SANOFI

Press Release

Source: Sanofi (EURONEXT: SAN) (NASDAQ: SNY)

Sanofi provides update on Phase 3 study evaluating rilzabrutinib for the treatment of pemphigus

PARIS – September 9, 2021 – The Phase 3 PEGASUS trial evaluating rilzabrutinib to treat pemphigus, a rare autoimmune skin condition, did not meet its primary or key secondary endpoints. Rilzabrutinib's safety profile remained consistent with previous results and no new safety signals were identified.

The Phase 3 study, which is the first placebo-controlled trial of a BTK inhibitor in pemphigus, enrolled adult patients with moderate-to-severe pemphigus vulgaris or pemphigus foliaceus. The primary endpoint was complete remission from weeks 29 to 37 with minimal doses of corticosteroids (≤10/mg day). Complete remission was defined as the absence of new and established skin lesions. Results show the proportion of patients meeting the primary endpoint on rilzabrutinib was not significantly different from placebo.

Sanofi is continuing to evaluate the data and plans to share detailed findings at a future medical meeting.

> *"While these results are disappointing, we believe the rilzabrutinib clinical program holds great potential to address the unmet treatment needs of people living with immune-mediated diseases,"* said Naimish Patel, Head of Global Development, Immunology and Inflammation. *"Our mission is to improve outcomes by exploring new scientific approaches and novel therapies to advance the standard of care. We are committed to investigating rilzabrutinib further and progressing our clinical programs forward to deliver new treatment options for patients."*

Pemphigus is a group of potentially life-threatening disorders characterized by blisters and ulceration affecting the skin and mucous membranes. Currently options for the treatment of pemphigus (including pemphigus vulgaris and pemphigus foliaceus) are limited and systemic corticosteroid treatment remains the standard of care.

Rilzabrutinib is a potential first-in-class, oral Bruton's tyrosine kinase (BTK) inhibitor in development for immune-mediated diseases. The BTK enzyme plays a key role in a number of immune processes including B cell expansion, production of immunoglobulins, and activation of innate cells such as mast cells, eosinophils, and basophils. Positive clinical trial data from placebo-controlled studies of BTK inhibitors have revealed the potential role for BTK in rheumatoid arthritis and in chronic spontaneous urticaria[1],[2]. Thus the function of BTK is biologically diverse and supports continued investigation in a range of diseases with significant unmet need where BTK is implicated.

Rilzabrutinib is being investigated in a Phase 3 trial for the treatment of immune thrombocytopenia, a rare blood disorder, and in a Phase 2 study for the autoimmune condition IgG4-related disease. Additional Phase 2 studies in immunological diseases including asthma, atopic dermatitis, chronic spontaneous urticaria and warm autoimmune hemolytic anemia are planned to start in 2021.

About the PEGASUS study

The PEGASUS study is a Phase 3 randomized, parallel-group, double-blind, placebo-controlled trial which enrolled 131 patients with newly diagnosed or relapsing moderate-to-severe pemphigus in 19 countries worldwide. The primary endpoint was complete remission from weeks 29 to 37 with minimal doses of corticosteroids (CS) (≤10/mg day). Complete remission is defined as the absence of new and established skin lesions. Key secondary endpoints include cumulative CS dose (from Baseline to Week 37), cumulative duration of complete remission with a CS dose ≤10 mg/day and time to first complete remission with a CS dose ≤10 mg/day. (NCT03762265)

About Rilzabrutinib

Rilzabrutinib is an oral Bruton's tyrosine kinase inhibitor incorporating Sanofi's TAILORED COVALENCY®[3] technology being investigated for the treatment of immune-mediated diseases. BTK is an intracellular signaling molecule involved in innate and adaptive immune responses involved in certain immune-mediated diseases. By inhibiting BTK, rilzabrutinib has the potential to target the underlying disease pathogenesis.

Orphan drug designation was granted by the US Food and Drug Administration (FDA) for pemphigus vulgaris (and from the European Commission for the treatment of pemphigus) and for its investigational use in immune thrombocytopenia (ITP). Rilzabrutinib was granted FDA Fast Track Designation for ITP in November 2020 and for pemphigus vulgaris in May 2021.

Rilzabrutinib is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

---

[1] Cohen S, et al, Fenebrutinib versus Placebo or Adalimumab in Rheumatoid Arthritis: A Randomized, Double-Blind, Phase II Trial (ANDES Study). Arthritis Rheumatol. 2020 Apr 9;72(9):1435–46. doi: 10.1002/art.41275. Epub ahead of print. PMID: 32270926; PMCID: PMC7496340

[2]. Metz,M, et al Fenebrutinib in Refractory Chronic Spontaneous Uticaria, 2020, Abstract. Allergy, 75: 100-119. https://doi.org/10.1111/all.14505

[3] TAILORED COVALENCY is a registered trademark of Principia Biopharma Inc., a Sanofi Company.

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 Contacts North America
Felix Lauscher

Tel.: +33 (0)1 53 77 45 45
investor.relations@sanofi.com

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.