KalVista Announces FDA Acceptance of New Drug Application for Sebetralstat for Oral On-Demand Treatment of Hereditary Angioedema

Sep 03, 2024

– If approved, sebetralstat will be the first, oral on-demand treatment for HAE –

– FDA PDUFA goal date of June 17, 2025 –

CAMBRIDGE, Mass. & SALISBURY, England--(BUSINESS WIRE)--Sep. 3, 2024-- KalVista Pharmaceuticals, Inc. (NASDAQ: KALV), today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for sebetralstat, a novel, investigational oral plasma kallikrein inhibitor for the on-demand treatment of hereditary angioedema (HAE) attacks in adult and pediatric patients aged 12 years and older. The FDA has set a Prescription Drug User Fee Act (PDUFA) goal date of June 17, 2025. If approved, sebetralstat would be the first oral, on-demand treatment for HAE in adult and pediatric patients aged 12 years and older. The FDA is not currently planning to hold an advisory committee meeting to discuss the application.

"We are thrilled with the FDA's acceptance of our NDA for sebetralstat as it moves us one step closer to bringing a potentially transformative therapy to the HAE community," said Ben Palleiko, Chief Executive Officer at KalVista. "We understand that people living with HAE and their families carry a tremendous burden every day as they don't know when the next attack may occur or if the attack could cause life-threatening consequences. The compelling data included in our NDA package show that sebetralstat has the potential to significantly alter the way people treat and manage their disease. Given that it could be the first, oral on-demand treatment for HAE, we continue to receive strong support and hear a sense of urgency among healthcare providers, advocates, patients and their families for sebetralstat. I am proud of the team at KalVista for their dedication to achieving this milestone and deeply grateful for the support of patients living with HAE, their families, the HAE scientific community, and the HAEA and HAEi patient advocacy organizations."

The NDA submission was supported by previously disclosed results, including data from the KONFIDENT phase 3 clinical trial and ongoing KONFIDENT-S open label extension trial. Sebetralstat met the primary endpoint for its phase 3 trial with both 300 mg and 600 mg formulations achieving the beginning of symptom relief significantly faster than placebo ($p<0.0001$ for 300 mg, $p=0.0013$ for 600 mg) and was well-tolerated, with a safety profile similar to placebo. In KONFIDENT-S, sebetralstat has enabled patients to treat attacks early with a median time from attack onset to treatment of 9 minutes, demonstrated a consistent safety and efficacy profile with KONFIDENT, and included a median time to beginning of symptom relief for laryngeal attacks of 1.3 hours.

KalVista's KONFIDENT-KID clinical trial, designed to evaluate the safety and efficacy of sebetralstat in a pediatric population aged 2-11 years, was initiated ahead of schedule in June 2024 and has since started dosing patients.

In addition to the NDA acceptance, KalVista recently announced that the European Medicines Agency (EMA) validated the submission of the Marketing Authorization Application (MAA) for sebetralstat. KalVista expects to file for approval in the UK, Japan, and other countries later in 2024.

About the KONFIDENT Phase 3 Trial

The KONFIDENT phase 3 clinical trial was a randomized, double blind, 3-way crossover trial evaluating the safety and efficacy of sebetralstat 300 mg and 600 mg versus placebo for the on-demand treatment of HAE in adult and pediatric patients aged 12 years and older. The trial randomized a total of 136 HAE patients from 66 clinical sites across 20 countries, making it the largest clinical trial ever conducted in HAE. In the trial, participants treated each eligible attack with up to two doses of study drug and treated up to three attacks over the course of the study. The trial included type 1 and type 2 HAE patients who had at least two documented HAE attacks in 90 days prior to randomization, including patients receiving long-term prophylaxis.

About the KONFIDENT-S Trial

KONFIDENT-S is an open label extension trial with numerous real-world elements evaluating the long-term safety and efficacy of sebetralstat for on-demand treatment of HAE attacks in adults and pediatric patients aged 12 years and older with HAE Type I or Type II. KalVista plans to transition ongoing participants in the trial to an oral disintegrating tablet (ODT) formulation in Q4 2024 to support a planned 2026 sNDA filing of this additional formulation. If approved, the ODT formulation would provide people living with HAE an alternative, novel option for oral, on-demand treatment.

About the KONFIDENT-KID Trial

KONFIDENT-KID is an open label trial enrolling approximately 24 children aged 2 to 11 years across seven countries in North America, Europe and Asia. KONFIDENT-KID will collect safety, pharmacokinetic, and efficacy data for each patient for up to one year and will feature a proprietary pediatric oral disintegrating tablet (ODT) formulation of sebetralstat. If approved, sebetralstat would be the first oral on-demand therapy for this age group, and only the second FDA-approved on-demand therapy of any type for this population.

About Sebetralstat

Discovered and developed entirely by the scientific team at KalVista, sebetralstat is a novel, investigational oral plasma kallikrein inhibitor for the on-demand treatment of hereditary angioedema (HAE). Sebetralstat received Fast Track and Orphan Drug Designations from the U.S. FDA, as well as Orphan Drug Designation and an approved Pediatric Investigational Plan from the EMA.

About Hereditary Angioedema

Hereditary angioedema (HAE) is a rare genetic disease resulting in deficiency or dysfunction in the C1 esterase inhibitor (C1INH) protein and subsequent uncontrolled activation of the kallikrein-kinin system. People living with HAE experience painful and debilitating attacks of tissue swelling in various locations of the body that can be life-threatening depending on the location affected. All currently approved on-demand treatment options

require either intravenous or subcutaneous administration.

About KalVista Pharmaceuticals, Inc.

KalVista Pharmaceuticals, Inc. is a global pharmaceutical company focused on the development and delivery of oral medicines for diseases with significant unmet need. KalVista announced positive phase 3 data from the KONFIDENT trial for its oral, on-demand therapy, sebetralstat for HAE in February 2024. The Company's NDA for sebetralstat has been accepted by the FDA with a PDUFA goal date of June 17, 2025. In addition, KalVista received validation of its MAA from the EMA in August 2024. KalVista expects to file for approval in the UK, Japan, and other countries later in 2024.

For more information about KalVista, please visit **www.kalvista.com** or follow on social media at **@KalVista** and **LinkedIn**.

Forward-Looking Statements

This press release contains "forward-looking" statements within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "anticipate," "intend," "plan," "goal," "seek," "believe," "project," "estimate," "expect," "strategy," "future," "likely," "may," "should," "will" and similar references to future periods. These statements are subject to numerous risks and uncertainties that could cause actual results to differ materially from what we expect. Examples of forward-looking statements include, among others, timing or outcomes of communications with the FDA, our expectations about safety and efficacy of our product candidates and timing of clinical trials and its results, our ability to commence clinical studies or complete ongoing clinical studies, including our KONFIDENT-S and KONFIDENT-KID trials, and to obtain regulatory approvals for sebetralstat and other candidates in development, the success of any efforts to commercialize sebetralstat, the ability of sebetralstat and other candidates in development to treat HAE or other diseases, and the future progress and potential success of our oral Factor XIIa program. Further information on potential risk factors that could affect our business and financial results are detailed in our filings with the Securities and Exchange Commission, including in our annual report on Form 10-K for the year ended April 30, 2024, our quarterly reports on Form 10-Q, and our other reports that we may make from time to time with the Securities and Exchange Commission. We undertake no obligation to publicly update any forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.

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