Pharming Group receives positive CHMP opinion for Joenja® (leniolisib) for the treatment of APDS in adult and pediatric patients 12 years and older

- **If approved, Joenja® (leniolisib) would become the first approved treatment in the European Union for activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS), a rare primary immunodeficiency**
- **Decision based on Phase II/III clinical data demonstrating statistically significant impact on measures of immune dysregulation and immunodeficiency**
- **Final European Commission decision expected in Q2 2026**

Leiden, the Netherlands, March 27, 2026: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending marketing authorization for Joenja® (leniolisib) for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS), a rare primary immunodeficiency, in adult and pediatric patients 12 years of age and older.

A final decision by the European Commission on the marketing authorization for Joenja under exceptional circumstances is expected within approximately two months. If approved, Joenja would become the first approved treatment for APDS in the European Union. The centralized marketing authorization would be valid in all 27 European Union Member States, as well as Norway, Iceland and Liechtenstein.

Fabrice Chouraqui, Chief Executive Officer of Pharming, commented:
"For patients living with APDS, there remains a significant unmet medical need. By targeting the underlying cause of the disease, Joenja could mark a step-change in APDS care in Europe. Today's positive CHMP opinion, together with the approval in Japan earlier this week, reflects the strength of the clinical data and the dedication of the patients, families, and physicians who participated in the clinical studies. We look forward to the European Commission's final decision and to working with relevant authorities across Europe to support patient access."

The positive CHMP opinion is based on results from a multinational, triple-blind, placebo-controlled, randomized Phase II/III clinical trial, which evaluated efficacy and safety in 31 patients diagnosed with APDS aged 12 years and older and demonstrated a statistically significant impact on immune dysregulation and immunodeficiency. Also submitted as part of the application were data from a long-term, open-label extension clinical trial in which 37 patients received leniolisib for a median of three years.

Virgil Dalm, MD, PhD, Head of Division of Allergy & Clinical Immunology of the Department of Internal Medicine and Principal Investigator, Erasmus University Medical Center, Rotterdam, the

Netherlands, commented:

"The clinical program for leniolisib has shown consistent, meaningful improvements across markers of both immune dysregulation and immune deficiency in patients with APDS. Taken together with a favorable safety profile, these results suggest Joenja could meaningfully change the clinical outlook for people living with this rare, complex and progressive inborn error of immunity. As a physician and investigator in the clinical program, I know how important it is to have treatment options that address the underlying cause of disease and have the potential to make a meaningful difference for patients and their families. I am proud to see the dedication of investigators and the patients who participated in the studies contribute to this milestone, and I look forward to the European Commission's decision and the potential for patients across Europe to access this novel targeted therapy."

Joenja is approved and marketed in the United States and the United Kingdom for patients aged 12 years and older with APDS.

About Activated Phosphoinositide 3-Kinase δ Syndrome (APDS)

APDS is a rare primary immunodeficiency that was first characterized in 2013. APDS is caused by variants in either one of two identified genes known as *PIK3CD* or *PIK3R1*, which are vital to the development and function of immune cells in the body. Variants of these genes lead to hyperactivity of the PI3Kδ (phosphoinositide 3-kinase delta) pathway, which causes immune cells to fail to mature and function properly, leading to immunodeficiency and dysregulation[1,2,3] APDS is characterized by a variety of symptoms, including severe, recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy.[4,5] Because these symptoms can be associated with a variety of conditions, including other primary immunodeficiencies, it has been reported that people with APDS are frequently misdiagnosed and suffer a median 7-year diagnostic delay.[6] As APDS is a progressive disease, this delay may lead to an accumulation of damage over time, including permanent lung damage and lymphoma.[4-7] A definitive diagnosis can be made through genetic testing. APDS affects approximately 1 to 2 people per million worldwide.[8]

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 the U.S., U.K., Australia and Israel in adult and pediatric patients 12 years of age and older and in Japan for patients 4 years of age and older. 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.[9,10]

Leniolisib is currently under regulatory review for the treatment of APDS in Canada and 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 Group N.V.

Pharming Group N.V. (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) is a global biopharmaceutical company dedicated to transforming the lives of patients with rare, debilitating, and life-threatening diseases. We are developing and commercializing a portfolio of innovative medicines, including small molecules and biologics. Pharming is headquartered in Leiden, the Netherlands, with a significant proportion of its employees based in the U.S.

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 2024 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2024, 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.

Inside Information

This press release relates to the disclosure of information that qualifies, or may have qualified, as inside information within the meaning of Article 7(1) of the EU Market Abuse Regulation.

References

1. Lucas CL, et al. Nat Immunol. 2014;15(1):88-97.
2. Elkaim E, et al. J Allergy Clin Immunol. 2016;138(1):210-218.
3. Nunes-Santos C, Uzel G, Rosenzweig SD. J Allergy Clin Immunol. 2019;143(5):1676-1687.
4. Coulter TI, et al. J Allergy Clin Immunol. 2017;139(2):597-606.
5. Maccari ME, et al. Front Immunol. 2018;9:543.
6. Jamee M, et al. Clin Rev Allergy Immunol. 2020 Dec;59(3):323-333.
7. Condliffe AM, Chandra A. Front Immunol. 2018;9:338.
8. Vanselow S, et al. Frontiers in Immunology. 2023;14:1208567.
9. Rao VK, et al Blood. 2023 Mar 2;141(9):971-983.
10. Rao VK, et al. J Allergy Clin Immunol 2024;153:265-74.

For further public information, contact:

Investor Relations

Michael Levitan, VP Investor Relations & Corporate Communications

T: +1 (908) 705 1696

E: investor@pharming.com

Media Relations

Global: Saskia Mehring, Corporate Communications Manager

T: +31 6 28 32 60 41

E: media.relations@pharming.com

U.S.: Ethan Metelenis (Precision AQ on behalf of Pharming)

T: +1 (917) 882-9038

Netherlands: Leon Melens (LifeSpring Life Sciences Communication on behalf of Pharming)

T: +31 6 53 81 64 27