European Commission approves pharmaand GmbH's Pegasys® (peginterferon alfa-2a) as a treatment for all eligible patients with the myeloproliferative neoplasms (MPNs) blood cancers polycythemia vera (PV) and essential thrombocythemia (ET)

- First European Commission (EC) approval of an interferon alfa as a monotherapy treatment for adults with essential thrombocythemia (ET)
- First European Commission (EC) approval of an interferon alfa as a monotherapy treatment for adults with polycythemia vera (PV) without any restrictions
- EC approval is based on trials conducted by the Myeloproliferative Disorders Research Consortium (MPD-RC) and follows a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) in June 2024

Intended for the Media

Vienna, Austria, August 14, 2024 – pharmaand GmbH (pharma&) announced today that the European Commission (EC) has granted marketing authorization of a Type II variation for Pegasys® (peginterferon alfa-2a) as a monotherapy treatment for adults with polycythemia vera (PV) or essential thrombocythemia (ET). PV and ET are both myeloproliferative neoplasms (MPNs), types of rare blood cancers in which the bone marrow produces peripheral blood cells that do not develop and function normally.[i, ii]

"Pursuing this approval comes in response to feedback from key opinion leaders and patient advocacy groups over the last few years, as well as the expanded use of Pegasys following its inclusion in independently developed oncology guidelines," said Frank Rotmann, Founder and Managing Director of pharma&. "We now have the opportunity to reach additional eligible patients in need of treatment options for two chronic, rare blood cancers, polycythemia vera and essential thrombocythemia. The approval of Pegasys for ET in Europe is the first monotherapy interferon alfa for the condition, and is unique to pharma&. This regulatory milestone aligns with our mission to foster the development of essential medicines worldwide, aspiring to leave no patient behind."

The EC based its approval on a Phase 3 multicenter trial (MPD-RC 112, NCT01259856) and a Phase 2 multicenter trial (MPD-RC 111, NCT01259817) conducted by the

Myeloproliferative Disorders Research Consortium (MPD-RC), both of which have been published in peer-reviewed journals in 2022.

"While Pegasys has been recommended for use in hematology guidelines for the treatment of polycythemia vera and essential thrombocythemia, today's European Commission approval provides clear and concise information to help healthcare professionals and eligible patients in the European Union understand how to use Pegasys safely and effectively long-term," said Professor Hans Hasselbalch, Head of research, Department of Hematology, at Zealand University Hospital, Denmark. "People living with these two chronic types of blood cancer need and deserve additional treatment options, and today's approval is positive news for eligible patients in Europe."

Following the acquisition of Pegasys in 2021 from F. Hoffmann La Roche AG (Roche), pharma& committed to the ongoing development and future certification of the bio-manufacturing capabilities by investing in the Company's wholly-owned manufacturing plant in Austria, Loba biotech GmbH. This label expansion will allow pharma& to better plan and forecast product availability, ensuring that all eligible patients who reside in the EU and need Pegasys can access it across all licensed uses in the long term.

###

About polycythemia vera (PV) and essential thrombocythemia (ET)

Polycythemia vera (PV) and essential thrombocythemia (ET) are both myeloproliferative neoplasms (MPNs), and types of rare blood cancers, in which the malignant cells within the bone marrow produces peripheral blood cells that do not function normally.[i,ii]

If well controlled and managed, PV and ET are both chronic, lifelong conditions.[ii,iii] PV is more common in men,[iv] whereas ET is more common in women.[v] In both PV and ET, the median age of diagnoses is around 60 years of age.[iv, v]

Almost all patients diagnosed with PV have a mutation of the Januskinase (*JAK2-V617F)* gene,[vi] and nearly 60% of ET patients also have this mutation.[vii] Its precise role in PV and ET is still being investigated.[viii]

About Pegasys® (peginterferon alfa-2a)

Pegasys is a type I interferon. The type I interferons present in humans are IFN-α, IFN-β, IFN-ε, IFN-κ and IFN-ω.[ix] Interferons (IFNs) and their receptors are a subset of class 2

alpha-helical cytokines that have existed in early chordates for about 500 million years and represent early elements in innate and adaptive immunity.[x] IFNs are noted for their ability to "interfere" with viral replication within the host cells.[xi] All type I IFNs bind to a specific cell surface receptor complex, the IFN-a receptor (IFNAR), consisting of IFNAR1 and IFNAR2 chains.[xii]

Pegasys is made when interferon alfa-2a undergoes the process of pegylation in which one or more chains of polyethylene glycol (PEG) are attached to another molecule.[xiii] In Pegasys, a large, branched, mobile PEG is bound to the interferon alfa-2a molecule and provides a selectively protective barrier.[xiv] To prolonged the pharmacokinetic the high molecular weight (40 kilodaltons) branched PEG is covalently bound to IFN alfa 2a to exert in Pegasys.[xv]

Pegasys was previously approved by the EC for the treatment of chronic hepatitis B (CHB) in adults and children aged 3 years and older or chronic hepatitis C (CHC) in adults and children aged 5 years and older in combination with other medicinal products in adults or ribvavirin in children.[xiii]

For a full list of adverse events and information on dosage and administration and other precautions when using Pegasys, please refer to the EU Summary of Product Characteristics, click here. For non-EU countries, please refer to your local health authority.

Healthcare professionals should report any suspected adverse reactions via their national reporting systems.

For medical information inquiries outside of the U.S., contact pharma& at medinfo@pharmaand.com.

For medical information inquiries within the U.S., contact pharma& at medinfo.us@pharmaand.com.

You may report adverse events to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Alternatively, to report an adverse event or reaction, contact pharma& at pv@pharmaand.com.

To report a product complaint, contact pharma& at complaints@pharmaand.com.

About pharma&

pharmaand GmbH (pharma&), a privately owned global company, aspires to breathe new life into proven medicines. The Company is dedicated to preserving the availability and fostering the further development of essential medicines worldwide to leave no patient behind. Over the past five years, pharma& has acquired and integrated 10+ medicines, expanding its portfolio across a wide range of therapy areas, with an increasing focus on hematology and oncology treatments. The Company's unique synthesis of subsidiaries, joint ventures, and partners enables pharma& to provide its portfolio of medicines to eligible patients worldwide by spanning the continuum of development, product and API manufacturing, partner distribution, healthcare provider engagement, distribution and services to patients.

pharma& cautions that any forward-looking statements or projections made, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. pharma& does not undertake to update or revise any forward-looking statements.

# # #

pharma& Media Contact:
media@pharmaand.com

---

[i] National Heart, Lung, and Blood Institute. Polycythemia Vera. Accessed June 18, 2024. https://www.nhlbi.nih.gov/health/polycythemia-vera

[ii] Essential thrombocythaemia. Blood cancer UK. Accessed June 14, 2024. https://bloodcancer.org.uk/understanding-blood-cancer/essential-thrombocythaemia-et/et-prognosis/

[iii] Tefferi A, et al. Polycythemia vera: historical oversights, diagnostic details, and therapeutic views. Leukemia. 2021 Dec;35(12):3339-3351. doi: 10.1038/s41375-021-01401-3.

[iv] Pillai AA, Fazal S, Mukkamalla SKR, et al. Polycythemia. In: StatPearls Treasure Island (FL): StatPearls Publishing; 2024 Jan

[v] Accurso V, et al. The Essential Thrombocythemia in 2020: What We Know and Where We Still Have to Dig Deep. Clin Med Insights Blood Disord. 2020 Dec

28;13:2634853520978210. doi: 10.1177/2634853520978210

[vi] Thiele J, et al. The international consensus classification of myeloid neoplasms and acute Leukemias: myeloproliferative neoplasms. Am J Hematol. 2023 Jan;98(1):166-179. doi: 10.1002/ajh.26751.

[vii] Publicover A, Medd P. Myeloproliferative neoplasms. Clin Med (Lond). 2013;13(2):188-192. doi:10.7861/clinmedicine.13-2-188

[viii] Dos Santos LC, et al. Cytogenetics, JAK2 and MPL mutations in polycythemia vera, primary myelofibrosis and essential thrombocythemia. Rev Bras Hematol Hemoter. 2011;33(6):417-24. doi: 10.5581/1516-8484.20110116

[ix] López de Padilla CM, Niewold TB. The type I interferons: Basic concepts and clinical relevance in immune-mediated inflammatory diseases. Gene. 2016 Jan 15;576(1 Pt 1):14-21. doi: 10.1016/j.gene.2015.09.058

[x] Pestka S. The interferons: 50 years after their discovery, there is much more to learn. J Biol Chem. 2007 Jul 13;282(28):20047-51. doi: 10.1074/jbc.R700004200

[xi] Devasthanam A. S. Mechanisms underlying the inhibition of interferon signaling by viruses. Virulence. 2014; 5(2), 270–277. https://doi.org/10.4161/viru.27902

[xii] de Weerd, et al. Type I interferon receptors: biochemistry and biological functions. The Journal of biological chemistry. 2007; 282(28), 20053–20057. https://doi.org/10.1074/jbc.R700006200

[xiii] EU SmPC: European Medicines Agency. Pegasys Summary of Product Characteristics.

[xiv] Centre Watch. Pegasys (peginterferon alfa-2a). 2024. Accessed June 17, 2024. https://www.centerwatch.com/directories/1067-fda-approved-drugs/listing/3996-pegasys-peginterferon-alfa-2a

[xv] Bailon P, et al. Rational design of a potent, long-lasting form of interferon: a 40 kDa branched polyethylene glycol-conjugated interferon alpha-2a for the treatment of hepatitis C. Bioconjug Chem 2001; 12: 195– 202.