Pharming Group announces presentations at 2025 ACAAI Annual Scientific Meeting showcasing new data across rare disease portfolio

Leiden, the Netherlands, October 20, 2025: Pharming Group N.V. ("Pharming" or "the Company") (Euronext: PHARM; Nasdaq: PHAR) today announced that 12 abstracts have been accepted for presentation at the American College of Allergy, Asthma & Immunology (ACAAI) 2025 Annual Scientific Meeting taking place in Orlando, Florida on November 6-10.

Five posters will present positive new clinical, economic, and comparative data for RUCONEST® (recombinant C1 esterase inhibitor) and its role in on-demand hereditary angioedema (HAE) treatment. Seven posters will highlight new evidence on the real-world effectiveness of Joenja® (leniolisib), including additional results from the Phase III pediatric trial, as well as advancing understanding of the pediatric and caregiver burden in activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS).

Anurag Relan, Chief Medical Officer of Pharming, commented:
"We are proud that our scientific contributions in both hereditary angioedema (HAE) and activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) have been recognized with a significant number of abstract acceptances at this year's ACAAI. The work underscores the rigor of our research and our commitment to advancing patient care. We look forward to the new data being presented to the medical community during the meeting in November."

Presentation Details:

RUCONEST®

Study Title: *Indirect Treatment Comparison (ITC) of Recombinant C1 Inhibitor and Sebetralstat for HAE On-Demand Therapy*
Presenting Author: Dr John Anderson, MD, AllerVie Health, Birmingham, AL, USA and University of Alabama at Birmingham, Birmingham, AL, USA
Poster Date/Time: Friday, November 7, 2025, 4:05 pm (EST)
Monitor: 14

Study Title: *Cost-Effectiveness of rhC1-INH Versus Sebetralstat for Treatment of HAE Attacks*
Presenting Author: Dr Jonathan A. Bernstein, MD, Advanced Allergy Services, LLC, Cincinnati, OH, USA and Bernstein Clinical Research Center, LLC, Cincinnati, OH, USA
Poster Date/Time: Friday, November 7, 2025, 4:05 pm (EST)
Monitor: 16

Study Title: *Re-Analysis of rhC1-INH Clinical Data With Contemporary Time-to-Event Endpoint Definitions*
Presenting Author: Joseph R. Harper, PharmD, Pharming Healthcare, Inc. Warren, NJ, USA
Poster Date/Time: Friday, November 7, 2025, 5:05 pm (EST)
Monitor: 15

Study Title: *Mapping Patient-Reported Outcome Measures Across Clinical Trials in HAE*
Presenting Author: Dr Michael Manning, MD, Allergy Asthma & Immunology Associates, LTD, Scottsdale, AZ, USA and University of Arizona College of Medicine-Phoenix, Phoenix, AZ, USA
Poster Date/Time: Sunday, November 9, 2025, 11:45 am (EST)
Monitor: 13

Study Title: *Clinician Perspectives on Modifiers of Observed Treatment Effect in On- Demand HAE Therapy Trials*
Presenting Author: Dr Raffi Tachdjian, MD, MPH, Division of Allergy and Clinical Immunology, David Geffen School of Medicine, University of California, Los Angeles, CA, USA, and Division of Allergy & Immunology, Providence Saint John's Health Center, Santa Monica, CA, USA
Poster Date/Time: Saturday, November 8, 2025, 12:15 pm (EST)
Monitor: 13

Joenja® (leniolisib)
Study Title: *Effectiveness of Leniolisib in Reducing Infections Among Patients With APDS*
Presenting Author: Dr Niraj C. Patel, MD, MS, Division of Pediatric Allergy and Immunology, Duke University, Durham, NC, USA
Poster Date/Time: Friday, November 7, 3:50 pm (EST)
Monitor: 20

Study Title: *Impact of Leniolisib on Healthcare Utilization in Patients With APDS*
Presenting Author: Ami Claxton, PhD, MS, Pharming Healthcare, Inc., Warren, NJ, USA
Poster Date/Time: Friday, November 7, 2025, 4:05 pm (EST)
Monitor: 20

Study Title: *Symptoms/Quality of Life in Pediatric Patients With APDS Receiving Leniolisib*
Presenting Author: Jason Bradt, MD , Pharming Healthcare, Inc., Warren, NJ, USA
Poster Date/Time: Friday, November 7, 2025, 4:35 pm (EST)
Monitor: 20

Study Title: *Adult/Pediatric Perspectives and Experiences With APDS and Leniolisib: APPEAL*
Presenting Author: Amanda Harrington, PhD, Pharming Healthcare Inc., Warren, NJ, USA
Poster Date/Time: Friday, November 7, 2025, 2:35 pm (EST)
Monitor: 20

Study Title: *APDS Burden of Illness: Pediatric and Adolescent Caregiver Perspectives*
Presenting Author: Dr Joud Hajjar, MD, PhD, MS, Section of Immunology, Allergy and Retrovirology, Department of Pediatrics, Baylor College of Medicine, Houston, TX, USA
Poster Date/Time: Friday, November 7, 2025, 4:20 pm (EST)
Monitor: 20

Study Title: *Real-World Adherence and Persistence With Leniolisib in Patients With APDS*
Presenting Author: Jessica Kuivinen, PharmD, PANTHERx Rare Pharmacy, Pittsburgh, PA, USA
Poster Date/Time: Saturday, November 8, 2025, 12:15 pm (EST)
Monitor: 17

Study Title: *Characterization of Activated Phosphoinositide 3-Kinase Delta Syndrome in the APDS-CHOIR Clinical Outcomes Registry*
Presenting Author: Kelli Williams, MD, Department of Pediatrics, Medical University of South Carolina, Charleston, SC, USA
Poster Date/Time: Friday, November 7, 2025, 14:50 pm (EST)
Monitor: 20

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 6, 2025, beginning at 08:00 EST on ACAAI's website.

About Hereditary Angioedema (HAE)

Hereditary Angioedema (HAE) is a rare genetic disorder.[1] The condition is caused by a deficiency of the C1 esterase inhibitor protein, which is normally present in blood and helps control inflammation (swelling) and parts of the immune system. Because defective C1-Inhibitor does not adequately perform its regulatory function, a biochemical imbalance can occur and produce unwanted peptides that induce the capillaries to release fluids into surrounding tissue, thereby causing swelling or edema.[2]

HAE is characterized by spontaneous and recurrent episodes of swelling (edema attacks) of the skin in different parts of the body, as well as in the airways and internal organs. Edema of the skin usually affects the extremities, the face, and the genitals. Almost all HAE patients suffer from bouts of severe abdominal pain, nausea, vomiting and diarrhea caused by swelling of the intestinal wall.[3]

Edema of the throat, nose or tongue is particularly dangerous and potentially life-threatening and can lead to obstruction of the airway passages.[4] Although there is currently no known cure for HAE, it is possible to treat the symptoms associated with angioedema attacks.[5] HAE affects about 1 in 10,000 to 1 in 50,000 people worldwide.[6] HAE is often misdiagnosed as the symptoms are similar to many other common conditions such as allergies or appendicitis resulting in diagnostic delay.[7]

About RUCONEST®

RUCONEST® is a recombinant C1 esterase inhibitor (rhC1INH) protein replacement therapy indicated for the treatment of acute attacks in adult and adolescent patients with hereditary

angioedema (HAE). RUCONEST® is approved in the U.S., Europe and the UK and is the only recombinant C1 esterase inhibitor worldwide.

RUCONEST® is delivered intravenously and is immediately and completely bioavailable enabling rapid intervention at the onset of an HAE attack. At the recommended dose of 50 U/kg, RUCONEST® has been shown to normalize C1INH activity levels, which are clinically relevant in the treatment of HAE. By irreversibly binding to and deactivating key mediators such as coagulation factor FXII and kallikrein, RUCONEST® halts the production of bradykinin and other inflammatory peptides, thereby stopping the progression of the attack. RUCONEST® is not indicated for the treatment of laryngeal attacks, as effectiveness in this subset of patients was not established in clinical studies.[8]

Please see Full Prescribing Information and Patient Product Information **here**

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[9,10,11] APDS is characterized by a variety of symptoms, including severe, recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy.[12,13] 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.[14] As APDS is a progressive disease, this delay may lead to an accumulation of damage over time, including permanent lung damage and lymphoma.[12-15] 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 (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.[16,17] Leniolisib is currently under regulatory review in the European Economic Area, Japan, Canada and several other countries for APDS. 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 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.

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