For reference purposes only – Japanese version prevails in case of any discrepancy

November 10, 2025

Announcement of the Result of Phase III Clinical Trial of KP-001 in Japan

Kaken Pharmaceutical Co., Ltd. (“Kaken”, head office: Bunkyo-ku, Tokyo; President and Representative Director: Hiroyuki Horiuchi) announced the result for the Phase III clinical trial of KP-001 (Serabelisib) in Japanese patients with refractory vascular malformations.

The clinical trial in Japan is an open-label, uncontrolled, and multi-center trial in patients with refractory vascular malformations (venous malformations, lymphatic malformations, Klippel-Trenaunay Syndrome).
The primary endpoint is the response rate based on the change in target lesion volume measured by MRI at last evaluation point after 24 weeks of treatment. The response rate is percentage of subjects who decreased their target lesion volume by 20% or more. The response rate was statistically significantly higher than response rate predefined on the basis of the natural history of untreated patients. In addition, there were no adverse reactions that could cause major concern in development of KP-001.
Detailed results of this study are planned to be presented at upcoming academic conferences.

We aim to file for marketing authorization in fiscal year 2026, based on the results of the ongoing Phase III long-term administration study in Japan.

About KP-001
KP-001 is an orally available PI3Kα inhibitor under clinical development for refractory vascular malformations. KP-001 potently and selectively inhibits PI3Kα and exhibits “anti-angiogenesis effects” in *in vitro* and *in vivo* preclinical studies. It is expected to improve quality of life of patients with high unmet medical needs and limited effective treatment.

About vascular malformation
Vascular malformation is the general term for diseases that cause pain, ulcers, growth disorders and disfunction of extremity, and cosmetic problems due to vein and lymphatic vessel morphological abnormality. There are several types of disease, venous malformation, lymphatic malformation, capillary malformation, arteriovenous malformation and combined vascular malformation. Treatment period can sometimes be prolonged depending on the location, size, and symptoms. If a vascular malformation shows poor reaction to standard care and inoperableness etc., it is called refractory vascular malformation.

Cautionary notes regarding forward-looking statement

This release contains forward-looking statements on the Kaken group's business. They are projections based on information available at the time this release was prepared, and may differ from actual results due to a variety of factors. In addition, although this release includes information related to pharmaceutical products (including those under development), these statements are not intended to be advertisement or medical advice.