TargED Biopharmaceuticals doses first participant in Phase 1 clinical trial of TGD001, a groundbreaking thrombolytic in acute ischemic stroke (AIS) and TTP

- *TGD001 is being developed to offer patients with clotting disorders a faster, safer and more effective treatment than today's standard of care – saving lives and reducing disability*
- *Following the Phase 1 safety study, TargED intends to further develop TGD001 in patients with AIS and iTTP (a rare micro-thrombotic disorder) in the second half of 2025*

Utrecht, the Netherlands, 19 December 2024 - TargED Biopharmaceuticals ("TargED"), a private biotechnology company focused on developing improved treatments for thrombotic diseases, today announces the start of clinical development with the dosing of the first participant with TGD001.

TGD001 is a groundbreaking thrombolytic designed to break down clots of all sizes and compositions – from large clots which cause AIS, down to 'micro-clots' which cause conditions such as the rare, life-threatening disorder immune-mediated thrombotic thrombocytopenic purpura (iTTP).

The first study participant was successfully dosed yesterday [Dec 18] at a Phase 1 clinic in Germany. The study's design is a randomized, double-blind, placebo-controlled single-ascending dose study to evaluate TGD001's safety, tolerability and pharmacokinetics in healthy volunteers (EU CT ID number: 2024-514931-63-00).

First safety read-out from the Phase 1 study is expected by mid-2025. TargED plans to continue clinical development with two proof-of-concept trials in the second half of 2025: a Phase 2a trial in AIS patients and a Phase 1b trial in iTTP patients, for which TargED received EU Orphan Drug Designation in July 2024 from the European Commission.

Kristof Vercruysse, Chief Executive Officer and co-founder of TargED, said: "The dosing of the first participant with our lead compound TGD001 marks a watershed in the treatment of thrombotic disorders. Rapid treatment with highly effective thrombolytics that are well tolerated and don't exaggerate the risk of bleeding is essential to reduce the risk of serious disability or even death from thrombotic disorders. Today's thrombolytics show efficacy but their significant drawbacks greatly limit their safety and effectiveness, and result in restricted utility. TGD001 is specifically designed to address these shortcomings. Its unique mode of action allows for blood clots of all sizes and compositions to be targeted and rapidly dissolved without increasing bleeding risk, resulting in clinical benefit for a broad population of patients."

Steven de Maat, Chief Scientific Officer and co-founder of TargED, said: "Acute ischemic stroke is one of the biggest causes of preventable death and disability in the world today, but up to 80% of AIS patients cannot be treated with currently available thrombolytics such as intravenous alteplase (tPA). Meanwhile, there is significant room for improvement in the treatment of iTTP. Despite recent advances, 15% of iTTP patients who receive treatment do not survive, and half of those who live still suffer long term health impairment. TGD001 has the potential to greatly improve outcomes in both these indications and, we believe, in other thrombotic indications as well."

TGD001 is a first-in-class 'fusion protein' drug with a unique two-step mode of action. It targets clots via an antibody fragment that binds to a non-functional domain of von Willebrand Factor (VWF), a protein

present in all types of thrombi. Then, in the immediate vicinity of the thrombus, it activates the endogenous enzyme system that degrades both VWF and fibrin, two key clotting ingredients which form the structural bond in the thrombus. This mode of action enables rapid and effective thrombolysis without systemic activation of the lysis cascade and the resulting bleeding risk. TGD001 is administered as short or bolus injection, making it a suitable therapeutic for the emergency, in-patient and first-response setting.

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For more information please contact:

TargED
Kristof Vercruysse
Chief Executive Officer
E: info@targedbio.com

Optimum Strategic Communications
Mary Clark, Stephen Adams, Karsa Ambikaibakan
T: + 44 203 821 6420
E: targedbio@optimumcomms.com

Notes to editors:

About TargED Biopharmaceuticals
TargED Biopharmaceuticals B.V. is a Netherlands based biotechnology company developing first-in-class biological drugs to improve treatment of thrombosis. TargED stands for Targeted Enzyme Delivery. TargED's biological drugs are unique by using antibody fragments ("VhH") to deliver enzymes to sites of thrombosis, enabling 'targeted' thrombolysis. Its lead compound TGD001 is currently under development for the treatment of immune-mediated thrombotic thrombocytopenic purpura (iTTP) 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.targedbiopharmaceuticals.com www.linkedin.com/company/targedbiopharmaceuticals/

Acute Ischemic Stroke (AIS), which occurs when one or more clots block blood flow to part of the brain, accounts for around 90% of all strokes. Worldwide, around 18 million AIS incidents take place every year, resulting in 5 million deaths and 5 million people permanently disabled. A delay in diagnosis and treatment can lead to loss of brain tissue, causing permanent disability or death. Standard of care thrombolysis must be initiated within 4.5 hours of ischemic stroke, since after this time the risk of tPA-induced bleeding outweighs the potential benefit. Due to their limitations, up to 80% of AIS patients are ineligible for current thrombolytics like tPA.

Immune-mediated thrombotic thrombocytopenic purpura (iTTP) is a rare, life-threatening blood disorder characterized by sudden episodes of clot formation in small blood vessels throughout the body. Blood flow to organs such as the kidney, heart, and brain is disrupted, causing functional failure. There are about 7,500 incidents of iTTP a year worldwide and it particularly affects young women, sometimes after pregnancy. Without treatment, up to 90% of cases are fatal. Treatment in specialized centers cuts

mortality to 15%; however, of the survivors, half still suffer long-term complications. Current treatment is complex, involving blood plasma exchange, immune suppression and administration of the drug caplacizumab, which stops new clot formation. To date, no thrombolytic – i.e. a drug that clears existing thrombi – is licensed in iTTP due to the perceived inherent bleeding risk of patients with thrombotic microangiopathies and the systemic thrombolytic activity of currently licensed thrombolytics. TGD001 has the potential to resolve iTTP attacks very rapidly, saving organ tissue and preventing disability and death.