Clinical Study Data Highlighting the Impact of Teprotumumab in Patients With Active Thyroid Eye Disease to Be Presented During ACR Annual Meeting

November 4, 2019

DUBLIN--(BUSINESS WIRE)--Nov. 4, 2019-- Horizon Therapeutics plc (Nasdaq: HZNP) today announced an integrated analysis of efficacy data from the Phase 2 and Phase 3 clinical trials of teprotumumab will be presented during an oral session at the American College of Rheumatology (ACR) Annual Meeting, Nov. 8-13 in Atlanta. The U.S. Food and Drug Administration is currently evaluating under Priority Review a Biologics License Application (BLA) for teprotumumab in the treatment of active thyroid eye disease (TED). If approved, teprotumumab would be the first FDA-approved medicine for active TED. The Prescription Drug User Fee Act (PDUFA) goal date is March 8, 2020. Teprotumumab is an investigational medicine and its safety and efficacy have not been established.

Presentation Details:

- **Oral Presentation:** Teprotumumab, A Novel Biologic for Active Thyroid Eye Disease (abstract 1807)
- **Speaker:** Raymond Douglas, M.D., Ph.D., director of the Orbital and Thyroid Eye Disease Program, Cedars-Sinai Medical Center
- **Date:** Monday, Nov. 11
- **Time:** 2:45-3 p.m. ET

About Thyroid Eye Disease

Thyroid eye disease (TED) is a serious, progressive and vision-threatening autoimmune disease with a limited window of activity that can last up to three years.[1,2,3] While TED often occurs in people living with hyperthyroidism or Graves' disease, it is a distinct disease that is caused by autoantibodies activating an IGF-1R-mediated signaling complex on cells within the orbit.[4,5] This leads to a cascade of negative effects, which may cause long-term, irreversible damage. Active TED is characterized by inflammation and tissue expansion behind the eye.[1,6] As TED progresses, it causes serious damage – including proptosis (eye bulging), strabismus (misalignment of the eyes) and diplopia (double vision) – and in some cases can lead to blindness.[2,7] TED has only been shown to respond to pharmacotherapy while the disease is active and inflammation is ongoing.[8] Currently, patients must live with active TED until the inflammation subsides, after which they are often left with permanent and vision-impairing consequences.[1,6]

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 has received Priority Review, Orphan Drug, Fast Track and Breakthrough Therapy designations from the FDA. The clinical development program for teprotumumab in the treatment of TED includes positive results from the Phase 2 clinical study, which were published in The New England Journal of Medicine, as well as positive results from the Phase 3 OPTIC confirmatory clinical trial. The OPTIC trial was conducted at leading centers in the U.S., Germany and Italy, with co-principal investigators Raymond Douglas, M.D., Ph.D., Cedars-Sinai Medical Center and George Kahaly, M.D., Ph.D., Johannes Gutenberg University Medical Center. Horizon is also conducting the OPTIC-X extension trial to gather further insight into the long-term efficacy and safety of teprotumumab.

About Horizon

Horizon is focused on researching, developing and commercializing medicines that address critical needs for people impacted by rare and rheumatic 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, please visit www.horizontherapeutics.com, follow us @HorizonNews on Twitter, like us on Facebook or explore career opportunities on LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential regulatory approval of teprotumumab and associated timing. Forward-looking statements speak only as of the date of this press release and Horizon does not undertake any obligation to update or revise these statements, except as may be required by law. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include, but are not limited to, risks regarding whether the FDA will approve teprotumumab as a treatment for active TED on the timeline Horizon expects or at all. For a further description of these and other risks facing Horizon, please see the risk factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon undertakes no obligation to update or revise these statements, except as may be required by law.

References

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