Pharming receives Orphan Drug Designation from the European Commission for leniolisib for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)

- Leniolisib is in a phase II/III registration-enabling study for the treatment of APDS, an ultra-rare and debilitating disease with no approved treatment
- If approved, the drug is expected to reach the market H2 2022

Leiden, The Netherlands, 21 October 2020: Pharming Group N.V. (Euronext Amsterdam: PHARM) today announced that the European Commission has granted orphan drug designation for leniolisib for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS), based on a positive opinion from the Committee for Orphan Medicinal Products (COMP) of the European Medicine Agency (EMA). Leniolisib was previously granted Orphan Drug Designation by the US Food and Drug Administration (FDA) in January 2018 for "the treatment of Activated PI3Kδ Syndrome (APDS) or p110δ-activating mutation causing senescent T cells, lymphadenopathy and immunodeficiency (PASLI)".

The European Commission orphan drug designation provides certain regulatory procedural and financial incentives including, but not limited to, product market exclusivity for ten years in the EU following regulatory approval. To qualify, an investigational drug must be intended to treat a life-threatening or chronically debilitating condition that affects fewer than five in 10,000 people in the EU, and where the treatment provides a significant benefit to those affected by the condition or no satisfactory treatment is available.

Sijmen de Vries, Chief Executive Officer of Pharming, commented:

"We are pleased to have received orphan drug designation from the European Commission, an important milestone in the development of leniolisib for the treatment for APDS, an ultra-rare and debilitating disease. With no currently approved treatment, leniolisib has the potential to address a significant unmet need for patients with APDS. Leniolisib is currently being studied in a registration-enabling Phase II/III trial and remains, subject to regulatory approval, on track to launch in H2 2022."

About Activated Phosphoinositide 3-kinase Delta Syndrome (APDS)

Activated phosphoinositide 3-kinase-delta (PI3Kδ) syndrome (APDS) is caused by mutations in the gene PIK3CD (Type 1 APDS) or PIK3R1 (Type 2 APDS) that activate PI3Kδ. Synonyms for Type 1 and Type 2 APDS are PASLI-CD and PASLI-R1, respectively. PASLI is the acronym for p110δ-activating mutation causing senescent T cells, lymphadenopathy, and immunodeficiency.

APDS is defined as an ultra-rare, genetic, primary immunodeficiency disease characterized by increased susceptibility to recurrent and/or severe bacterial and viral infections, chronic benign lymphoproliferation, and/or autoimmune disease. The APDS incidence rate around the world is currently estimated to be 1-2 per million. The diagnosis of APDS is made by sequencing the genes PIK3CD and/or PIK3R1 in patients with a compatible phenotype, i.e., immunodeficiency and lymphoproliferation of unknown origin.

Beginning in childhood, people with APDS develop recurrent infections, particularly in the lungs, sinuses, and ears. Over time, recurrent respiratory tract infections can lead to a condition called bronchiectasis, which damages the passages leading from the windpipe to the lungs (bronchi) and can

cause breathing problems. People with APDS may also have chronic active viral infections, commonly Epstein-Barr virus or cytomegalovirus infections. Sufferers also frequently develop lymphomas and other cancers.

Another possible feature of APDS is abnormal clumping of white blood cells. These clumps can lead to enlarged lymph nodes (lymphadenopathy), or the white blood cells can build up to form solid masses (nodular lymphoid hyperplasia), usually in the moist lining of the airways or intestines. While lymphadenopathy and nodular lymphoid hyperplasia are noncancerous (benign), APDS also increases the risk of developing a form of cancer called B-cell lymphoma.

About leniolisib

Leniolisib is a small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor with immunomodulating and potentially anti-neoplastic activities. Leniolisib inhibits the production of phosphatidylinositol-3-4-5-trisphosphate (PIP3). PIP3 serves as an important cellular messenger specifically activating Akt (via PDK1) and regulates a multitude of cell functions such as proliferation, differentiation, cytokine production, cell survival, angiogenesis, and metabolism. Unlike PI3Kα and PI3Kβ which are ubiquitously expressed, PI3Kδ and PI3Kγ are expressed primarily in cells that are of hematopoietic origin. The central role of PI3Kδ in regulating numerous function of cells of the adaptive immune system (B-cells and to a lesser extent T cells) as well as the innate immune system (neutrophil, mast cells, and macrophages) strongly indicates that PI3Kδ is a valid and potentially effective therapeutic target for APDS.

Leniolisib was recently in-licensed from Novartis and is being studied in a registration-enabling Phase II/III trial which is currently enrolling patients in clinical sites in the US and Europe. To date, leniolisib has proven to be safe and well tolerated in healthy subjects as well as APDS patients during the first-in-human trial and the ongoing open label extension trial.

About Pharming Group N.V.

Pharming is a specialty pharmaceutical company developing innovative products for the safe, effective treatment of rare diseases and unmet medical needs. Pharming's lead product, RUCONEST® (conestat alfa) is a recombinant human C1 esterase inhibitor approved for the treatment of acute attacks of angio-edema in patients with hereditary angioedema (HAE) in Europe for adults, adolescents and children from two years of age, in the US for adults and adolescents, and in Israel and South Korea for adults. The product is available on a named-patient basis in other territories where no marketing authorisation application was yet submitted.

RUCONEST® is commercialised by Pharming in the US and in Europe, and the Company holds all other commercialisation rights in other countries not specified below. In some of these other countries distribution is made in association with the HAEi Global Access Program (GAP). RUCONEST® is distributed in Argentina, Colombia, Costa Rica, the Dominican Republic, Panama, and Venezuela by Cytobioteck, in South Korea by HyupJin Corporation and in Israel by Kamada.

RUCONEST® is also being evaluated for various additional indications. Pharming's technology platform includes a unique production process that has proven capable of producing industrial quantities of pure high-quality recombinant human proteins in a more economical and less immunogenic way compared with current cell-line based methods.

Leads for enzyme replacement therapy ("ERT") for Pompe and Fabry's diseases are also being developed and optimised respectively, at present.

Pharming has recently in-licensed leniolisib from Novartis, a small molecule and selective PI3Kδ inhibitor, which is in a registrational study for activated PI3K-delta syndrome (APDS), a rare form of Primary Immunodeficiency.

Pharming has a long-term partnership with the China State Institute of Pharmaceutical Industry ("CSIPI"), a Sinopharm company, for the commercialisation of RUCONEST® in the Republic of China and the future development of new products.

Forward-looking Statements

This press release of Pharming Group N.V. and its subsidiaries ("Pharming", the "Company") may contain forward-looking statements including without limitation those regarding Pharming's financial projections, market expectations, developments, partnerships, plans, strategies and capital expenditures.

The Company cautions that such forward-looking statements may involve certain risks and uncertainties, and actual results may differ. Risks and uncertainties include without limitation the effect of competitive, political and economic factors, legal claims, the Company's ability to protect intellectual property, fluctuations in exchange and interest rates, changes in taxation laws or rates, changes in legislation or accountancy practices and the Company's ability to identify, develop and successfully commercialise new products, markets or technologies.

As a result, the Company's actual performance, position and financial results and statements may differ materially from the plans, goals and expectations set forth in such forward-looking statements. The Company assumes no obligation to update any forward-looking statements or information, which should be taken as of their respective dates of issue, unless required by laws or regulations.

For further public information, contact:

Pharming Group, Leiden, The Netherlands
Sijmen de Vries, CEO: T: +31 71 524 7400
Susanne Embleton, Investor Relations Manager: T: +31 71 524 7400 E: investor@pharming.com

FTI Consulting, London, UK
Victoria Foster Mitchell/Mary Whittow
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