

Pharming Group announces presentations at International Primary Immunodeficiencies Congress and the American College of Allergy, Asthma & Immunology Annual Scientific Meeting

Leiden, The Netherlands, November 7, 2023: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) announces 14 poster presentations by the Company or its collaborators regarding leniolisib and RUCONEST® at the International Primary Immunodeficiencies Congress (IPIC) and the American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting in November.

IPIC, Rotterdam, The Netherlands, November 8 - 10, 2023

- Approaches to family testing for autosomal dominant inborn errors of immunity in Europe: results of a blinded survey; Poster #204: Thursday, November 9, 08:00–08:30 CET
- Results of a second interim analysis of an ongoing single-arm open-label extension study of leniolisib in activated PI3K delta syndrome: long-term efficacy and safety through to March 2023; Poster #214: Thursday, November 9, 10:00–10:30 CET
- Qualitative international study to explore the symptoms and health-related quality of life impact of activated phosphoinositide 3-kinase delta syndrome (APDS): interim findings; Poster #152: Friday, November 10, 15:15–15:45 CET
- A randomised, placebo-controlled Phase III trial of leniolisib in activated PI3Kδ syndrome: adult versus adolescent subgroup analysis; Poster#213
- Gastrointestinal manifestations in patients with activated PI3K delta syndrome (APDS) treated with leniolisib; Poster #133
- Assessing long-term treatment with leniolisib and its effects on bronchiectasis in patients with activated PI3K delta syndrome (APDS); Poster #148
- Treatment with the selective PI3K inhibitor leniolisib in an atypical case of activated PI3K delta syndrome (APDS); Poster #194
- Utilizing proxy diseases to model activated PI3K delta syndrome (APDS) healthcare utilization and outcomes; Poster #151
- The PIDCAP Project: Developing A Warning-Sign-Based Algorithm For Use In An Electronic Health Record Screening Tool For Inborn Errors Of Immunity Screening; Poster #85
- Complicated course of activated PI3Kδ syndrome-1 (APDS-1) ameliorated by leniolisib: a case study; Poster #264

All posters are displayed for the duration of the congress. Dates and times for posters included in the 'Guided Poster Walk' mini oral presentations are listed above.



ACAAI, Anaheim, California, U.S, November 9 – 13, 2023

APDS

- Mortality in Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome, a Systematic Literature Review; Poster #P165: Presented Saturday, November 11, 2023 at 12:20 PT, Anaheim Convention Center Exhibit Hall
- Overall survival among patients with activated phosphoinositide 3-kinase delta syndrome;
 Poster #P166: Presented Saturday, November 11, 2023 at 12:35 PT, Anaheim Convention
 Center Exhibit Hall

RUCONEST®

- Managing primary immunodeficiency immunoglobulin replacement therapy-related adverse events: Prophylaxis with recombinant human C1 esterase inhibitor; Poster #M234: Presented Friday, November 10, 2023 at 15:00 PT, Anaheim Convention Center Exhibit Hall
- On-demand treatment for hereditary angioedema attacks: Role of individualized management strategy; Poster #M150: Presented November 12, 2023 at 12:15 PT, Anaheim Convention Center Exhibit Hall

In addition to displaying in the exhibit hall at the noted times, ePosters will be accessible online and on demand to registered attendees on Thursday, November 9, 2023, beginning at 08:00 PT on ACAAI's website.

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 (PI3K δ) inhibitor approved in the US as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3K δ) 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 II/III clinical trial demonstrated clinical efficacy of leniolisib in the



coprimary endpoints; demonstrating statistically significant impact on immune dysregulation and normalization of immunophenotype within these patients, and interim open label extension data has supported the safety and tolerability of long-term leniolisib administration. Leniolisib is currently under regulatory review by the European Medicines Agency, with plans to pursue further regulatory approvals in the UK, Canada, Australia and Japan. Leniolisib is also being evaluated in a Phase III clinical trial in children aged 4 to 11 with APDS, with a further trial planned in children aged 1 to 6 years with 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. Pharming is commercializing and developing an innovative portfolio of protein replacement therapies and precision medicines, including small molecules, biologics, and gene therapies that are in early to late-stage development. Pharming is headquartered in Leiden, 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 <u>www.pharming.com</u> and find us on <u>LinkedIn</u>.

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 and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2022 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2022, 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.

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

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- 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. 2019; May 21.
- 7. Condliffe AM, Chandra A. Front Immunol. 2018;9:338.
- 8. RAO VK, et al Blood. 2023 Mar 2;141(9):971-983

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