Star Therapeutics Receives FDA Rare Pediatric Disease and Breakthrough Therapy Designations for VGA039 in Von Willebrand Disease Prophylaxis

VGA039 is a once monthly, subcutaneously self-administered investigational therapy for the treatment of bleeding disorders, initially being evaluated for von Willebrand disease (VWD)

Pivotal Phase 3 trial of VGA039 in adolescent and adult patients with VWD currently enrolling

SOUTH SAN FRANCISCO, CA, April 21, 2026 – Star Therapeutics, a late clinical-stage biotechnology company focused on the discovery and development of life-changing therapies for diseases with significant unmet need, today announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation (RPDD) and Breakthrough Therapy designation (BTD) to Star's lead program, VGA039, for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with von Willebrand disease (VWD). VWD, the most common inherited bleeding disorder in which the blood does not clot properly, is a serious and life-threatening disease. VGA039 is a differentiated monoclonal antibody therapy that targets Protein S, with dual actions promoting platelet attachment and enhancing fibrin deposition to restore hemostasis.

"People living with VWD may face serious health complications, including frequent bleeding and hospitalizations that can significantly impact their quality of life. The FDA's decision to grant both RPDD and BTD to VGA039 underscores the urgent need for new treatments for these patients, as well as Star's potential to make a meaningful difference," said Gary Patou, M.D., Chief Medical Officer of Star Therapeutics. "VGA039 could be transformative for patients, as it is designed to prevent or reduce bleeding across all types of VWD while reducing treatment burden via its once monthly, subcutaneous dosing regimen. We are excited to continue to partner with physicians, patients and advocacy organizations to enroll people aged 12 and over with all types of VWD in our ongoing, pivotal Phase 3 study evaluating VGA039 prophylaxis. We aim to bring this new treatment to people with VWD as efficiently as possible."

The FDA's RPDD is intended to encourage the development of therapies for serious or life-threatening rare diseases that primarily affect individuals aged 18 years or younger and impact fewer than 200,000 people in the U.S. Upon approval of a Biologics License Application (BLA), this designation makes Star eligible to receive a Priority Review Voucher (PRV), which may be redeemed to obtain priority review for a subsequent marketing application or transferred or sold to another sponsor. According to the National Organization for Rare Disorders, as of 2025, approximately 63 rare pediatric disease PRVs had been awarded across 47 conditions since the program began in 2012.

Building upon the Fast Track designation that VGA039 was awarded in 2025, the FDA's BTD aims to expedite the development and review of therapies intended to treat serious conditions and address unmet medical needs. This designation is supported by interim data from the Phase 1/2 multidose study of VGA039 in adult and adolescent patients with VWD. These data were presented at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2025, demonstrating substantial bleed reductions across all types of VWD and all types of bleeds.

About VGA039
VGA039 is an investigational monoclonal antibody therapy with a novel mechanism of action that targets Protein S, with dual actions promoting platelet attachment and enhancing fibrin deposition to restore hemostasis. VGA039 has the potential to be a universal hemostatic therapy that can treat numerous bleeding disorders, starting with all types of von Willebrand disease (VWD). As a subcutaneously self-administered investigational antibody therapy with a convenient once monthly dosing regimen, VGA039 has the potential to meaningfully improve convenience and quality of life for patients. VGA039 has received Fast Track, orphan drug, rare pediatric disease and Breakthrough Therapy designations from the U.S. Food and Drug Administration (FDA). VGA039 has advanced into a Phase 3 study (NCT07115004), VIVID-6, a global single arm cross-over study designed to investigate the safety and efficacy of subcutaneous administration of VGA039 as prophylaxis for bleeding in patients with every type of VWD. For additional information on our VIVID trials of VGA039, including how to enroll, please visit the website here.

About von Willebrand disease
Von Willebrand disease (VWD) is the most common inherited bleeding disorder in which the blood does not clot properly, caused by low or defective von Willebrand factor (VWF). VWD patients may experience excessive bleeding with varying severity and frequency, negatively impacting their daily lives. Current therapies for VWD prophylaxis include factor replacement therapies requiring multiple intravenous (IV) infusions every week. VWD affects more than 134,000 patients in the United States.

About Star Therapeutics
Star Therapeutics is a late-stage biotechnology company focused on the discovery and development of life-changing therapies for diseases with significant unmet need. Star's leadership team brings deep expertise in antibody discovery, development, commercialization and strategic M&A, with a proven record of value creation across multiple biotechnology companies. For more information, please visit Star-Therapeutics.com and follow us on LinkedIn and X.

Contacts:

Media:
1AB
Katie Engleman
katie@1abmedia.com

Investors:
Star Therapeutics
Scott Robertson
scott@star-therapeutics.com