

Category

Best Startup

General Information**Company Name ***

TargED Biopharmaceuticals B.V.

Turnover and/or Funding

45 million funding (Series A & Non-dilutive) .

We have recently been awarded a prestigious blended finance grant from the European Innovation Council under the EIC Accelerator program. This funding consists of a 2.5M grant and up to €10 million in equity investment.

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457

Sub-Category *

Biotechnology

Background

**Corporate history (creation, key milestones, main funding,...)Information on Condition / Disease and need for solution / product (prevalence, existing treatments / solutions)
(please be as specific as possible in your description; limit 500 words)**

TargED Biopharmaceuticals B.V. is a Dutch biotech company founded in 2020 as a spin-off from UMC Utrecht following a seed investment of 850K Euro. In February 2022, TargED successfully raised €39 million in Series A financing to advance the clinical development of its lead compound, TGD001, for the treatment of immune-mediated thrombotic thrombocytopenic purpura (iTTP) - an orphan indication - and acute ischemic stroke (AIS). The company also secured a €5 million in non-dilutive funding through the Dutch Government's Innovation Credit program.

Since its inception with four founders, TargED has grown to a team of over 20 professionals and continues to expand. The company has completed all required toxicology studies and clinical GMP manufacturing to support the submission of its Clinical Trial Application (CTA).

TargED is now finalizing its healthy volunteer study and has been granted Orphan Drug Designation for TGD001 in both Europe and the United States. The company is on track to initiate its first clinical trial in AIS in Q4 2025, marking a major milestone in its mission to bring its innovative treatment to patients with high unmet medical needs.

From the 18 Million Acute Ischemic Stroke patients per year, only 20% is eligible to receive thrombolytic agents and only 10% will receive a mechanical removal of the thrombus. This leaves up to 80% of the patients that will not receive a suitable treatment today. The patients that do receive a

treatment still face threatening outcomes as 1/3 dies, 1/3 is permanently disabled and only 1/3 has a fully good outcome. The current treatment needs to be administered within 4,5 hours after the onset of clinical symptoms and come with major bleeding complications and neurotoxicity. The standard of care is currently off patent and has been used for the last 20 years (tissue Plasminogen Activator (tPA) e.g. Altoplase, Tenecteplase).

In the Orphan Indication there is currently no thrombolytic agent available as treatment is a combination therapy of Plasma Exchange, Immune therapy and a blood clot prevention drug (Cablivi). This results in a suboptimal treatment where attack duration is approximately 3 to 4 days. Our compound has the ability of decreasing the attack duration to less than 1 day, yielding in less tissue damage and better outcome for these patients.

After the COVID Pandemic it has also been seen that quite some patients were suffering from uncontrollable thrombosis where no existing therapeutic could help, here TGD001 could potentially offer a solution.

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History of the development of the solution/product (Intellectual Property, preclinical and clinical datas, development collaborations) *

(please be as specific as possible in your description; 500 words)

At the end of 2015, Coen Maas, one of the co-founders of TargED, received a Landsteiner Research Grant (€295k) for the idea of using nanobodies to target functional compounds (mostly enzymatic domains) to the site of disease. The combination of a nanobody and functional compound is a "fusion-protein"; a single polypeptide. Together with Steven de Maat, also co-founder of TargED, he developed multiple fusion proteins. In 2018, Coen and Steven, filed a patent application covering TGD001 technology and variants in multiple thrombotic indications. Since 2019, in vivo Proof-of-Concept (POC) was established for TGD001 in aTTP mouse models and in vivo POC of Microlyse in different relevant AIS mouse models (Both published in BLOOD). The company is currently finalizing its first healthy volunteer study and preparing to launch its first clinical trial in AIS, with the goal of achieving first-in-patient Proof of Concept data by the end of 2026.

Between 2016 and TargEDs formal establishment in July 2020 various grants were awarded:

- o In 2016, the founding team was awarded the Désiré Collen Award from the International Society for Fibrinolysis and Proteolysis for most innovative fibrinolytic therapy.
- o In 2018, the founding team was awarded the Winner of the Investor Forum at the Dutch Life Sciences conference.
- o In 2018, the founding team received the CONTRAST Young Talent Program voucher (CVON) for most innovative treatment of stroke.
- o In 2019, TargED got awarded with a Take-Off Phase 1 which enabled the finalization of the lead compound and delivered in vivo PoC of TGD001 in aTTP mouse models in early 2020.
- o In 2020, TargED got awarded with a PPS allowance which is used to establish PoC in mouse models of stroke, among other things.

Since July 2020, TargED was granted with additional grants:

- o The MIT R&D with the aim to develop a new biomarker test to assess the efficacy of TGD001 during clinical studies.
- o The NWO Take-Off Phase 2 with the aim to perform the manufacturing steps and preclinical toxicology and pharmacokinetics studies.

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Why this drug or device is innovative, the broad implications for future research, and/or how it will improve the human condition *

We are developing our proprietary compound TGD001 to effectively treat thrombosis, a common phenomenon defined by the formation of blood clots that obstruct the blood flow in the vasculature, which can result in severe tissue and organ damage and even death. Interestingly, the clinical presentation and clot composition of thrombosis is correlated with its location within the vasculature: thrombosis in the macrovasculature is characterized by fibrin-rich thrombi whereas thrombosis in the microvasculature is fibrin-poor. In both situations, however, von Willebrand Factor (vWF) is always present. TGD001 is a first-in-class fusion protein composed of a vWF-targeting nanobody domain and plasminogen-activating catalytic domain. Through this composition, TGD001 has the unique ability to enable targeted and local breakdown of thrombi by effectively degrading both vWF and fibrin, making it applicable for the treatment of all thrombi regardless of their composition. Current available therapies (Tenecteplase, Alteplase) need to bind to fibrin in order to activate plasminogen into plasmin which will cleave the blood clot, unfortunately fibrin is not always present or very difficult to bind to.

Our motto is "Time is Tissue" and by targeting vWF, which is present in all forms of thrombosis, TGD001 could potentially become a first generalistic thrombolytic medication that could be administered in all suspected thrombosis patients yielding to effective blood clot breakdown and hence, saving tissue with very limited side effects.

This is unthinkable for current treatments as they need imaging diagnostic (CT scan, MRI) to confirm the thrombotic nature of the disease. If administered to e.g. hemorrhagic stroke patients, these patients will bleed to death. TGD001 is designed in such a way that it potentially only works on the blood clot at hand and will not additionally aggravate bleeding risk which needs to be further investigated. TGD001 has the potential to change the treatment paradigm of thrombosis drastically (Administration direct at location of patient without need for imaging diagnostic, storage next to AED (Automated External Defibrillator), transportation via Drone for difficult to reach geographical regions etc ...)

As Thrombosis is the leading cause of death worldwide, accounting for over 16 million deaths per year (World Health Organization, 2020), TGD001 could impact the human condition and thus provide better outcomes for these patients suffering from Thrombosis. Treatment for more patients, resulting in fewer deaths and less disabilities, has the potential to reduce the economic costs of healthcare to society.

words remaining :

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Please provide appropriate references (PubMed, Abstract, Website) *

www.targedbio.com

<https://targedbiopharmaceuticals.com/app/uploads/2024/01/bloodbld2021011776.pdf>

https://targedbiopharmaceuticals.com/app/uploads/2024/01/VWF-targeted-thrombolysis-to-overcome-rh-tPA-resistance-in-experime_2022_Blo.pdf

<https://ashpublications.org/blood/article/143/20/2089/514748/Plasmin-cleaved-von-Willebrand-factor-as-a>

*Kindly clearly label your files with company name and asset name.