

## *Australia among world's best in treating debilitating and often fatal muscle disorder*

**Sydney – 4 September 2022** - Australia has become one of the first countries in the world to fund a new, next-generation treatment for people with a potentially fatal muscle disorder known as Pompe disease.

The Federal Government will add Nexviazyme® (avalglucosidase alfa) to the Life Saving Drugs Program from September 1 for the treatment of Australians one year of age and older with Pompe disease,<sup>1</sup> a progressive and debilitating muscle disorder that impairs a person's ability to move and breathe.<sup>2</sup>

Approximately 70 Australians are living with Pompe disease,<sup>2</sup> which if left untreated is fatal in young babies, and when it occurs in children and adults, leads to severe disability requiring mechanical ventilation to help with breathing or a wheelchair to assist with mobility, and premature death.<sup>3</sup>

Without government funding, Nexviazyme would remain out of reach for Australians with Pompe disease. Until now, the therapy has only been available in a small number of countries across the globe.

Neurologist Associate Professor Robert Henderson from the Royal Brisbane and Women's Hospital said the addition of Nexviazyme to Australia's Life Saving Drugs Program was "a significant step forward for the Pompe community and represents the first new treatment for the condition in seven years".

"Clinicians treating Pompe disease are in a race against time to save muscle function so new treatments are urgently needed," he said.

"The opportunity to deliver this therapy for the first time for some patients through home infusion services will also make a significant difference to people who have work, school or family commitments, or those who had to travel extended distances to hospital for treatment," said Associate Professor Henderson.

Sanofi Australia and New Zealand Head of Medical, Specialty Care – Dr Kasia Siwek said that the addition of Nexviazyme to the Life Saving Drugs Program is an "important moment for the rare disease community".

"Sanofi welcomes the Government's decision to list Nexviazyme on the Life Saving Drugs Program. This is a significant and life-changing decision for Australians affected by this devastating disorder – many of whom have spent years seeking a diagnosis and effective treatment," she said.

"We are proud of our long-standing commitment to the rare disease community in Australia, and that we now supply six of the sixteen medicines on the Life Saving Drugs Program – a vital program that ensures those affected by rare diseases do not miss out on treatment, simply because their condition is rare.

“We look forward to working with the new Government to transform the treatment of Pompe disease and other rare conditions.”

A rare genetic condition that can strike at any age, Pompe disease occurs when those affected are unable to produce a protein that breaks down complex sugars. This leads to sugar accumulating in muscle cells, irreversible muscle damage, respiratory difficulties, difficulty moving, and impaired heart function.<sup>4</sup>

Due to the wide variety of symptoms affecting breathing and movement, alongside the rarity of the condition, it can take up to ten years for adults with the late onset form of the condition to be diagnosed.<sup>4</sup>

Nexviazyme enzyme replacement therapy is a monotherapy, administered by intravenous infusion every two weeks.<sup>1</sup>

All medicines have side effects. In clinical trials, the most frequently reported adverse reactions (>5%) in Nexviazyme-treated patients were headache, pruritus (itching sensation), nausea, hives and fatigue.<sup>1</sup> No patients discontinued treatment due to infusion-associated reactions or serious adverse reactions.<sup>1</sup>

**Note to Editor:** Associate Professor Robert Henderson has served on advisory boards and been involved in clinical trials sponsored by Sanofi for which compensation was received. In relation to this Sanofi media announcement, no compensation was provided, and the opinions expressed are his own.

For information about Nexviazyme, please contact Sanofi Medical Information on 1800 818 806.

**The Nexviazyme CMI is available at the following link:**

<https://secure.guildlink.com.au/gc/ws/sw/cmi.cfm?product=swcnexvi>

#### *About Sanofi*

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 centre of our ambitions. Sanofi is listed on Euronext: SAN and NASDAQ: SNY

**Issued by Ethical Strategies on behalf of Sanofi Australia.**

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#### *References:*

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