Pharming Group announces presentations at CIS 2026 Annual Meeting, including leniolisib pediatric data in APDS and clinical experience in CVID and related disorders

Leiden, the Netherlands, May 7, 2026: Pharming Group N.V. ("Pharming" or "the Company") (Euronext Amsterdam: PHARM/Nasdaq: PHAR) today announced presentations at the 2026 Annual Meeting of the Clinical Immunology Society (CIS), taking place May 6-9 in New Orleans, LA.

Across multiple presentations, the Company and its collaborators will share interim outcomes of a long-term extension study for leniolisib in pediatric patients aged 4 to 11 years with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS). The presentations will also include clinical data from expanded access use of leniolisib to treat immune dysregulation in patients with Common Variable Immunodeficiency (CVID) and CVID-like disorders, as well as further insight into APDS and additional primary immunodeficiencies (PIDs) with immune dysregulation.

Two phase II clinical trials (NCT06897358 and NCT06549114) are currently underway to formally evaluate the safety and tolerability of leniolisib in patients with CVID and PIDs with immune dysregulation beyond APDS.

Anurag Relan, Chief Medical Officer of Pharming, commented:

"*We are pleased to present data at CIS that advance the understanding of APDS and other primary immunodeficiencies characterized by immune dysregulation. The clinical experience observations support the ongoing clinical trials evaluating leniolisib in CVID and CVID-like disorders, and we look forward to results in the second half of this year.*"

Presentations:

Title: *Interim Safety and Efficacy Outcomes of an Open-Label Long-Term Extension 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 8, 2026, 11:00 – 11:30 am (EDT)
Abstract/Poster Number: 218

Title: *Clinical Experience with Use of the PI3Kδ Inhibitor Leniolisib to Treat Immune Dysregulation in Patients with CVID and CVID-Like Disorders*
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: Thursday, May 7, 2026, 1:30 -2:30 pm (EDT)
Abstract/Poster Number: 42

Title: *A Phase 2 Clinical Study of Leniolisib in Primary Immunodeficiencies with Enhanced PI3K Pathway Signaling: Study Design & Subject Baseline Characteristics*
Presenting Author: Gulbu Uzel, MD, National Institute of Allergy and Infectious Diseases, National Institutes of Health, Bethesda, MD, USA
Session Type: Poster
Session Date/Time: Friday, May 8, 2026, 2:10-3:10 pm (EDT)
Abstract/Poster Number: 194

Title: *Pediatric and Adolescent Activated Phosphoinositide 3-kinase Delta Syndrome (APDS): Demographic and Clinical Findings from the APDS-Characterization and Clinical Outcomes Immunologic Registry (APDS-CHOIR)*
Presenting Author: Kelli Williams, MD, MPH, Department of Pediatrics, Medical University of South Carolina, Charleston, SC, USA
Session Type: Poster
Session Date/Time: Thursday, May 7, 2026, 1:30-2:30 pm (EDT)
Abstract/Poster Number: 86

Title: *Caregiver- and Clinician-Reported Symptoms in Pediatric Patients with Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS) Receiving Leniolisib*
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: Thursday, May 7, 2026, 1:30-2:30 pm (EDT)
Abstract/Poster Number: 35

Title: *Symptom Changes in Pediatric Patients with Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS) Receiving Leniolisib*
Presenting Author: Amanda Harrington, PhD, Pharming Healthcare Inc., Warren, NJ, USA
Session Type: Poster
Session Date/Time: Thursday, May 7, 2026, 1:30-2:30 pm (EDT)
Abstract/Poster Number: 105

Title: *Use of Sirolimus and Immunoglobulin Replacement Therapy (IRT) In Patients with Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS): A Systematic Literature Review (SLR)*
Presenting Author: Nicholas Hartog, MD, Cornwell Health Pediatric Allergy and Immunology, Grand Rapids, MI, US
Session Type: Poster
Session Date/Time: Thursday, May 7, 2026, 1:30-2:30 pm (EDT)
Abstract/Poster Number: 112

Title: *Caregivers of Children with APDS Balance Complex Medical Care, Family, and Work Responsibilities*
Presenting Author: Kristie Cline, MBA, Pharming Healthcare Inc., Warren, NJ, USA
Session Type: Poster
Session Date/Time: Friday, May 8, 2026, 2:10-3:10pm (EDT)
Abstract/Poster Number: 115

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.[8]

About leniolisib

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in the U.S., U.K., Australia and Israel in adult and pediatric patients 12 years of age and older and in Japan for patients 4 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 open label extension data has supported the safety and tolerability of long-term leniolisib administration.[9,10]

Leniolisib is currently under regulatory review for the treatment of APDS in Canada and several other countries. Leniolisib is also being evaluated 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 develop and commercialize a portfolio of innovative medicines, including small molecules and biologics. Pharming is headquartered in Leiden, the Netherlands, with U.S. and European operations.

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 2025 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2025, 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.

References

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