

## TargED Biopharmaceuticals' TGD001 receives Orphan Drug Designation in Europe

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TargED Biopharmaceuticals B.V., a Dutch biotech company that develops targeted biological drugs to improve the treatment of thrombosis and thrombosis related diseases, announced that the European Commission has granted Orphan Drug Designation to its lead product TGD001, a von Willebrand factor targeted thrombolytic, for the treatment of thrombotic thrombocytopenic purpura (TTP). This significant milestone underscores the potential of TGD001 to address unmet medical needs in patients suffering from TTP and its promise to complement the currently available treatment options.

“We are thrilled to receive Orphan Drug Designation for TGD001,” said Kristof Vercruysse, CEO of TargED Biopharmaceuticals. “This designation not only validates the promise of a substantial clinical benefit that our innovative approach to treat TTP may offer but also provides us with important incentives to expedite the development and to bring this treatment to patients as swiftly as possible.”

Orphan Designation is granted to medicinal products intended for the treatment, prevention, or diagnosis of seriously debilitating or life-threatening rare diseases that affect fewer than five in 10,000 people in the European Union. Benefits of an orphan designation include a 7 to 10-year market exclusivity upon marketing authorization, protocol assistance and regulatory fee reductions .

### **About TTP**

TTP is a rare, life-threatening blood disorder characterized by sudden episodes of clot-formation in small blood vessels throughout the body. This leads to disruption of the blood flow to organs such as the kidney, heart, and brain, resulting in the functional failure of these organs. Patients experiencing a TTP attack present with fatigue, breathing difficulties, bleeding underneath the skin or bleeding in general, and, in severe cases, neurological dysfunction leading to a seizure, stroke, or coma. About 5 individuals per 1 million people in the EU experience a TTP episode each year. TTP has a mortality of up to 15%, and a high recurrence rate.

### **About TGD001**

TGD001 is an innovative thrombolytic agent that activates the natural clot lysis cascade after binding to Von Willebrand factor in blood clots. It is a so-called fusion protein consisting of the catalytic domain of urokinase, which causes the clot breakdown, and a single-chain antibody that binds to von Willebrand factor (VWF). VWF is present in blood clots and binding to VWF directs the urokinase domain of TGD001 to the blood clot, which aims to avoid systemic effects. Urokinase activates plasmin, the natural enzyme in human blood that breaks down the fibrin and VWF fibers that form clots. By dissolving the blood clots, TGD001 is expected to restore the blood flow to the organs and thus limiting ischemic organ damage. This is recognized by the COMP as the basis of significant benefit over the existing available therapies.



We would like to express our gratitude to the researchers, healthcare professionals, and our amazing team who have contributed to the development of TGD001. The company remains steadfast in its mission to transform the lives of patients with rare and severe diseases through cutting-edge science and innovation.

### **About TargED Biopharmaceuticals**

TargED Biopharmaceuticals B.V. is a Netherlands based biotechnology company that develops first-in-class biological drugs to improve treatment of thrombosis. TargED stands for Targeted Enzyme Delivery. Their biological drugs are unique by using single-chain antibodies (“VhH”) to deliver enzymes to sites of thrombosis, enabling ‘targeted’ thrombolysis. The lead compound is TGD001, which is currently under development for the treatment of thrombotic thrombocytopenic purpura (TTP) and Acute Ischemic Stroke (AIS). The objective is to accelerate thrombolysis in all forms of thrombosis, irrespective of the thrombus composition.

TargED, a spin-off of the University Medical Center Utrecht, was founded in July 2020. Since then, TargED raised more than €45 million of funding, supporting its growth to become a clinical-stage company.

For more information, please visit [www.targedbio.com](http://www.targedbio.com)

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