

Qualitative Study Exploring the Lived Experiences of Adolescents and Adults with von Willebrand Disease Treated with VGA039 (Latarcibart) and Their Families in the VIVID-3 Phase 1/2 Clinical Study

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Introduction

- Von Willebrand disease (VWD) is characterized by frequent, prolonged, and excessive bleeding that can substantially affect long-term outcomes and quality of life
- Despite available treatments, many people with VWD continue to experience physical limitations, psychosocial burden, and treatment-related challenges
- Latarcibart is an investigational, once-monthly subcutaneous (SC) monoclonal antibody that targets protein S with dual action on TFPIα and activated protein C pathways, promoting platelet deposition and enhancing fibrin accumulation to restore haemostasis¹
- The VIVID-3 phase 1/2 trial was designed to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of latarcibart administered SC every 4 weeks to patients with any type of VWD over 17 weeks^{2,3}
- The FDA has emphasized the importance of incorporating the patient voice into clinical trials and regulatory decision-making to support the development of meaningful therapies for patients⁴
- To complement clinical endpoints, qualitative insights from patients and caregivers are needed to understand the holistic experience with investigational treatments like latarcibart

Objective

- This pilot study characterizes the lived experience and quality of life impact in a small sample of patients with VWD and a parent caregiver before and after treatment with a novel agent, latarcibart, during the VIVID-3 study

Methods

- This is an IRB-approved, non-interventional, pilot study at a single center with three participants from VIVID-3 and one family member
- Eligible participants were ≥ 12 years old, English-speaking, and completed the VIVID-3 study or are a caregiver
- Participants completed a ~60-minute web-video semi-structured interview using an interview guide developed for this study
- Interview domains included bleeding experience; emotional, physical, social, and daily functioning; navigating work/school; perceived QoL; perceptions of treatment logistics & usability; thoughts about trial participation; and future thoughts
- All interviews were audio-recorded, transcribed, and analyzed using MAXQDA software
- Results include the number of participants that reported the experience and representative quotes

Results

Participant Characteristics and Efficacy Endpoint Results from VIVID-3

Age	Gender	VWD Type	% Reduction in Annualized Bleeding Rate in VIVID-3 ²
15-year-old	Female	Type 1	73%
22-year-old	Female	Type 2M	87%
22-year-old	Female	Type 1C	81%
Parent Caregiver	Female	NA	NA

Summary of Common Themes Among Participants

Since receiving VGA039 (latarcibart), all participants reported

- Easier to control bleeding (4/4)
- Improvement in menstrual bleeding, such as shorter duration and reduced heaviness of flow (4/4)
- Improvement to epistaxis (3/4)
- Increase in activities they had previously avoided (2/4)
- Improvement in the emotional impact of VWD, including less worry about bleeding episodes, and improvements in school/work participation, such as fewer missed days (4/4)
- Despite reporting significant pre-treatment symptom burden, some participants perceived their overall QoL as comparable to peers without VWD (n=2) or a little worse (n=1), suggesting the presence of a disability paradox within the VWD population
- Participants found it convenient to receive subcutaneous Q4W VGA039 (latarcibart) (3/4)
- All participants have continued into the open-label extension study

Key Domains

	Before VGA039	After VGA039
Bleeding experience	“...pretty much every morning I woke up, I would have blood in my nose.” “I’m constantly worried that I’m bleeding through my pants or I’m having trouble controlling that. [Menstrual bleeding] would be my biggest concern.” “The bruises could show up anywhere on my body. I don’t think I’ve ever not had a bruise on a specific limb or something.”	“My nosebleeds have been completely different. I haven’t had an hour-long one in months.” “After the drug, she really hasn’t had a nosebleed, and her periods are definitely cut in half.” (caregiver parent) “Just, you know, smaller bruises. Not as frequent.”
Daily life experience	“... I’ve been more cautious about my body more than others just because of the factor of if something crazy serious happens, injury happens, I need to make sure I know how to take the right steps, and I don’t think that thought crosses other people’s minds that don’t have a disorder like this.”	“I feel like a lot of my issues are way less serious than before. So, it’s amazing to me. So, I’m trying to think of it in the perspective of if I didn’t have a bleeding disorder.”
Emotional impact	“[Bleeds] would come out of nowhere. So, I wasn’t ever really prepared for them, but when I was having them, there were a couple of times where I would have intense panic attacks because it just wasn’t stopping, and I didn’t know what to do.”	“I feel confident that there’s something working in my body, and that I don’t need to have that thought on my mind all of the time. That like, okay, if something really bad happens, this is what I’m going to do because I trust my body to react the way it should react.”
School/work experience	“At work and school, it would be very inconvenient, especially in work, because I have to be holding my nose and controlling a nosebleed, while also trying to do something.”	“I just think it’s a lot better overall. She has a longer break between when her period ends and her next one starts. So, she’s definitely missing less school.” (caregiver parent)
Social impact	“If it’s big bruises that I would have, I would just feel insecure of what people would think of my relationships with others. Like if they viewed it as abuse or something.”	“I can definitely go out more. I have more energy when I’m not bleeding out. So, in general, I feel like I just have more energy, which is really what I wanted.”

Conclusions

- This qualitative pilot study provides patient-centered context on the burden of VWD before and after VGA039 (latarcibart), a novel once-monthly, subcutaneous therapy, helping to identify domains of impact to be used alongside existing clinical trial outcome measures
- Although the pilot sample size was small, participants reported decreased impact of bleeding symptoms, as well as improved perception of QoL and bleed control with latarcibart
- These findings support conduct of a future, larger qualitative study, which will be conducted alongside VIVID-6, a phase 3 trial of latarcibart⁵

References

- Sakurai, Y. et al. A protein S-targeting monoclonal antibody, VGA039, improves both primary and secondary hemostatic activity of von Willebrand disease patient blood in an ex vivo vascularized hemostasis-on-a-chip. *Blood* 2025; 146 (Supp 1):305.
- Wheeler, A.P. et al. Subcutaneous, every-four-week maintenance dosing of a novel protein S antibody is well-tolerated and substantially reduces bleeding rates: Results from A phase 1/2 multidose study of VGA039 in patients with von Willebrand disease. *Blood* 2025; 146 (Supp 1):308.
- VIVID-3 (Phase 1/2). ClinicalTrials.gov Identifier: NCT05776069. <https://clinicaltrials.gov/study/NCT05776069>.
- U.S. Food and Drug Administration. FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient’s Voice in Medical Product Development and Regulatory Decision Making. Updated October 23, 2025. Accessed April 22, 2026.
- ClinicalTrials.gov Identifier: NCT07115004. VIVID-6.