

TargED Biopharmaceuticals Presents Positive Clinical Results for TGD001 from Phase 1 Study During Oral Session at ASH 2025

- TGD001 was well tolerated with no dose-limiting toxicities across all dose cohorts, exhibiting potent thrombolytic activity
- New preclinical data demonstrate efficacy across multiple *in vivo* models of thrombosis
- TargED will rapidly advance clinical development of TGD001 in several thrombotic diseases

Utrecht, the Netherlands, December 8, 2025 – TargED Biopharmaceuticals B.V., a clinical-stage biotechnology company developing next-generation targeted treatments for thrombotic diseases, today announced positive results from a Phase 1 clinical study on its lead investigational thrombolytic fusion protein, TGD001, in an oral presentation at the 67th American Society of Hematology (ASH) Annual Meeting in Orlando, Florida. The first-in-human results from 34 healthy study participants demonstrate that TGD001 is safe and was well tolerated with no spontaneous bleeding events across four ascending dose cohorts.

“These are the first clinical data to validate the excellent safety profile and promising pharmacological activity of this novel thrombolytic with an exciting mechanism of action,” said Marie Scully, Professor of Haemostasis and Thrombosis at the University College London. “Despite some treatment advancements in the last decade, current treatments have limited efficacy in removing clots, which remains the major barrier to full recovery for many patients. This thrombolytic could be a game changer with its potential power to degrade clots across a range of thrombotic indications. I am looking forward to participating in the clinical development of TGD001 with my colleagues around the globe.”

“The very positive first-in-human results are an important milestone, as they enable us to rapidly advance the clinical development of TGD001 for the treatment of acute ischemic stroke and thrombotic microangiopathies,” said Josefin-Beate Holz, MD, Chief Medical Officer at TargED Biopharmaceuticals. “With this first-in-class, universal thrombolytic, we have the opportunity to transform the treatment paradigm for patients suffering from thrombotic diseases or events.”

In addition to the Phase 1 study data, TargED presented new preclinical data demonstrating the broad potential of TGD001. The presentation showcased positive results in four established *in vivo* models of acute ischemic stroke, representing distinct clotting triggers and clot compositions, as well as in two *in vivo* models of thrombotic thrombocytopenic purpura (TTP), a thrombotic microangiopathy. Together, these findings support TGD001’s breakthrough potential to dissolve clots of different composition and location across a range of thrombotic indications.

Phase 1 Study Highlights

The Phase 1 clinical trial was a first-in-human, randomized, double-blind, placebo-controlled single-ascending dose study, evaluating the safety, tolerability, and pharmacokinetics (PK) of intravenously administered TGD001.

Safety and Tolerability

- The rapid (1-minute infusion) intravenous administration of TGD001 was well-tolerated at all dose levels with no dose-limiting toxicities
- No severe adverse events and no spontaneous bleeding events were reported
- All treatment-emergent adverse events (TEAEs) were transient and moderate in nature
- Immunogenicity: No treatment-emergent anti-drug antibodies were detected

Pharmacology and Efficacy

- Evidence of rapid and dose-dependent plasminogen activation that coincided with an increase in fibrin degradation products (D-dimer) was demonstrated by pharmacodynamic biomarkers
- Results suggest potent thrombolytic activity without causing safety concerns
- Pharmacodynamic changes occurred within minutes and were reversible, an ideal profile for a thrombolytic agent

Study Population and Baseline Characteristics

- 34 healthy male participants; 24 participants received TGD001 and 10 received placebo
- TGD001 was administered intravenously as a single injection at four different dose levels: 1.0 mg, 1.4 mg, 1.7 mg and 2.0 mg
- Median age: 38 years (SD 10 years)

The full abstracts will be published in a special online issue of Blood, ASH's official peer-reviewed journal.

TargED plans to initiate two Phase 1/2 clinical proof-of-concept studies evaluating TGD001 as a potential treatment for patients with acute ischemic stroke (AIS) and thrombotic microangiopathies (TMAs), including immune thrombotic thrombocytopenic purpura (iTTP).

About TargED Biopharmaceuticals

TargED Biopharmaceuticals B.V. is a clinical-stage biotechnology company pioneering targeted enzyme delivery for improved treatment of thrombotic diseases. Founded in 2020 as a spin-off from the University Medical Center Utrecht, the company has raised more than €45 million to advance its pipeline and was awarded a prestigious blended finance grant from the European Innovation Council (EIC) Accelerator Program in 2025. For more information, please visit targedbiopharmaceuticals.com

About TGD001

TGD001 is a first-in-class, targeted thrombolytic designed to treat a broad range of thrombotic events. As an antibody-enzyme fusion protein, it binds to von Willebrand factor (VWF) and promotes site-specific clot breakdown through the enzyme urokinase. Unlike currently approved therapies, TGD001 leverages the ubiquitous presence of VWF in all clots, enabling broad clot lysis by degrading both VWF and fibrin. TGD001 has been evaluated in a Phase 1 first-in-human clinical trial and will be further developed for the treatment of acute ischemic stroke (AIS) and thrombotic microangiopathies (TMAs), including immune thrombotic thrombocytopenic purpura (iTTP).

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