

# Press Release

Zurich-Schlieren, Switzerland, 9 September 2025

## Topadur Pharma AG Secures FDA Orphan Drug Designation for TOP-N53 Targeting Digital Ulcers in Systemic Sclerosis

Topadur Pharma AG today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to its investigational therapy TOP-N53 for the treatment of digital ulcers in patients with systemic sclerosis.

Systemic sclerosis is a rare, life-threatening autoimmune disease characterized by fibrosis, vascular damage, and chronic digital ulcers that severely impair patients' quality of life. Currently, there is no approved therapy for the treatment of digital ulcers in systemic sclerosis patients, highlighting the urgent need for new therapeutic options. With Orphan Drug Designation now granted by both the FDA and the European Medicines Agency (EMA), Topadur has achieved two major milestones. These designations provide development incentives, including market exclusivity and enhanced regulatory guidance in both the U.S. and Europe.

*"This important recognition from the FDA underscores the urgent unmet need in systemic sclerosis and validates our innovative approach with TOP-N53's unique bifunctional mechanism,"* said Reto Naef, Founder and President of the Board at Topadur Pharma AG.

Topadur is currently conducting a Phase 2a clinical trial evaluating TOP-N53 in patients with systemic sclerosis-related digital ulcers. To support recruitment and strengthen the clinical dataset, the company is expanding the study by including selected clinical centers.

*"Digital ulcers are a particularly severe manifestation of systemic sclerosis, and effective targeted treatments are urgently needed. Innovative approaches such as TOP-N53 could represent an important step toward improving care for these patients,"* said Professor Oliver Distler, M.D., Zurich, Switzerland.

### About TOP-N53

TOP-N53 is a first-in-class bifunctional small molecule designed to address both vascular dysfunction and impaired tissue regeneration in systemic sclerosis. By combining nitric oxide donation with phosphodiesterase-5 (PDE5) inhibition, TOP-N53 aims to improve blood flow and promote wound healing, offering patients a novel therapeutic option. The topical administration of TOP-N53 enables maximal therapeutic efficacy directly at the site of the ulcer, while minimizing systemic drug exposure.

### About Topadur

Topadur Pharma AG is a Swiss clinical-stage biopharmaceutical company dedicated to developing transformative therapies in regenerative medicine, aging-related and rare diseases. Topadur is advancing breakthrough treatments designed to improve patient outcomes and enhance quality of life for people with high unmet medical needs worldwide. For more information regarding Topadur, please visit: [www.topadur.com](http://www.topadur.com)

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