



Pharming Announces New ICD-10-CM Code for APDS, a Rare Primary Immunodeficiency

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Implemented by the Centers for Disease Control and Prevention, the diagnosis code will accurately identify US patients with APDS, supporting care and research efforts

LEIDEN, Netherlands, Aug. 2, 2022 /PRNewswire/ -- Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) announces that a new diagnosis code for reporting cases of activated phosphoinositide 3-kinase delta syndrome (APDS), a rare primary immunodeficiency, will be added to the International Classification of Diseases, 10th Revision, Clinical Modification (ICD-10-CM) by the US Centers for Disease Control and Prevention (CDC). The diagnosis code, D81.82 – Activated Phosphoinositide 3-kinase Delta Syndrome (APDS), will be effective starting October 1, 2022.



Anurag Relan, Chief Medical Officer of Pharming, commented:

"By assigning this ICD-10-CM code, the CDC is formally recognizing APDS as a discrete immunological disease, and that will make a life-altering difference for people affected by the condition. By using the unique diagnostic code to identify both established and new patients with APDS, physicians will increase care options for affected individuals while helping to boost the world's understanding of the prevalence, mechanisms, and outcomes of this progressively debilitating disease. For healthcare practitioners, this milestone marks an opportunity to make a big difference by taking a simple action."

The assignment of the ICD-10-CM code will, for the first time, enable physicians and payors in the US to add a diagnosis of APDS to patients' health records, which will help connect these individuals with researchers studying the prevalence and course of the disease. In addition, by allocating a specific diagnosis, the new ICD-10-CM code may help confirm medical necessity in individual patients, thus improving their access to relevant care options through US health insurance plans.

Caused by genetic variants affecting approximately one to two people per million, APDS causes significant lymphoproliferation and immune dysfunction, as well as an increased risk of lymphoma. There is no approved therapy for the disease and treatment is generally limited to supportive care, such as antibiotics and immunoglobulin replacement therapy. Physician and patient advocacy groups specializing in immunodeficiency disorders, along with Pharming, expect the decision to raise awareness about this rare disease.

Vicki and Fred Modell, co-founders of the Jeffrey Modell Foundation, commented:

"We are excited that US regulatory authorities have assigned APDS an ICD-10-CM code. As a foundation dedicated to early diagnosis, meaningful treatments, and cures for primary immunodeficiency, we are aware of the physical and emotional challenges people with APDS face due to misdiagnosis of their disease. By increasing recognition of the condition, we expect the new diagnostic code to help ensure that every patient is included when it comes to the delivery of appropriate and meaningful treatments for APDS."

About Activated Phosphoinositide 3-Kinase δ Syndrome (APDS)

APDS is a rare primary immunodeficiency that affects approximately one to two people per million. Also known as PASLI, it is caused by variants in either of two genes, *PIK3CD* or *PIK3R1*, that regulate maturation of white blood cells. Variants of these genes lead to hyperactivity of the PI3K δ (phosphoinositide 3-kinase delta) pathway.^{1,2} Balanced signaling in the PI3K δ pathway is essential for physiological immune function. When this pathway is hyperactive, immune cells fail to mature and function properly, leading to immunodeficiency and dysregulation.^{1,3} APDS is characterized by 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, 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 The only way to definitively diagnose this condition is through genetic testing.

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. Pharming is commercializing and developing an innovative portfolio of protein replacement therapies and precision medicines, including small molecules, biologics, and gene therapies that are in early to late-stage development. Pharming is headquartered in Leiden, 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.

About the Jeffrey Modell Foundation

Vicki and Fred Modell established the Jeffrey Modell Foundation (JMF) in 1987, in memory of their son Jeffrey, who died at the age of 15, from complications of Primary Immunodeficiency (PI) — a genetic condition that is chronic, serious, and often fatal. JMF is a global nonprofit organization dedicated to early diagnosis, meaningful treatments and, ultimately, cures through research, physician education, public awareness, advocacy, patient support, newborn screening, and genetic sequencing. For more information, visit <https://www.info4pi.org/>.

Forward-Looking Statements

This press release contains forward-looking statements, including with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, Pharming's ability to overcome the challenges posed by the COVID-19 pandemic to the conduct of its business, 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 and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2021 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2021 filed with the US 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. 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.

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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