For media and investors only

Pharming receives positive recommendation from NICE for Joenja® ▼(leniolisib) as a treatment for APDS

Joenja® is the first and only medicine specifically for APDS to be reimbursed within the NHS

Recommended for adult and pediatric patients 12 years of age and older with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)

Leiden, the Netherlands, April 23, 2025: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) announces that the National Institute for Health and Care Excellence (NICE) has issued positive final guidance recommending Joenja® (leniolisib) for reimbursement and use within the National Health Service (NHS) in England and Wales for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older.

The NICE recommendation is based on the totality of the data, including the Phase III clinical trial evaluating leniolisib, an oral selective PI3Kδ inhibitor, in patients with APDS, a rare and progressive primary immunodeficiency. In the primary analysis, treatment with leniolisib led to significant improvements in immune deficiency and immune dysregulation compared to placebo. The Phase III trial demonstrated an improvement in clinical outcomes, which was maintained during longer-term treatment within the open-label extension trial.

***Fabrice Chouraqui, Chief Executive Officer of Pharming, said*:**
"This positive recommendation for Joenja® *from NICE is a significant milestone for patients living with APDS in England and Wales. It underscores Pharming's commitment to work closely with regulatory and reimbursement authorities, the medical immunology community, and patient associations to bring this first targeted treatment to patients who may benefit."*

***Professor Sinisa Savic, Professor of Clinical Immunology at St James's University Hospital, said*:**
"Data from clinical trials demonstrate that leniolisib provides real benefits for patients with APDS, a rare primary immunodeficiency. APDS is a complex and progressive condition that leads to immune dysregulation and recurrent infections, significantly impacting patients' quality of life. The recommendation by NICE, which allows this targeted therapy to be prescribed on the NHS, marks an important step forward in improving treatment options for individuals with this condition."

***Dr Susan Walsh, Chief Executive Officer at Immunodeficiency UK, said*:**
"This is a hugely welcomed decision that will make a massive difference to the lives of people with APDS, and their families, by potentially allowing them to have a better quality of life. With leniolisib,

we now have a targeted treatment available that addresses the fundamental cause of the immune system problems experienced in APDS. This is a huge leap forward. We are grateful to Pharming for working with NICE to make this drug available via the NHS. Together, they have shown a real commitment to providing people affected by ultra-rare, immune system conditions the treatments they need."

The final NICE recommendation aligns with the U.K. Medicines and Healthcare products Regulatory Authority (MHRA) approval and falls under NICE's Highly Specialised Technologies (HST) pathway, which evaluates treatments for very rare and severe diseases. Leniolisib is now available for use and funded in England through the Innovative Medicines Fund, ensuring immediate patient access. In Wales, leniolisib is expected to be funded within the next three months through the NHS in specialist centers.

Important Safety Information

The full Summary of Product Characteristics (SPC/SmPC) for Joenja® (leniolisib) is available on the MHRA website at https://products.mhra.gov.uk/.

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 Joenja® (leniolisib)

Joenja® (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. Joenja® 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.

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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