

# Media Update: New data at WMS 2023 reaffirm long-term efficacy of Nexviazyme® (avalglucosidase alfa) for the treatment of Pompe disease



PUBLISHED OCT 6, 2023  
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New data at WMS 2023 reaffirm long-term efficacy of Nexviazyme® (avalglucosidase alfa) for the treatment of Pompe disease

- Findings include long-term data of up to nearly three years, as well as real-world evidence, across a wide range of patients and clinical circumstances
- Data add to the totality of evidence demonstrating Nexviazyme's value to the Pompe community

Paris, October 6, 2023. New data being shared this week by Sanofi at the 28th Annual Congress of the World Muscle Society (WMS) in Charleston, South Carolina, U.S., build upon the considerable body of evidence supporting the use of Nexviazyme® (avalglucosidase alfa) to treat a wide range of patients living with Pompe disease across various clinical circumstances. Nexviazyme is a monotherapy approved in the United States and other markets for the treatment of late-onset Pompe disease and is approved for infantile-onset Pompe disease in Europe and other countries. In the U.S., Nexviazyme is currently being evaluated in Phase 3 clinical trials for infantile-onset Pompe disease.

Data include research across people living with late-onset or infantile-onset Pompe disease, people who are treatment naïve (never before treated) or have switched from previous treatment with the long-time standard of care, alglucosidase alfa, which is marketed under the brand name Myozyme® or in the U.S. as Lumizyme®, and patients with varying baseline characteristics.

Of note, key data being shared at WMS stem from the Phase 3 COMET trial evaluating the long-term efficacy, safety and durability of Nexviazyme in those living with late-onset Pompe. The nearly three-year data demonstrate that patients who started treatment with Nexviazyme, whether treatment naïve or previously treated, had improvement or stabilization of respiratory function and mobility. Data also indicate that Nexviazyme was well tolerated by patients who switched from alglucosidase alfa.

## Global Head of Medical Affairs, Rare Diseases

Our findings being shared at WMS build upon existing evidence that supports the value of Nexviazyme in treating Pompe disease. With efficacy and safety demonstrated across a variety of patient cohorts, we remain confident in Nexviazyme as a compelling treatment option.

As part of a long-standing commitment to helping improve the lives of those living with rare diseases, Sanofi has been focused on providing treatments for people living with Pompe disease for more than 20 years, beginning with the development of the first approved treatment for this condition, Myozyme in 2006. Since then, Sanofi has continued to work closely with the Pompe community to help address unmet patient needs which resulted in the development of Nexviazyme.

People living with Pompe disease have low levels of the enzyme acid alpha-glucosidase (GAA), which results in build-up of glycogen in muscle cells throughout the body, which can lead to irreversible damage to skeletal and cardiac muscles.

Pompe disease can present as infantile-onset Pompe disease (IOPD), the most severe form of the disease with rapid onset in infancy, or late-onset Pompe disease (LOPD), which progressively damages muscles over time. If left untreated, IOPD can lead to heart failure and death within the first year of life, while people living with LOPD may require mechanical ventilation to help with breathing or a wheelchair to assist with mobility as the disease progresses.

## About Nexviazyme (avalglucosidase alfa)

Nexviazyme (avalglucosidase alfa and avalglucosidase alfa-ngpt, in the U.S.) is an enzyme replacement therapy (ERT) designed to target the mannose-6-phosphate (M6P) receptor, the key pathway for uptake

and transport of ERT. Nexviazyme aims to help improve uptake and enhance glycogen clearance in target tissues with an average 15-fold higher level of M6P moieties as compared to alglucosidase alfa, the comparator therapy in the pivotal study. Nexviazyme is approved in multiple markets around the world for the treatment of people living with Pompe disease, with specific indications varying by country. In the U.S., Nexviazyme is indicated for the treatment of late-onset Pompe Disease in patients 1 year of age and older. In Europe, the medicine is marketed under the brand name Nexviadyme and is indicated for the treatment of both late-onset and infantile-onset Pompe disease.

We are an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives. Our team, across some 100 countries, is dedicated to transforming the practice of medicine by working to turn the impossible into the possible. We provide potentially life-changing treatment options and life-saving vaccine protection to millions of people globally, while putting sustainability and social responsibility at the center of our ambitions.

Sanofi is listed on Euronext: SAN and Nasdaq: SNY

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in Sanofi’s annual report on Form 20-F for the year ended December 31, 2022. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

*Press release distributed by Wire Association on behalf of Sanofi, on Oct 6, 2023. For more information subscribe and [follow us](#).*

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<https://wireassociation.eu/newsroom/sanofi/releases/en/media-update-new-data-at-wms-2023-reaffirm-long-term-efficacy-of-nexviazyme-avalglucosidase-alfa-for-the-treatment-of-pompe-disease-1454>

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