Pharming Group announces upcoming presentations at the 2025 Annual Meeting of the Clinical Immunology Society (CIS)

Leiden, the Netherlands, April 25, 2025: Pharming Group N.V. ("Pharming" or "the Company") (Euronext Amsterdam: PHARM/Nasdaq: PHAR) announces the following presentations by the Company or its collaborators at the 2025 Annual Meeting of the Clinical Immunology Society (CIS), taking place May 1-4, 2025 in Philadelphia, PA. These presentations include Phase III clinical data for leniolisib for pediatric patients aged 4-11 years with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) and insights into APDS and additional primary immunodeficiencies (PIDs) with immune dysregulation.

Presentations:

Title: *Primary and Safety Outcomes of a Phase 3 Open-Label, Single-Arm, 12-Week Study of Treatment With PI3Kδ Inhibitor Leniolisib in Pediatric Patients Aged 4-11 Years With Activated PI3Kδ Syndrome (APDS)*
Presenting Author: Shanmuganathan Chandrakasan, MD, Division of Bone Marrow Transplant, Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta, Emory University School of Medicine, Atlanta, GA, USA
Session Type: Poster
Session Date/Time: Friday, May 2, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 70

Title: *The Impact of Immune Dysregulation on Clinical Outcomes in Common Variable Immunodeficiency: A Systematic Literature Review*
Presenting Author: Jocelyn R. Farmer, MD, PhD, Department of Medicine, UMass Chan Medical School, Worcester, MA, USA, and Clinical Immunodeficiency Program of Beth Israel Lahey Health, Division of Allergy and Immunology, Lahey Hospital & Medical Center, Burlington MA, USA
Session Type: Poster
Session Date/Time: Saturday, May 3, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 30

Title: *Defining an activated PI3K-delta syndrome-like endotype within broader common variable immunodeficiency*
Presenting Author: Jocelyn R. Farmer, MD, PhD, Department of Medicine, UMass Chan Medical School, Worcester, MA, USA, and Clinical Immunodeficiency Program of Beth Israel Lahey Health, Division of Allergy and Immunology, Lahey Hospital & Medical Center, Burlington MA, USA
Session Type: Poster
Session Date/Time: Friday, May 2, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 32

Title: *Leniolisib, a PI3Kδ inhibitor, improves lymphoproliferative disease in a murine model of autoimmune lymphoproliferative syndrome (ALPS-FAS)*
Presenting Author: Kevin Thorneloe, PhD, Pharming Healthcare, Inc., Warren, NJ, USA
Session Type: Poster
Session Date/Time: Saturday, May 3, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 37

Title: *Demographic and clinical characteristics of people with activated phosphoinositide 3-kinase delta syndrome (APDS) in the APDS-Characterization and Clinical Outcomes Immunologic Registry (APDS-CHOIR)*
Presenting Author: Nicholas Hartog, MD, Cornwell Health Pediatric Allergy and Immunology, Grand Rapids, MI, USA
Session Type: Poster
Session Date/Time: Friday, May 2, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 49

Title: *Patient insights expand understanding of APDS*
Presenting Author: Kristie Cline, MBA, Pharming Healthcare, Inc., Warren, NJ, USA
Session Type: Poster
Session Date/Time: Saturday, May 3, 2025, 1:30-2:30 pm (ET)
Abstract/Poster Number: 40

The effectiveness and safety of leniolisib for pediatric patients aged 4-11 years with APDS has not been reviewed or approved by a regulatory authority. Preclinical data in ALPS-FAS may not be predictive of clinical results.

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.[1,2] 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.

References

1. Rao VK, et al Blood. 2023 Mar 2;141(9):971-983.
2. Rao VK, et al. J Allergy Clin Immunol 2024;153:265-74.

For further public information, contact:

Pharming Group, Leiden, the Netherlands
Michael Levitan, VP Investor Relations & Corporate Communications
T: +1 (908) 705 1696
E: investor@pharming.com

FTI Consulting, London, UK
Simon Conway/Alex Shaw/Amy Byrne
T: +44 203 727 1000

LifeSpring Life Sciences Communication, Amsterdam, the Netherlands
Leon Melens
T: +31 6 53 81 64 27
E: pharming@lifespring.nl

US PR
Christina Skrivan
T: +1 (636) 352-7883
E: Christina.Skrivan@precisionaq.com