FDA clears Pharming's Joenja for young children with immune disorder

The FDA on Friday expanded the label of Joenja (leniolisib), an oral, selective PI3Kδ inhibitor developed by Pharming to treat activated phosphoinositide 3-kinase delta syndrome (APDS). Previously cleared for APDS patients 12 and older, Joenja is now approved for children between the ages of 4 and 11 with the rare and progressive primary immunodeficiency who weigh at least 27 kg.

Pharming expects the treatment to be available next month for paediatric patients in the US, for whom Joenja is the first FDA-approved therapy.

The regulatory nod follows an earlier rejection from the US agency. In February, Pharming received a complete response letter for Joenja, which flagged a problem with one of the analytical methods used for production batch testing. The FDA also raised an issue with the potential for underexposure in lower weight paediatric patients and requested additional pharmacokinetic (PK) data to reassess the proposed doses and confirm exposure levels. The regulator in June accepted Pharming's resubmitted application.

Supporting data

The initial approval, granted in 2023, was based on data from a 12-week Phase II/III trial that evaluated Joenja versus placebo in 31 patients diagnosed with APDS aged 12 years and older. The study met its co-primary endpoints, with Joenja achieving a reduction in lymph node size and a 37% improvement in naïve B cells counts.

The label expansion used data from an open-label, single-arm Phase III study involving eight children with APDS aged 4 to 11 years who received a Joenja dosage based on weight.

Over the trial's 12-week course, patients taking Joenja experienced improvements in naïve B cells counts and reductions in lymph node size. In addition, the PK data demonstrated no clinically significant difference between patients younger or older than 12 years.

No serious adverse events (AEs) were seen in patients who received the PI3Kδ inhibitor, and all treatment emergent AEs were mild to moderate in nature.

Next, Pharming is hoping to win a supplemental approval for a low-dose Joenja in children with APDS who weigh at least 13 kg and less than 27 kg. It submitted an sNDA for the indication on July 30.

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