Press Release

July 1st, 2021

TOPADUR Pharma AG announces the submission of Orphan Drug Designation Application for TOP-N53

Zurich-Schlieren, Switzerland, July 1st, 2021. TOPADUR Pharma AG, a biopharmaceutical start-up company today is pleased to announce the submission of an application to the European Medicines Agency (EMA) to receive Orphan Drug Designation (ODD) status for its lead product TOP-N53 to treat digital ulcers in patients with systemic sclerosis.

"The submission of our ODD represents a major milestone for the Company and it brings significant benefits to the development process of TOP-N53," said Reto Naef, Chairman of the Board of Directors and CEO at TOPADUR Pharma AG. "We believe that TOP-N53 has significant potential to provide a valuable treatment option for digital ulcers in patients afflicted by systemic sclerosis, a disabling condition."

About EU Orphan Designation

The EMA, via its Committee for Orphan Medicinal Products (COMP), evaluates applications for orphan designation. The orphan designation advances the development of a medicine that demonstrate promise for the treatment, prevention or diagnosis of life-threatening or chronically debilitating rare diseases that affect not more than 5 in 10'000 people across the EU. Furthermore, there must be no satisfactory method of treating the condition. ODD provides incentives for sponsors, including protocol assistance, a reduction or waiving of fees and 10 years of market exclusivity once the therapy is approved.

About TOP-N53

TOP-N53 is a dual mode of action phosphodiesterase type 5 inhibitor (PDE5) / organic nitrate ester that targets the cGMP-Enzyme Regulation System. In a process called `bioactivation`, TOP-N53 gets converted into nitric oxide (NO) and the more potent PDE5 inhibitor TOP-52 in the wound tissue. NO activates soluble guanylyl cyclase (sGC) to synthesize cyclic guanosine-3',5'-monophosphate (cGMP), while TOP-52 and TOP-N53 reduce degradation of cGMP by inhibiting PDE5. TOP-N53 locally applied, increases microcirculation and induces the formation of new blood vessels. This new drug principle demonstrated unprecedented wound healing effects during the preclinical development of the drug candidate. Additionally, the Company is completing Phase 1 clinical trial, a double-blind, dose-escalation of TOP-N53 to evaluate its safety and tolerability in healthy subjects.

Digital Ulcers in Systemic Sclerosis

Systemic sclerosis is a rare, debilitating, autoimmune disease of the connective tissue. It is characterized by inflammation, vasculopathy, progressive fibrosis in the skin, joints, internal organs with excessive collagen accumulation. About 95% of patients with systemic sclerosis are afflicted with recurrent episodes of Raynaud´s phenomenon, a painful condition with tissue ischemia-reperfusion cycles due to vasospasms and accumulating structural damage to the digital arterioles from oxidative stress that favors the occurrence of digital ulcers. These ulcers are very painful and often result in impaired hand function.

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About TOPADUR

TOPADUR is a patient-oriented biotech company enhancing quality of life through cutting-edge research. The Swiss-based biotech company developed the DualTOP™ technology platform consisting of new dual-acting drugs that target the cGMP-Enzyme Regulation System to stimulate microcirculation, enable tissue regeneration, and avoid local oxygen deficiency.

For more information regarding TOPADUR PHARMA AG, please go to: www.topadur.com

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