Pharming Group announces 2026 financial guidance and highlights rare disease pipeline at Investor Day

- **Highlights advancing clinical-stage pipeline, including two major value-creating programs for primary immunodeficiencies (PIDs) with immune dysregulation and mtDNA-driven mitochondrial disease**
- **Introduces napazimone (KL1333) as the compound name for the mtDNA-driven mitochondrial disease program**
- **2026 total revenue guidance of US$405 – US$425 million (8% to 13% growth)**
- **2026 total operating expense guidance of US$330 – US$335 million**
- **Investor Day will be webcast today starting at 10:00 am EST (16:00 CET)**

Leiden, The Netherlands, February 3, 2026: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) today announced 2026 financial guidance and will highlight its advancing clinical-stage rare disease pipeline, including two major value-creating programs, at a virtual Investor Day taking place later today.

The Investor Day will focus on the scientific rationale, clinical strategy, and long-term opportunity underpinning Pharming's pipeline programs in primary immunodeficiencies (PIDs) with immune dysregulation and mitochondrial DNA (mtDNA)-driven mitochondrial disease.

Fabrice Chouraqui, Chief Executive Officer of Pharming, commented:
"Pharming's vision of developing into a leading global rare disease company is grounded in the strength of our two commercial products and our high-value pipeline. We expect our commercial portfolio to continue delivering robust growth, with total revenues between US$405 million and US$425 million in 2026. We are excited to showcase the breadth and quality of our pipeline at today's Investor Day. Leniolisib and napazimone (KL1333) are being developed for large, underserved rare disease populations with significant unmet need, and are supported by a strong and growing body of biological and clinical evidence. We believe both programs offer substantial long-term value-creating potential for Pharming."

2026 financial guidance:

- Total revenues between US$405 million and US$425 million (8% to 13% growth), driven by significant and accelerating growth for Joenja® and continued growth for RUCONEST®
- Total operating expenses between US$330 million and US$335 million, with the increase driven primarily by an increase in Research & Development expenses related to the ongoing leniolisib Phase II clinical trials and the napazimone (KL1333) pivotal clinical trial

Pipeline overview:

Leniolisib – primary immunodeficiencies with immune dysregulation
Pharming management will outline the scientific and clinical rationale supporting the expansion of leniolisib into broader patient populations with primary immunodeficiencies (PIDs) with immune dysregulation, beyond its currently approved indication. Two Phase II proof-of-concept clinical trials are ongoing — one in genetically defined PIDs linked to PI3K signaling and one in common variable immunodeficiency (CVID) with immune dysregulation — with top-line data for both trials expected in the second half of 2026.

Leniolisib is an oral, selective phosphoinositide 3-kinase delta (PI3Kδ) inhibitor that is currently approved and marketed as Joenja in the United States, and marketed and/or approved in additional countries, as the first and only targeted treatment for activated PI3Kδ syndrome (APDS)[1], a rare and progressive primary immunodeficiency, for patients 12 years of age and older. APDS represents a genetically defined form within the broader CVID spectrum and serves as a clinically validated proof-of-concept for targeting PI3Kδ-driven immune dysregulation, supporting the potential applicability of leniolisib across significantly broader PID and CVID patient populations.

Napazimone (KL1333)– mtDNA-driven mitochondrial disease
Pharming management will outline the scientific and clinical rationale for napazimone (KL1333), the newly named compound being developed for mtDNA-driven mitochondrial disease, which has the potential to become the first standard of care in this setting, if approved. The pivotal FALCON clinical trial is ongoing and remains on track for a readout in 2027.

Napazimone (KL1333) is an investigational therapy being developed for adult patients with primary mitochondrial disease caused by mitochondrial DNA (mtDNA) mutations — a rare and debilitating condition characterized by impaired energy production, significant fatigue and muscle weakness (myopathy), and reduced life expectancy.

Clinical expert perspectives
The Investor Day will feature presentations from leading clinical experts, providing context on disease biology, unmet medical need, and the potential role of Pharming's programs.

- Jocelyn Farmer, MD, PhD, Lahey Hospital & Medical Center — an internationally recognized authority on CVID and immune dysregulation
- Amel Karaa, MD, Massachusetts General Hospital, Harvard Medical School — an internationally recognized authority on mitochondrial medicine

[1] FDA press release published on March 24, 2023, titled FDA approves first treatment for activated phosphoinositide 3-kinase delta syndrome. Available via: https://www.fda.gov/drugs/news-events-human-drugs/fda-approves-first-treatment-activated-phosphoinositide-3-kinase-delta-syndrome

Investor Day webcast information:
The Investor Day event will be held today, Tuesday, February 3, from 10:00 a.m. to 12:00 p.m. EST (16:00 to 18:00 CET). To register for and view the live event, please visit: https://www.pharming.com/pharming-investor-day-2026.

A replay will be available on the Pharming.com website shortly after the conclusion of the event.

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.

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