Horizon Therapeutics plc Initiates Phase 3 Clinical Trial (OPTIC-J) in Japan Evaluating Teprotumumab for the Treatment of Active Thyroid Eye Disease (TED)

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-- Teprotumumab is marketed under the brand name TEPEZZA® in the United States --

DUBLIN--(BUSINESS WIRE)--Feb. 23, 2022-- Horizon Therapeutics plc (Nasdaq: HZNP) today announced that the first patient has been enrolled in a Phase 3 clinical trial (OPTIC-J) in Japan evaluating teprotumumab for the treatment of active TED. TED is a serious, progressive and potentially vision-threatening rare autoimmune disease that can cause proptosis (eye bulging), diplopia (double vision), eye pain, redness and swelling.[1] Teprotumumab, marketed under the brand name TEPEZZA in the United States (U.S.), was approved by the U.S. Food and Drug Administration (FDA) in January 2020 as the first and only medicine for TED, and received Priority Review, Orphan Drug, Fast Track and Breakthrough Therapy designations. Teprotumumab has not been approved for commercial use in Japan.

The OPTIC-J trial is a randomized, double-masked, placebo-controlled, parallel-group study that will evaluate the efficacy, tolerability and safety of teprotumumab in the treatment of patients in Japan with moderate-to-severe active TED. The trial methodology is based on the OPTIC Phase 3 trial conducted in the United States and Europe and will include approximately 50 adults who meet the trial eligibility criteria at trial sites across Japan. Patients will be randomized in a 1:1 ratio to receive teprotumumab or placebo once every three weeks for a total of eight infusions (10 mg/kg for the first infusion and 20 mg/kg for the remaining seven infusions). This dosage and administration are based on approval in the United States and may not be the same if approved in Japan.

The primary efficacy endpoint is proptosis response rate at Week 24, measured by the percentage of participants with at least a 2 mm reduction in proptosis from baseline in the study eye, without deterioration in the fellow eye (≥ 2 mm increase). The trial will also assess overall responder rate, the percentage of patients with a Clinical Activity Score (CAS) of 0 or 1 at Week 24 in the study eye, change from baseline at Week 24 in proptosis measurement in the study eye, diplopia responder rate, and the change from baseline to Week 24 in the Graves' Ophthalmopathy Quality of Life (GO-QoL) questionnaire. Study participants who complete the treatment period and are proptosis non-responders at Week 24 or relapse during the 48-week follow-up may choose to enter an open-label extension period to receive an additional eight infusions of teprotumumab.

"There is a significant medical need in Japan for a medicine that can effectively treat proptosis and diplopia, which are two of the most debilitating symptoms of Thyroid Eye Disease," said Yuji Hiromatsu, M.D., emeritus professor, Kurume Medical Center and co-coordinating trial investigator. "Steroids are commonly used in Japan to help ease inflammation but can have serious side effects and have not been shown to reduce proptosis or improve diplopia. If left untreated, Thyroid Eye Disease can cause vision loss, severe pain and other symptoms that make it difficult to work, sleep and spend time with family and friends."

"Given the unmet need in Japan for a medicine that treats Thyroid Eye Disease at the source, as well as the robust clinical response with teprotumumab treatment that was observed in prior clinical trials and the post-marketing experience in the U.S., we are eager to understand how this therapy may help patients in Japan specifically," said Elizabeth H.Z. Thompson, Ph.D., executive vice president, research and development, Horizon. "We are committed to collaborating with Japanese researchers and regulators on this important initiative, with the ultimate goal of bringing a valuable new treatment option to patients."

The trial was designed in consultation with Japan's Pharmaceuticals and Medical Devices Agency (PMDA). OPTIC-J is an acronym for Treatment of Graves' Orbitopathy (Thyroid Eye Disease) to Reduce Proptosis with Teprotumumab Infusions in a Randomized, Placebo-Controlled, Clinical Study – Japan. More information about the trial, including eligibility criteria, is available on the Japan Registry of Clinical Trials website (trial ID number jRCT2031210453).

About Thyroid Eye Disease (TED)

TED is a serious, progressive and potentially vision-threatening rare autoimmune disease.[1] TED often occurs in people living with Graves' disease, but is a distinct disease that is caused by autoantibodies activating an IGF-1R-mediated signaling complex on cells within the retro-orbital space.[2,3] This leads to a cascade of negative effects, which may cause long-term, irreversible damage, including blindness.[4,5] TED begins with an acute (active) phase where inflammatory signs and symptoms, such as eye pain, swelling, proptosis and diplopia progress over time.[1,4] The disease then enters a chronic phase where inflammation is no longer present or has markedly diminished, but significant signs and symptoms may remain.

About Teprotumumab

Teprotumumab is a fully human monoclonal antibody (mAb) and a targeted inhibitor of the insulin-like growth factor-1 receptor (IGF-1R). Teprotumumab is a biologic that is administered to patients once every three weeks for a total of eight infusions. It received Priority Review, Orphan Drug, Fast Track and Breakthrough Therapy designations from the United States FDA. In prior clinical trials, the most common adverse reactions (incidence ≥5% and greater than placebo) were muscle spasm, nausea, alopecia, diarrhea, fatigue, hyperglycemia, hearing impairment, dysgeusia, headache, dry skin, and menstrual disorders.

About Horizon

Horizon is focused on the discovery, development and commercialization of medicines that address critical needs for people impacted by rare, autoimmune and severe inflammatory diseases. Our pipeline is purposeful: We apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives. For more information on how we go to incredible lengths to impact lives, visit www.horizontherapeutics.com and follow us on Twitter, LinkedIn, Instagram and Facebook.

Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the potential benefits of TEPEZZA (teprotumumab); the expected scope, endpoints and timing of the OPTIC-J clinical trial and other statements that are not historical facts. These forward-looking statements are based on Horizon's current expectations and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks related to clinical trials, including the fact that prior results may not predict future clinical trial outcomes; impacts of the COVID-19 pandemic and actions taken to slow its spread, including potential delays in clinical trials; regulatory obligations and oversight, including any changes in the legal and regulatory environment in Japan and those risks detailed from time-to-time under the caption "Risk Factors" and elsewhere in Horizon's filings and reports with the SEC. Horizon undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information.

References

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