For media and investors only

Pharming Group to host webcast on findings of a new study published in *Cell* advancing functional classification of variants of uncertain significance (VUS) to improve APDS diagnosis

- **Researchers identified variants which may cause activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)**
- **Results enable clinical genetic testing labs to appropriately reclassify VUSs, accelerating the path to a definitive APDS diagnosis for many patients**
- **Findings reveal APDS may be more prevalent than previously estimated**
- **Webcast to take place on Monday, June 30, 2025, at 16:30 CEST / 10:30 EDT**

Leiden, the Netherlands, June 24, 2025: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) announces it will host a webcast for investors and analysts featuring Joshua Milner, MD, an internationally renowned immunologist, to discuss the findings of a recent study published in the peer-reviewed journal *Cell*.

The study titled "Scalable generation and functional classification of genetic variants in inborn errors of immunity for improved clinical diagnosis and management" was led by Zachary Walsh, MD/PhD candidate, Dr. Milner and Benjamin Izar, MD, PhD of Columbia University. The publication details significant advances in diagnosing inborn errors of immunity, also known as primary immune disorders. The researchers' approach helps resolve a major limit to interpretation of genetic testing that often yields variants of uncertain significance (VUS) when evaluating such disorders, including activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS).

There are currently over 1,300 known U.S. patients with a VUS in the PIK3CD and PIK3R1 genes implicated in APDS. The team at Columbia introduced more than 2,000 PIK3CD/PIK3R1 variants, representing a portion of all potential variants, into human T-cell lines and assessed PI3Kδ pathway activity. These studies successfully confirmed known disease-causing APDS variants and, importantly, also identified over 100 new variants with evidence for PI3Kδ pathway hyperactivity. By analyzing very large datasets of patients who agreed to have their genetic testing linked to their medical records, the research team at Columbia concludes that the real prevalence of APDS may be higher than previously estimated.

During the call, Dr. Milner will provide insights into the study's methodology, key findings, implications, and next steps. Anurag Relan, Pharming Chief Medical Officer, will lead a Q&A session with Dr. Milner and discuss next steps to collaborate with genetic testing laboratories on their VUS

reclassification efforts, extend the study to assess additional variants, and further investigate the clinical phenotype of APDS in the newly identified variants.

Anurag Relan, MD, MPH, Chief Medical Officer of Pharming, commented:

"This important study, recently published in Cell, is a key step towards providing answers to patients with a VUS in the PIK3CD or PIK3R1 genes. The study highlights the importance of increased genetic screening and awareness to ensure timely diagnosis of APDS. We expect these data to enable clinical genetic testing laboratories to reclassify a portion of the VUSs, accelerating the path to a definitive APDS diagnosis for many patients. We look forward to additional near-term studies to facilitate the reclassification of the remaining VUSs and to further exploring the prevalence and phenotype of this rare disease."

Dr Joshua Milner, MD, Director Division of Pediatric Allergy, Immunology and Rheumatology at Columbia University Irving Medical Center, commented:

"This study offers a powerful new lens for interpreting VUSs and uncovering therapeutic insights in conditions like APDS. We hope these findings will support clinicians in making more informed decisions and ultimately lead to better outcomes for patients navigating rare immune disorders."

Note: This study was supported by a National Institutes of Health (NIH)/National Cancer Institute (NCI) grant and was in part supported through a sponsored research agreement with Pharming.

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To participate, please register at https://edge.media-server.com/mmc/p/q6x83at2. Questions can be submitted in advance, via email to investor@pharming.com.

For more information and to access the full peer-reviewed study in *Cell*, please visit https://www.cell.com/cell/abstract/S0092-8674(25)00624-5.

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.

About leniolisib

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved in the U.S., U.K., Australia and Israel 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. 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 interim open label extension data has supported the safety and tolerability of long-term leniolisib administration.[8,9] Leniolisib is currently under regulatory review in the European Economic Area, Canada and several other countries for APDS, with plans to pursue regulatory approval in Japan. Leniolisib is also being evaluated in two Phase III clinical trials in children with APDS and 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 commercializing and developing a portfolio of innovative medicines, including small molecules and biologics. Pharming is headquartered in Leiden, the Netherlands, and has employees around the globe who serve patients in over 30 markets in North America, Europe, the Middle East, Africa, and Asia-Pacific.

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.

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