

Supporting information for adults, children and adolescents of all ages who have been prescribed Nexviadyme.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See www.mhra.gov.uk/yellowcard for how to report side effects.

 **Nexviadyme**[®] ▼
(avalglucosidase alfa)



POMPE
YOUR WAY

This leaflet is not intended to be a substitute for medical advice. Please refer to the Patient Information Leaflet in the pack for full safety information and speak to a healthcare professional should you need more information or advice.

Let's get started

Whether you are just starting treatment for Pompe disease, switching from another treatment or caring for someone who has been prescribed Nexviadyme, this brochure is for YOU!

It is a great way to learn about getting started on Nexviadyme therapy and, together with the help and advice of your healthcare team, has the information and support you need to take this important step.



Read on to learn more about Pompe disease and what can be expected from treatment with Nexviadyme. This brochure includes how Nexviadyme works, how it is given, possible side effects and additional resources for living with Pompe disease. Definitions for the underlined terms in this brochure can be found in the Glossary on page 9.

Pompe disease

Pompe disease is often classified as 1 of 2 different forms: late-onset Pompe disease (LOPD) and infantile-onset Pompe disease (IOPD), which can range in symptoms and severity, and the age when symptoms first appear. Pompe disease affects every person differently.^{1,2}



LOPD

Symptoms of LOPD can present at any age, range from mild to severe, and often include difficulty walking and breathing.^{1,2}



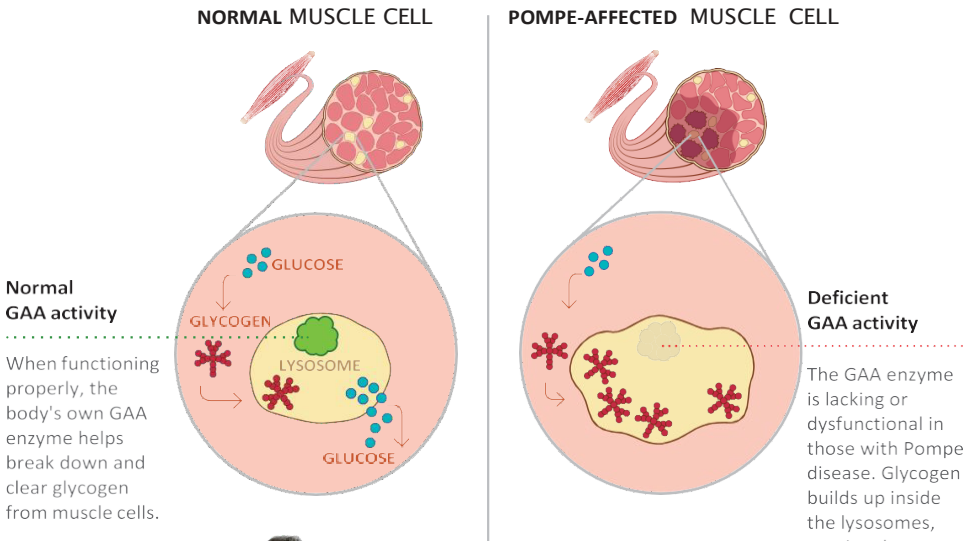
IOPD

Symptoms of IOPD are serious and appear early. Infants may experience heart and breathing problems, as well as muscle weakness, a few days to a few months after birth.^{1,2}

What causes Pompe disease?

In people with Pompe disease, an enzyme called acid alpha-glucoSIDase (GAA) is lacking or dysfunctional. This enzyme has the important function of breaking down glycogen into glucose inside the lysosomes of muscle cells.^{1,2}

In Pompe disease, the breakdown of glycogen to glucose inside the lysosomes of muscle cells is impaired^{1,2}



Widespread muscle damage

Continuous glycogen build-up can lead to potentially irreversible muscle damage and difficulty with breathing, walking and other daily activities.

Pompe disease can vary in severity; work with your healthcare team to manage your condition as best fits your lifestyle.

Nexviadyme: an enzyme replacement therapy (ERT) for people with Pompe disease

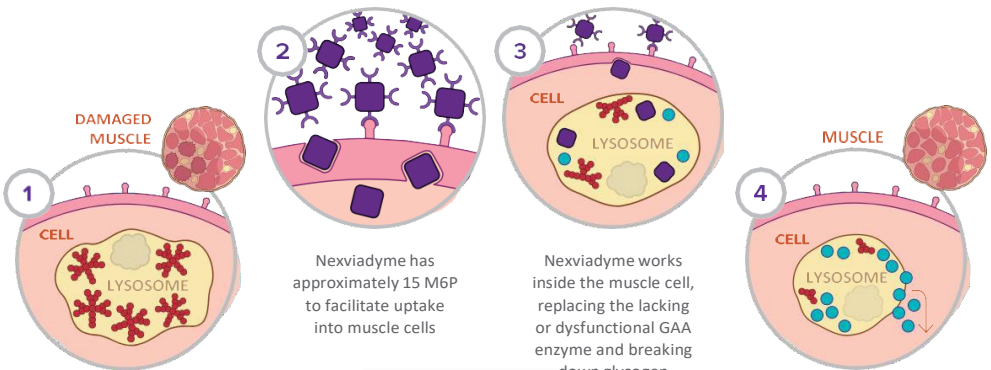
How does Nexviadyme treat Pompe disease³?

Having Pompe disease means your body needs help breaking down glycogen, particularly in muscle cells. Nexviadyme is an ERT that delivers the GAA enzyme to replace the one that is lacking or dysfunctional.

ERT uptake into muscle cells is facilitated by mannose-6-phosphate (M6P) on the enzyme. When M6P binds to M6P receptors on muscle cells, it allows the enzyme to enter the cells where it is needed.

Inside the muscle cell, Nexviadyme breaks down glycogen to prevent or slow potentially irreversible muscle damage.

Nexviadyme at work



In Pompe disease, glycogen builds up in the lysosomes of muscle cells due to lacking or dysfunctional GAA enzyme

Nexviadyme has approximately 15 M6P to facilitate uptake into muscle cells

Nexviadyme works inside the muscle cell, replacing the lacking or dysfunctional GAA enzyme and breaking down glycogen in the lysosomes

Nexviadyme reduces the build-up of glycogen to help prevent ongoing muscle damage

LACKING OR DYSFUNCTIONAL GAA

GLYCOGEN

M6P CELL RECEPTOR

NEXVIADYME

M6P

GLUCOSE



What to expect from treatment with Nexviadyme

Each person's Nexviadyme treatment experience is unique. Talk with your healthcare team to understand the potential benefits and risks that treatment with Nexviadyme might have for you or your loved ones.

Nexviadyme treatment is one important part of the Pompe disease care plan. During the treatment journey, work with your healthcare team to set goals and strategies for managing your Pompe disease symptoms, and to schedule appointments for monitoring overall well-being.

Living with Pompe disease

Nexviadyme provides a treatment and your healthcare team provides the guidance. Here are additional things that you can do^{4,5}:



Work your heart and lungs, keep your body moving with regular stretching and exercise, and consider a physiotherapist if needed



Remember to use your assistive devices when needed (such as your cane, walker, or ventilator)



Maintain a healthy diet and drink plenty of liquids



Stay engaged with family, friends and co-workers, and ask for emotional support when needed



Ask your doctor for regular check-ups; this may include heart or other types of monitoring



Never forget your mind, body, and spirit work together to keep you feeling and performing your best

Get the most out of treatment with Nexviadyme; work with your healthcare team to develop a care plan for managing Pompe disease.

How is Nexviadyme given?

Nexviadyme is given through intravenous infusion by a healthcare professional at a hospital or clinic. If you tolerate infusions well, they may be done in the comfort of your home. Your healthcare professional will determine when this may be appropriate.

How to take Nexviadyme?



Nexviadyme is given every 2 weeks by intravenous (IV) infusion.



The infusion usually takes 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 pre-treatment. Also, infusion times may vary based on your response to therapy and comfort.



Initially, you will receive bi-weekly infusions in a hospital setting, such that you can be closely monitored. After this, you may be able to receive these bi-weekly infusions from home.



Your healthcare provider can give you more details about what to expect during and after your infusion and how to prepare.

Reporting of side effects

If you get any side effects, talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. You can also report side effects directly via the Yellow Card Scheme at www.mhra.gov.uk/yellowcard.

By reporting side effects, you can help provide more information on the safety of this medicine.

Safety and side effects of Nexviadyme

Like all medicines, this medicine can cause side effects, although not everybody gets them. Side effects were mainly seen while patients were being given the medicine or shortly after (infusion-associated reactions [IARs]). You must tell your doctor immediately if you get an infusion-associated reaction or an allergic reaction. Your doctor may give you medicines before your infusion to prevent these reactions.

The very common side effects (may affect more than 1 in 10 people) in people of all ages with Pompe disease receiving Nexviadyme are:

- Hypersensitivity
- Headache
- Nausea
- Itchy skin
- Rash

Common side effects (may affect up to 1 in 10 people) may include:

- Feeling cold or hot
- Dizziness
- Sleepiness
- Shaking
- Burning sensation
- Red eyes
- Itchy eyes
- Swelling of eyelid
- Rapid heartbeat
- Redness of hands
- Redness of skin
- Muscle spasm
- Flank pain
- Fever
- Flu-like illness
- Abdominal (belly) pain upper
- Flushing
- Raised blood pressure
- Low blood pressure
- Skin and lips turning blue
- Hot flush
- Pale skin
- Cough
- Difficulty breathing
- Throat irritation
- Red rash
- Excessive sweating
- Muscle aches
- Fatigue
- Chest discomfort
- Infusion site pain
- Severe allergic reaction (anaphylaxis)
- Mouth and throat pain
- Diarrhoea
- Vomiting
- Lip swelling
- Swollen tongue
- Abdominal (belly) pain
- Weakness
- Indigestion
- Itchy, lumpy rash (hives)
- Itchy rash
- Skin plaque
- Pain in arm or leg
- Chills
- Pain
- Low blood oxygen
- Swelling of face



Communication is key: If you have any questions, experience swelling of your lower limbs or widespread swelling across your body, or do not feel well after your infusion, let your doctor know.



Pregnancy, fertility and breast-feeding: If you are pregnant or breast-feeding, think you may be pregnant, or are planning to have a baby, ask your doctor or pharmacist for advice before using this medicine. There is no information about the use of Nexviadyme in pregnant women. You must not receive Nexviadyme during pregnancy unless your doctor specifically recommends it. You and your doctor should decide if you can use Nexviadyme if you are breast-feeding.

For the complete list of possible side effects and Full product information, please read the Patient Information Leaflet.

Make more connections

Support. Understanding. Information you can trust.

Your experience living with Pompe disease is unique, but you are not alone. Connect with others who know what you are going through.

Support & resources

Various patient advocacy groups (PAGs) offer support for people with Pompe disease in the UK. Some of these PAGs are listed below:

Association for Glycogen Storage Disease UK: a national support group for those affected by glycogen storage disease (GSD) and their families

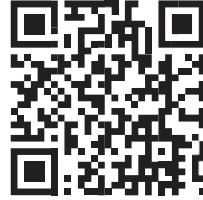
Pompe Support Network: a support network specifically for those affected by Pompe disease.

Metabolic Support UK: a patient organisation for Inherited Metabolic Disorders supporting thousands of patients worldwide.

References:

1. Kishnani et al. (2006). Pompe disease diagnosis and management guideline. *Genetics in medicine : official journal of the American College of Medical Genetics*, 8(5), 267–288. <https://doi.org/10.1097/01.gim.0000218152.87434.f3>
2. Van der Ploeg and Reuser. (2008) Pompe's disease. *The Lancet*, 372(9646), 1342–1353. [https://doi.org/10.1016/S0140-6736\(08\)61555-X](https://doi.org/10.1016/S0140-6736(08)61555-X).
3. Nexviadyme (avalglucosidase alfa) Summary of Product Characteristics. Sanofi, 2024..
4. Pompe Support Network. (2020) Community Support Groups. Available at: <<https://pompe.uk/community-groups>> [Accessed February 2024].
5. Association for Glycogen Storage Disease (2022). Pompe Disease (GSD2) Diagnosis & Follow up - Late Onset. Available at: <<https://agsd.org.uk/all-about-gsd/gsd-variants/pompe-disease-gsd2/gsd2-section1b-tab5/>> [Accessed February 2024].

FOR MORE INFORMATION ON
NEXVIADYME PLEASE SCAN THE
BELOW QR CODE:



Or visit www.nexviadyme.co.uk



Glossary of important terms

Acid alpha-glucosidase (GAA): an enzyme responsible for breaking down glycogen to glucose. This enzyme is in low amounts or not working in people with Pompe disease

Bind: Connection between a cell and another substance (enzyme, medication, etc.)

Echocardiogram: A painless and non-invasive technique to check how the heart is working through the use of ultrasound waves

Enzyme: A type of protein that facilitates biochemical reactions in the body

Enzyme replacement therapy (ERT): A treatment that replaces an enzyme that is missing or only present in low amounts

Glucose: A type of simple sugar that is used by the body for energy

Glycogen: A complex sugar made up of many bits of glucose, stored in cells (especially muscle cells) or broken down and used by the body for energy

Intravenous infusion: A method of giving medicine via a drip through a needle placed into a vein

Lysosome: An inner part of the cell that holds many different enzymes and acts as the digestive system for the cell to break down complex molecules

Mannose-6-phosphate (M6P): A type of marker on both natural and therapeutic enzymes that helps them bind and enter the cells and lysosomes where they belong


Uptake: The process of bringing a material (enzymes, medications, sugars, etc.) from outside the cell to inside the cell so it can be broken down and used by the body

Ventilator: A machine that moves air in and out of the lungs to help people who cannot breathe properly

Talk with your healthcare team if you have any questions about Nexviadyme or Pompe disease.

Sanofi: a legacy in rare diseases for 40 years

A rare commitment to the Pompe community.

A photograph of a man and a young girl walking hand-in-hand on a paved path in a park. The man is on the right, wearing a grey t-shirt and blue pants, smiling. The girl is on the left, wearing a grey t-shirt with a floral pattern and white shoes, also smiling. The background shows trees and a park bench.

Living with Pompe disease is a journey.
We continue our commitment to support you and
the Pompe disease community for years to come.

sanofi

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 Nexviadyme®