



Media Update

EMA accepts regulatory submission for olipudase alfa, the first potential therapy for ASMD

- * Olipudase alfa has been granted PRImity Medicines (PRIME) designation in Europe, Breakthrough Therapy designation in the United States, and SAKIGAKE designation in Japan
- * European regulatory decision anticipated second half of 2022

DECEMBER 6, 2021

The European Medicines Agency (EMA) has accepted for review under an accelerated assessment procedure the Marketing Authorization Application (MAA) for olipudase alfa, Sanofi's investigational enzyme replacement therapy which is being evaluated for the treatment of acid sphingomyelinase deficiency (ASMD). Historically referred to as Niemann-Pick disease (NPD) type A and type B, ASMD is a rare, progressive, and potentially life-threatening disease for which no treatments are currently approved. The estimated prevalence of ASMD is approximately 2,000 patients in the U.S., Europe (EU5 Countries) and Japan. If approved, olipudase alfa will become the first and only therapy for the treatment of ASMD.

"Today's milestone has been decades in the making and our gratitude goes to the ASMD community who has stood by us with endless patience while olipudase alfa advanced through clinical development," said Alaa Hamed, MD, MPH, MBA, Global Head of Medical Affairs, Rare Diseases, Sanofi. *"Olipudase alfa represents the kind of potentially life-changing innovation that is possible when industry, medical professionals and the patient community work together toward a common goal."*

The MAA is based on positive results from two separate clinical trials (ASCEND and ASCEND-Peds) evaluating olipudase alfa in adult and pediatric patients with non-central nervous system (CNS) manifestations of ASMD type A/B and ASMD type B.

Olipudase alfa has received special designations from regulatory agencies worldwide, recognizing the innovation potential of the investigational therapy.

"Scientific innovation is the greatest source of hope for people living with diseases like ASMD where there are no approved treatments and is a critical component for ensuring a viable healthcare ecosystem," said Bill Sibold, Executive Vice President of Sanofi Genzyme. *"At Sanofi, we have a long history of pioneering scientific innovation,*

and we remain committed to finding solutions to address unmet medical needs, including those of the rare disease community.”

The EMA awarded olipudase alfa the PRImity MEdicines designation, also known as PRIME, intended to aid and expedite the regulatory process for investigational medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options.

The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to olipudase alfa. This designation is intended to expedite the development and review of drugs intended to treat serious or life-threatening diseases and conditions. The criteria for granting Breakthrough Therapy designation include preliminary clinical evidence indicating that the molecule may demonstrate substantial improvement on a clinically significant endpoint over available therapies.

In Japan, olipudase alfa was awarded the SAKIGAKE designation, which is intended to promote research and development in Japan for innovative new medical products that satisfy certain criteria, such as the severity of the intended indication. In September, Sanofi filed the J-NDA submission for olipudase alfa.

About ASMD

ASMD results from a deficient activity of the enzyme acid sphingomyelinase (ASM), which is found in special compartments within cells called lysosomes and is required to breakdown lipids called sphingomyelin. If ASM is absent or not functioning as it should, sphingomyelin cannot be metabolized properly and accumulates within cells, eventually causing cell death and the malfunction of major organ systems. The deficiency of the lysosomal enzyme ASM is due to disease-causing variants in the sphingomyelin phosphodiesterase 1 gene (SMPD1). The estimated prevalence of ASMD is approximately 2,000 patients in the U.S., Europe (EU5 Countries) and Japan.

ASMD represents a spectrum of disease caused by the same enzymatic deficiency, with two types that may represent opposite ends of a continuum sometimes referred to as ASMD type A and ASMD type B. ASMD type A is a rapidly progressive neurological form of the disease resulting in death in early childhood due to central nervous system complications. ASMD type B is a serious and potentially life-threatening disease that predominantly impacts the lungs, liver, and spleen, as well as other organs. ASMD type A/B represents an intermediate form that includes varying degrees of neurologic involvement. Patients with ASMD type A/B or ASMD type B were studied in the ASCEND trial program. Another type of NPD is NPD type C, which is unrelated to ASMD.

About olipudase alfa

Olipudase alfa is an investigational enzyme replacement therapy designed to replace deficient or defective ASM, allowing for the breakdown of sphingomyelin. Olipudase alfa is currently being investigated to treat non-CNS manifestations of ASMD. Olipudase alfa has not been studied in ASMD type A patients. Olipudase alfa is an investigational agent

and the safety and efficacy have not been evaluated by the FDA, EMA, or any other regulatory authority worldwide.

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.

Media Relations Contact

Sally Bain

Tel: +1 (781) 264-1091

Sally.Bain@sanofi.com

Investor Relations Contacts Paris

Eva Schaefer-Jansen

Arnaud Delepine

Nathalie Pham

Investor Relations Contact North America

Felix Lauscher

Tel.: +33 (0)1 53 77 45 45

investor.relations@sanofi.com

<https://www.sanofi.com/en/investors/contact>

Sanofi Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions, and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding 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 fact that product candidates if approved may not be commercially successful, the future approval and commercial success of therapeutic alternatives, Sanofi's ability to benefit from external growth opportunities, to complete related transactions 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 and market conditions, cost containment initiatives and subsequent changes thereto, and the impact that COVID-19 will have on us, our customers, suppliers, vendors, and other business partners, and the financial condition of any one of them, as well as on our employees and on the global economy as a whole. Any material effect of COVID-19 on any of the foregoing could also adversely impact us. This situation is changing rapidly, and additional impacts may arise of which we are not currently aware and may exacerbate other previously identified risks. The risks and uncertainties also include the uncertainties 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, 2020. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.