Takeda Pharma Vertrieb GmbH & Co. KG

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The European Commission Approves Label Update for TAKHZYRO® (lanadelumab), Expanding Its Use to a Broader Group of Paediatric Patients with Recurrent Attacks of Hereditary Angioedema (HAE)

Zurich (ots/PRNewswire) -

- ***TAKHZYRO® is the First Routine Prevention Treatment of HAE Approved in the EU for Patients Under the Age of Six.***

- **The therapeutical indication for *TAKHZYRO®* has been extended to patients aged 2 years and older.*1***

- ***Offers a New Preventative Treatment Option for young HAE patients with high unmet need***

Takeda (TSE:4502/NYSE:TAK) today announced the European Commission has approved TAKHZYRO® (lanadelumab) for the routine prevention of recurrent attacks of Hereditary Angioedema (HAE) in patients aged 2 years and older1, expanding its initial approved use and making it the first long-term prophylactic treatment of HAE available in European Economic Area for patients under the age of six.2,3,4

The recently approved extension of the indication in paediatric patients was paired with an additional strength of 150 mg for Lanadelumab solution for injection in pre-filled syringe. TAKHZYRO® 150mg should be used in patients aged 2 years and older and weighing less than 40 kg to prevent angioedema attacks in patients with Hereditary Angioedema (HAE).1

Previously indicated for the routine prevention of recurrent attacks of HAE in patients aged 12 years and older in the EEA5, the update is supported by clinical data from Phase 3 Study SHP643-3016, in combination with extrapolation of data from the pivotal Adult and Adolescent Study DX-2930-037, and by quality data supporting the new 150 mg pre-filled syringe formulation8. Overall, the safety and efficacy of TAKHZYRO® in preventing Hereditary Angioedema (HAE) attacks in paediatric patients aged 2 and above was demonstrated and the benefit risk-balance was considered positive.

The EMA is the second Agency recommending approval in paediatric population 2 years of age and older, following the U.S. Food and Drug Administration's (FDA) approval of the supplemental Biologics License Application (sBLA) in February this year, for the same expanded use of TAKHZYRO® (lanadelumab-flyo).9

"Potentially fatal upper airway angioedema has been reported in patients as young as 3 years old10, presenting an acute unmet need in some of the most vulnerable of HAE patients." said Didier Relin, Head of International Regulatory at Takeda. "With this expanded label, TAKHZYRO® offers a welcomed new preventative treatment option for the paediatric HAE patient population, and one that can be administered at home with the support of a trained caregiver."

Notes to editors:

European Commission Decision number: EMEA/H/C/004806/X/0034/G

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.11 Attacks that obstruct the airways can cause asphyxiation and are potentially life threatening.12 HAE affects an estimated 1 in 50,000 people worldwide.13 It is often under recognised, under diagnosed and under treated.11

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 2 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.5

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® (lanadelumab) 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.6

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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1 https://www.jaci-inpractice.org/article/S2213-2198(23)01019-X/pdf#:~:text=How%20does%20this%20study%20impact,prophylaxis%20in%20this%20age%20group.

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

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

4 Farkas, H., Martinez–Saguer, I., Bork, K., Bowen, T., Craig, T., Frank, M., ... & Zuraw, B. (2017). International consensus on the diagnosis and management of pediatric patients with hereditary angioedema with C1 inhibitor deficiency. Allergy, 72(2), 300-313.

5 European Medicines Agency, TAKHZYRO Summary of Product Characteristics. Available at: http://ec.europa.eu/health/documents/community-register/index_en.htm Last Accessed: November 2023.

6 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.

7 https://www.takeda.com/newsroom/newsreleases/2022/takedas-takhzyro-open-label-phase-3-study-met-its-objectives-in-children-ages-2-to-12-years-of-age-with-hereditary-angioedema/

8 https://www.clinicaltrialsregister.eu/ctr-search/trial/2015-003943-20/results.

9 TAKHZYRO®(lanadelumab-flyo) injection. U.S. Prescribing Information.

10 Bork, K., Hardt, J., Schicketanz, K. H., & Ressel, N. (2003). Clinical studies of sudden upper airway obstruction in patients with hereditary angioedema due to C1 esterase inhibitor deficiency. Archives of Internal Medicine, 163(10), 1229-1235.

11 Mau rer, 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.

12 Banerji, A., Davis, K. H., Brown, T. M., Hollis, K., Hunter, S. M., Long, J., ... & Devercelli, G. (2020). Patient-reported burden of hereditary angioedema: findings from a patient survey in the United States. Annals of Allergy, Asthma & Immunology, 124(6), 600-607.

13 Longhurst, H. J., & Bork, K. (2019). Hereditary angioedema: an update on causes, manifestations and treatment. British Journal of Hospital Medicine, 80(7), 391-398.

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