute underlying breathing problems or compromised heart or breathing function that may require fluid restriction, there may be a risk of worsening of your heart or breathing status during NEXVIAZYME infusion. Your doctor may decide that close observation during NEXVIAZYME administration may be necessary.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions Including Anaphylaxis: Life-threatening hypersensitivity reactions, including anaphylaxis, were observed in NEXVIAZYME-treated patients. Some of the hypersensitivity reactions were related to the immune system. Symptoms of anaphylaxis may include difficulty breathing, chest discomfort, flushing/feeling hot, cough, redness of the skin, lip swelling, itching, swollen tongue, difficulty swallowing, and rash. Symptoms of severe hypersensitivity reactions may include difficulty breathing, redness of the skin, hives, swollen tongue, and rash. Your doctor may decide to give you antihistamine, anti-fever and/or steroid medications before your infusions.

Infusion-Associated Reactions (IARs): In clinical studies, IARs were observed during and/or within a few hours after the NEXVIAZYME infusion. Symptoms of IARs that required stopping the infusion included chest discomfort, cough, dizziness, redness of the skin, flushing/feeling hot, nausea, redness of the eye, and difficulty breathing. Your doctor may decide to give you antihistamine, anti-fever and/or steroid medications before your infusions to decrease the risk of IARs; however, IARs may still occur after receiving these medications.

Risk of Acute Cardiorespiratory Failure in Susceptible Patients: If you are likely to develop fluid volume overload, or have acute underlying breathing problems or compromised heart or breathing function that may require fluid restriction, there may be a risk of worsening of your heart or breathing status during NEXVIAZYME infusion, and your doctor may decide that close observation during and after NEXVIAZYME administration may be necessary.

ADVERSE REACTIONS

The most common adverse reactions (>5%) were headache, fatigue, diarrhea, nausea, joint pain, dizziness, muscle pain, itching, vomiting, shortness of breath, rash, “pins-and-needles” sensation, and hives.

INDICATION

NEXVIAZYME (avalglucosidase alfa-ngpt) is used for the treatment of patients 1 year of age and older with late-onset Pompe disease [lysosomal acid alpha-glucosidase (GAA) deficiency].

Please see full Prescribing Information for complete details, including Boxed WARNING.

Health information contained herein is provided for general educational purposes only. Your healthcare provider is the single best source of information regarding your health. Please consult your healthcare provider if you have any questions about your health or treatment.

SANOFI GENZYME

MAT-US-2106394-v1.0-08/21

Nexviazyme™
(avalglucosidase alfa-ngpt)