Takeda Pharma Vertrieb GmbH & Co. KG

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Takeda Receives Positive CHMP Opinion Recommending Approval of Lanadelumab for Routine Prevention of Recurrent Attacks of Hereditary Angioedema (HAE) in Patients Aged 2 years and Older

Zurich (ots/PRNewswire) -

- ***If Approved, Lanadelumab Will be the First Long-Term Prophylactic Treatment of HAE Available in the EU for Patients Under the Age of Six.***
- ***Positive Opinion Based on Pivotal Study SHP643-301, Evaluating the Safety Profile and Pharmacokinetics (PK) of Lanadelumab, in Combination with Extrapolation of Data from the Pivotal Adult and Adolescent Study DX-2930-03.***
- ***HAE is a Rare, Genetic Disorder Estimated to Affect About 1 in 10,000 to 1 in 50,000 People Worldwide. The Condition Results in Recurring Attacks of Oedema (Swelling) in Various Parts of the Body that can be Debilitating and Painful.[1],[2],[3]***

Takeda (TSE:4502) (NYSE:TAK) today announced the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended the approval of lanadelumab (trade name TAKHZYRO®) for the routine prevention of Hereditary Angioedema (HAE) in patients aged 2 years and older. If approved, lanadelumab will be the first long-term prophylactic treatment available in the EU for patients under the age of six.[4],[5],[6] The European Commission (EC) will consider the CHMP positive opinion and decide upon potential marketing authorization in the coming months. Lanadelumab is currently indicated for the routine prevention of recurrent attacks of HAE in patients aged 12 years and older.[7]

HAE attacks, which can involve serious and severely debilitating swelling in the abdomen, face, feet, genitals, hands and throat, may occur very early in childhood.[8] Potentially fatal upper airway angioedema has been reported in patients as young as 3 years old.[9] HAE diagnosis can take an average of 8.4 years after symptom onset.2

"We are so pleased to achieve this positive step towards providing the first long-term prophylactic treatment option to prevent attacks in this vulnerable population," said Didier Relin, Head of International Regulatory at Takeda. "We know that HAE can be a complex, debilitating condition, and we are committed to being a champion for all individuals living with HAE."

The positive opinion is supported by data from Phase 3 Study SHP643-301, also known as the SPRING study, a multicenter, open-label Phase 3 study to evaluate the safety profile and pharmacokinetics (PK) of lanadelumab in patients 2 to <12 years of age. The safety profile was consistent with that seen in the clinical program for patients 12 years of age and older; there were no serious adverse events and no dropouts due to adverse events. The study also successfully reached the secondary objective evaluating the clinical activity/outcome of lanadelumab in preventing hereditary angioedema (HAE) attacks as well as characterizing the pharmacokinetics of lanadelumab in paediatric patients 2 to <12 years of age.[10]

In February, the U.S. Food and Drug Administration (FDA) approved the supplemental Biologics License Application (sBLA) for the expanded use of TAKHZYRO® (lanadelumab-flyo) for prophylaxis to prevent attacks of hereditary angioedema (HAE) in paediatric patients 2 to <12 years of age.[11]

Notes to Editors

About HAE

Hereditary angioedema (HAE) is a rare genetic disorder that results in recurring attacks of oedema – swelling – in various parts of the body, including the abdomen, face, feet, genitals, hands and throat. The swelling can be debilitating and painful.1 Attacks that obstruct the airways can cause asphyxiation and are potentially life threatening.2 HAE affects an estimated 1 in 50,000 people worldwide.3 It is often under recognized, under diagnosed and under treated.3

HAE, like so many other rare diseases, is highly complex, and patients, their families and caregivers often undergo years of strain trying to understand their disease, get a definitive diagnosis and gain access to the medicines they need. At Takeda we are a committed champion for the patients we serve. Every individual living with HAE is unique and by listening and reacting to their needs, we translate the insights we gain into innovative solutions – from diagnosis to ongoing management. Advancing the science is crucial to the way we operate and we are bold in our mission to accelerate diagnosis and develop treatments that will make a difference to the lives of HAE patients, their support networks and those medical professionals who care for them.

About Lanadelumab (TAKHZYRO®)

Lanadelumab is a fully human monoclonal antibody that specifically binds and decreases plasma kallikrein and is indicated for routine prevention of recurrent attacks of HAE in patients aged 12 years and older. It was studied in one of the largest prevention studies in HAE with the longest active treatment duration, and Lanadelumab consistently demonstrated HAE attack reduction. Lanadelumab is formulated for subcutaneous administration and has a half-life of approximately two weeks. Lanadelumab is intended for self-administration or administration by a caregiver once trained by a healthcare professional.4

About Study SHP643-301 (SPRING Study)

SHP643-301 is A Multicenter, Open-Label Phase 3 Study to Evaluate Safety, PK, Pharmacodynamics, And Clinical Activity/Outcomes of TAKHZYRO for Prevention Against Acute Attacks of HAE in Pediatric Patients 2 To <12 Years of Age. The safety profile was consistent with that seen in the clinical program for patients 12 years of age and older; there were no serious adverse events and no dropouts due to adverse events. The study also successfully reached the secondary objective evaluating the clinical activity/outcome of TAKHZYRO in preventing hereditary angioedema (HAE) attacks as well as characterizing the pharmacodynamics of TAKHZYRO in paediatric patients 2 to <12 years of age.10

About Takeda Pharmaceutical Company

Takeda is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to discover and deliver life-transforming treatments, guided by our commitment to patients, our people and the planet. Takeda focuses its R&D efforts on four therapeutic areas: Oncology, Rare Genetics and Hematology, Neuroscience, and Gastroenterology (GI). We also make targeted R&D investments in Plasma-Derived Therapies and Vaccines. We are focusing on developing highly innovative medicines that contribute to making a difference in people's lives by advancing the frontier of new treatment options and leveraging our enhanced collaborative R&D engine and capabilities to create a robust, modality-diverse pipeline. Our employees are committed to improving quality of life for patients and to working with our partners in health care in approximately 80 countries and regions.

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References

[1] Maurer, M., Magerl, M., et al. (2022). The international WAO/EAACI guideline for the management of hereditary angioedema—The 2021 revision and update. Allergy, 77(7), 1961–1990. https://doi.org/10.1111/all.15214.

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[4] HAEGARDA® (C1 Esterase Inhibitor Subcutaneous [Human]). Product Characteristics.

[5] CINRYZE® (C1 esterase inhibitor [human]). Product Characteristics.

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[10] Maurer M, Lumry WR, Li H, et al. Efficacy and Safety of Lanadelumab in Pediatric Patients Aged 2 to <12 years With Hereditary Angioedema: Results From the Open-Label, Multicenter Phase 3 SPRING Study. Abstract submitted to European Academy of Allergy and Clinical Immunology Hybrid Congress 2022.

[11] TAKHZYRO®(lanadelumab-flyo) injection. U.S. Prescribing Information.

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