TOPADUR

Press Release

October 15th, 2021

EMA grants TOP-N53 Orphan Drug Designation for the Treatment of Digital Ulcers in Systemic Sclerosis

Zurich-Schlieren, Switzerland, October 15th, 2021. TOPADUR Pharma AG, a clinical-stage biopharmaceutical start-up company developing first-in-class drugs for aging diseases, today is pleased to announce that the European Medicines Agency (EMA) has granted Orphan Drug Designation (ODD) to TOP-N53, the company's lead product, for the treatment of digital ulcers in patients with systemic sclerosis (SSc).

"This ODD by EMA marks an important milestone in the clinical development of TOP-N53 and supports our conviction that innovative treatments for chronic wounds are urgently needed" said Reto Naef, Chairman of the Board of Directors and CEO at TOPADUR Pharma AG. "TOP-N53 potency, tolerability and efficacy were demonstrated in First-in-Human study. We believe that this novel drug candidate has the potential to transform the standard of care of digital ulcers in SSc patients and to provide a valuable treatment option for this 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 just completed the first clinical study, 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.

About TOPADUR

TOPADUR is a patient-oriented biotech company developing disruptive therapies for aging diseases. The Swiss-based biotech company developed the DualTOP™ technology platform consisting of new dual-acting drugs that increase the levels of cGMP to stimulate microcirculation, enable tissue regeneration, and avoid local oxygen deficiency. TOPADUR's R&D portfolio consists of promising development candidates in regenerative medicine, oncology, ophthalmology and medical aesthetics. The DualTOP™ technology will contribute to promoting long healthy life.

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

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