



## Horizon Pharma plc to Present 48-Week Follow-Up Analysis of the Teprotumumab Phase 2 Trial at the Annual Meeting of the American Thyroid Association

September 25, 2018

-- Horizon to host an investor call on Oct. 4 following the teprotumumab data presentation --

DUBLIN--(BUSINESS WIRE)--Sep. 25, 2018-- Horizon Pharma plc (NASDAQ: HZNP) will present new data on the 48-week follow up for the Phase 2 clinical trial evaluating teprotumumab for the treatment of moderate-to-severe active thyroid eye disease (TED). The data will be presented on Oct. 4, 2018, at the 2018 Annual Meeting of the American Thyroid Association (ATA) in Washington, D.C.

"These new data provide an analysis of Phase 2 results for teprotumumab nearly a full year off therapy and allows for evaluation of durability of response," said Shao-Lee Lin, M.D., Ph.D., executive vice president, head of research and development and chief scientific officer, Horizon Pharma plc. "Our clinical presence at this year's ATA meeting underscores the medical community's interest in understanding the potential role teprotumumab may have in the treatment of TED."

### Oral Presentation Details:

*48-Week Follow-Up Of A Multicenter, Randomized, Double-Masked, Placebo Controlled Treatment Trial Of Teprotumumab In Thyroid-Associated Ophthalmopathy*

- **Abstract:** Highlighted Oral Abstract 2
- **Authors:** Kahaly, George; Douglas, Raymond; Holt, Robert J; Perdok, Renee; Ball, Julie; Smith, Terry
- **Date:** Thursday, Oct. 4
- **Time:** 9:05 a.m. – 9:20 a.m. ET

Horizon Pharma will host an investor call on Thursday, Oct. 4, 2018, at 10:30 a.m. ET to discuss these data, as well as provide an overview of TED and the current treatment landscape.

These studies are investigational and the information included has not been approved by health authorities. Safety and efficacy have not been established.

### About Thyroid Eye Disease

Thyroid eye disease (TED) is a rare autoimmune disease that is active for up to three years. During active TED the insulin-like growth factor receptor (IGF-1R) is overexpressed on orbital fibroblasts, resulting in local inflammation and tissue expansion, which can in turn lead to proptosis, or bulging of the eye. This can result in stiffness of the muscles, displacement of the eyes so they are no longer in line with each other, and/or the eyelids are unable to close. The inability for people living with active TED to close their eyelids can lead to corneal ulcerations and potential blindness, and many also endure challenges with double vision, known as diplopia.

### 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 Breakthrough Therapy, Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration (FDA). A Phase 3 confirmatory study was launched in October 2017 after results from the randomized double-blind, placebo controlled Phase 2 study. Results from this study were [published in the May 4, 2017, issue of \*The New England Journal of Medicine\*](#).

The Phase 2 study was designed to evaluate the efficacy and safety of teprotumumab in patients with recent onset, moderate-to-severe active TED. The primary endpoint was response in the study eye defined as a reduction in Clinical Activity Score of  $\geq 2$  points and reduction of proptosis of  $\geq 2$  mm at week 24. In the intent-to-treat population, 29 of 42 (69%) patients receiving teprotumumab and 9 of 45 (20%) patients receiving placebo were responders at week 24 ( $p < 0.001$ ). The most frequent adverse events reported ( $\geq 5$  percent) were nausea, muscle spasms, dysgeusia, diarrhea, alopecia, hyperglycemia, dry skin, headache, paresthesia, hearing impairment and weight loss. Teprotumumab is an investigational medicine and its safety and efficacy have not been established.

### About Horizon Pharma plc

Horizon Pharma plc is focused on researching, developing and commercializing innovative medicines that address unmet treatment needs for rare and rheumatic diseases. By fostering a growing pipeline of medicines in development and exploring all potential uses for currently marketed medicines, we strive to make a powerful difference for patients, their caregivers and physicians. For us, it's personal: by living up to our own potential, we are helping others live up to theirs. For more information, please visit [www.horizonpharma.com](http://www.horizonpharma.com). Follow [@HZNPplc](#) on Twitter, like us on [Facebook](#) or explore career opportunities on [LinkedIn](#).

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