Pharming announces positive Phase II topline data for leniolisib in PIDs with immune dysregulation accepted as late-breaking abstract at ESID 2026

- **Leniolisib was generally well-tolerated, with clinical improvements across multiple measures of immune dysregulation, including reductions in lymphoproliferation**
- **Separately, Pharming expects to report topline results from a Phase II trial of leniolisib in CVID in Q4 2026**

Leiden, the Netherlands, September 22, 2026: Pharming (Euronext Amsterdam: PHARM/Nasdaq: PHAR), a global biotechnology company focused on rare immune and genetic diseases, today announced that a late-breaking abstract highlighting positive topline data from its Phase II trial with leniolisib in genetically identifiable primary immunodeficiencies (PIDs) with immune dysregulation linked to PI3Kδ signaling has been accepted at the 22nd Biennial Meeting of the European Society for Immunodeficiencies (ESID), which will take place October 14-17, in the Netherlands.

This trial is a single-arm, open-label, intra-patient dose-escalation Phase II study of leniolisib evaluating safety and tolerability, pharmacokinetic, pharmacodynamic and efficacy measures in 13 subjects with genetically defined PIDs. The abstract will highlight leniolisib's favorable safety and tolerability profile, as well as clinical improvements across measures of immune dysregulation. Clinical results included improvements in lymphoproliferative disease, with a mean 26.4% spleen volume reduction (SVR) and reductions in the size of index lesions. The safety observations were consistent with the known safety profile of leniolisib, with infections as the most common events observed in study subjects, and no new safety signals identified. Additional data will be presented at ESID 2026.

Of the 13 patients enrolled in the study, nine also had a diagnosis of common variable immunodeficiency (CVID). Pharming expects to report Phase II results for leniolisib in CVID patients with immune dysregulation, with or without an identified genetic cause, in the fourth quarter of 2026.

"These results mark an important step in assessing leniolisib's potential to address immune dysregulation in PIDs beyond APDS, potentially benefiting a substantially larger patient population," said **Anurag Relan, Chief Medical Officer** of Pharming. "Given PI3Kδ's central role in immune dysregulation mechanisms, these results are encouraging and support ongoing development of leniolisib in PID patients with APDS-like manifestations. We look forward to sharing additional results from this trial at ESID, along with topline results from our separate Phase II trial in CVID, expected in the fourth quarter, which will inform our plans for a potential registrational study in the broader CVID population."

Abstract details:

Title: Single-arm, open-label, Phase 2 study of leniolisib in patients with inborn errors of immunity linked to dysregulated PI3K pathway signaling: Topline safety and efficacy outcomes
Author: Gulbu Uzel, MD

The presentation will be available for viewing at ESID 2026 for the full duration of conference.

About leniolisib

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older in the U.S., U.K., Australia, Israel, the EU, Canada, and South Korea; in children 4 to 11 years of age who weigh at least 27 kg in the U.S., and for patients 4 years of age and older in Japan.

Leniolisib inhibits the production of phosphatidylinositol-3-4-5-trisphosphate, which serves as an important cellular messenger and regulates a multitude of cell functions such as proliferation, differentiation, cytokine production, cell survival, angiogenesis, and metabolism. Results from a randomized, placebo-controlled Phase III clinical trial demonstrated statistically significant improvement in the coprimary endpoints, reflecting a favorable impact on the immune dysregulation and deficiency seen in these patients, and open label extension data has supported the safety and tolerability of long-term leniolisib administration.[1,2]

Leniolisib is currently under regulatory review for the treatment of APDS in several other countries. Leniolisib is also being evaluated in two Phase II clinical trials in primary immunodeficiencies (PIDs) with immune dysregulation. The safety and efficacy of leniolisib has not been established for PIDs with immune dysregulation beyond APDS.

About Pharming

Pharming Group N.V. (Euronext Amsterdam: PHARM/Nasdaq: PHAR) is a global biotechnology company that develops and commercializes innovative medicines for people living with rare immune and genetic diseases.

We combine specialized scientific, medical, regulatory and commercial expertise to advance a focused portfolio of approved medicines and development programs that address significant unmet medical needs. Guided by insights from patients and the wider rare disease community, we are dedicated to delivering innovative therapies for some of the most challenging rare diseases.

Pharming is headquartered in Leiden, the Netherlands, with operations in the United States and Europe.

For more information, visit www.pharming.com and find us on LinkedIn.

Forward-looking Statements

This press release may contain forward-looking statements. Forward-looking statements are statements of future expectations that are based on management's current expectations and assumptions and involve known and unknown risks and uncertainties that could cause actual results, performance, or events to differ materially from those expressed or implied in these statements. These forward-looking statements are identified by their use of terms and phrases such as "aim", "ambition", ''anticipate'', ''believe'', ''could'', ''estimate'', ''expect'', ''goals'', ''intend'', ''may'', "milestones", ''objectives'', ''outlook'', ''plan'', ''probably'', ''project'', ''risks'', "schedule", ''seek'', ''should'', ''target'', ''will'' and similar terms and phrases. Examples of forward-looking statements may include statements with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, and Pharming's expectations regarding its projected working capital requirements and cash resources, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope, progress and expansion of Pharming's clinical trials and ramifications for the cost thereof; and clinical, scientific, regulatory, commercial, competitive and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2025 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2025, filed with the U.S. Securities and Exchange Commission, the events and circumstances discussed in such forward-looking statements may not occur, and Pharming's actual results could differ materially and adversely from those anticipated or implied thereby. All forward-looking statements contained in this press release are expressly qualified in their entirety by the cautionary statements contained or referred to in this section. Readers should not place undue reliance on forward-looking statements. Any forward-looking statements speak only as of the date of this press release and are based on information available to Pharming as of the date of this release. Pharming does not undertake any obligation to publicly update or revise any forward-looking statement as a result of new information, future events or other information.

References

1. Rao VK, et al. Blood. 2023;141(9):971-983.
2. Rao VK, et al. J Allergy Clin Immunol 2024;153:265-74.

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