

Sanofi to present Phase 3 results of avalglucosidase alfa in patients with late-onset Pompe disease

- Virtual scientific session June 16, 2020, 8:00-9:00am ET/2:00-3:00pm CET
- Avalglucosidase alfa receives FDA Breakthrough Therapy designation

PARIS – June 8, 2020 – Sanofi will host a virtual scientific session to present data from the Phase 3 COMET trial of investigational enzyme replacement therapy (ERT) avalglucosidase alfa in patients with late-onset Pompe disease (LOPD).

The session, open to healthcare professionals and members of the media, will include a data presentation by Jordi Diaz-Manera, M.D., Ph.D., Professor of Neuromuscular Disorders, Translational Medicine and Genetics at the John Walton Muscular Dystrophy Research Center, Newcastle University, UK, and Professor of Neuromuscular Diseases, Translational Medicine and Genetics in the Neuromuscular Diseases Unit, Neurology department of Hospital de la Santa Creu, Barcelona, Spain.

The presentation will be followed by a Q&A session moderated by Alaa Hamed, M.D., MPH, MBA, Global Head of Medical Affairs, Rare Diseases at Sanofi.

The scientific session, endorsed by the COMET trial author group, is being scheduled as a result of the postponement of the July 2020 International Congress on Neuromuscular Diseases (ICNMD) due to the COVID-19 pandemic. Data from the Phase 3 COMET trial would have been presented at the July 2020 ICNMD. Pre-registration is required for the June 16, 2020 scientific session. Please click [here](#) to register.

The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation status to avalglucosidase alfa for the treatment of patients with a confirmed diagnosis of Pompe disease. Breakthrough Therapy designation serves to expedite the development and review of drugs that target serious or life-threatening conditions. Drugs qualifying for this designation must show preliminary clinical evidence of a substantial improvement on a clinically significant endpoint over available therapies, or over placebo if there is no available therapy.

About Pompe disease

Pompe disease is caused by a genetic deficiency or dysfunction of the lysosomal enzyme acid alpha-glucosidase (GAA), resulting in build-up of glycogen in muscles, including the proximal muscles and the diaphragm, and eventually causing progressive and irreversible muscle damage. This rare disease affects an estimated 50,000 people worldwide and can manifest at any age from infancy to late adulthood.

Pompe disease is often classified as late-onset Pompe disease (LOPD) or infantile-onset Pompe disease (IOPD). Patients with LOPD typically present any time after the first year of life to late adulthood. The hallmark symptoms of LOPD are impaired respiratory function and skeletal muscle weakness, which often leads to impaired mobility. Patients often require wheelchairs to assist with mobility and may require mechanical ventilation to help with breathing.ⁱ Respiratory failure is the most common cause of death in patients with Pompe disease.ⁱⁱ Pompe disease is classified as IOPD when symptoms begin prior to one year of age. In addition to skeletal muscle weakness, heart function is also commonly impacted.

About Avalglucosidase alfa

The goal of ERT for Pompe disease is to deliver enzyme into the lysosomes within muscle cells to replace the missing or deficient GAA that is needed to prevent build-up of glycogen in the muscles. Avalglucosidase alfa is an investigational ERT for Pompe disease designed to improve the delivery of enzyme to the cells in the muscles, most notably into skeletal muscle. With approximately 15 moles of mannose-6-phosphate (M6P) per mole of GAA, avalglucosidase alfa aims to help improve cellular enzyme uptake and enhance glycogen clearance in target tissues.ⁱⁱⁱ

The FDA granted Fast Track designation to avalglucosidase alfa for the treatment of patients with Pompe disease. Avalglucosidase alfa has not been approved by the U.S. FDA or any other regulatory agency worldwide for the uses under investigation.

About Sanofi

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

Sanofi, Empowering Life

Media Relations Contact

Sally Bain
Tel.: +1 781-264-1097
Sally.Bain@sanofi.com

Investor Relations Contact

Felix Lauscher
Tel.: +33 (0)1 53 77 45 45
ir@sanofi.com

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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, 2018. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements..

ⁱ Hagemans ML, et al. Brain 2015;128:671-677.

ⁱⁱ Winkel LP et al. J Neurol. 2005;252(8):875–84.

ⁱⁱⁱ Zhou Q. Bioconjug Chem. 2011 Apr 20;22(4):741-51