Viridian Therapeutics Announces Positive Topline Results from Elegrobart Phase 3 REVEAL-2 Clinical Trial in Chronic Thyroid Eye Disease

2026年 5月 5日

- REVEAL-2 met its primary endpoint with a highly statistically significant treatment effect -

- Elegrobart Q4W and Q8W achieved 50% and 54% proptosis responder rates (PRR) at week 24, respectively, versus 15% placebo, both highly statistically significant results (p < 0.0001) -

- Elegrobart Q4W achieved a statistically significant 61% diplopia responder rate at week 24, versus 38% placebo (p = 0.0118) -

- Elegrobart was generally well tolerated in both dose groups, with a safety profile consistent with REVEAL-1 and low rates of hearing impairment -

- Elegrobart is the only subcutaneous program to demonstrate positive phase 3 data in both active and chronic TED pivotal clinical trials and has the potential to be the first autoinjector treatment for TED; BLA submission on track for Q1 2027 -

- Conference call and webcast to be held today, May 5th at 8:00 a.m. ET -

WALTHAM, Mass.--(BUSINESS WIRE)-- Viridian Therapeutics, Inc. (Nasdaq: VRDN), a biotechnology company focused on discovering, developing, and commercializing potentially best-in-class medicines for autoimmune and rare diseases, today announced positive topline data from the REVEAL-2 phase 3 clinical trial of elegrobart in patients with chronic thyroid eye disease (TED). Elegrobart is a subcutaneously delivered, half-life-extended monoclonal antibody targeting the insulin-like growth factor-1 receptor (IGF-1R). REVEAL-2 evaluated two dosing regimens, every four weeks (Q4W) and every eight weeks (Q8W), compared with placebo.

“We are excited by today’s positive REVEAL 2 results and view these data as a major step forward for the chronic TED patient population. Given the IV-like proptosis response and our plans to launch with an at-home autoinjector, we believe elegrobart can meaningfully attract chronic patients to seek treatment. Elegrobart’s unmatched simplicity and convenience could uniquely drive expansion of the large and underserved chronic TED market,” said Steve Mahoney, President and Chief Executive Officer of Viridian Therapeutics. “With our anticipated launch of veligrotug, which is a short IV infusion course, and two positive phase 3 REVEAL pivotal clinical trials supporting both Q4 weekly and Q8 weekly subcutaneous dosing for elegrobart, our portfolio has the potential to offer anti-IGF-1R efficacy and safety in convenient treatment regimens for TED patients with active or chronic disease.”

“Chronic TED remains a challenging condition. Many patients have been living with this disease for years or decades and would benefit from an effective and convenient treatment option,” said John Mandeville, MD, PhD, an oculoplastic surgeon at Ophthalmic Consultants of Boston and who is also a clinical associate at the Massachusetts General Hospital. “These REVEAL 2 results demonstrate the potential for elegrobart to provide meaningful improvement in the signs and symptoms of TED in as few as three doses. What’s more, a simple autoinjector that patients can use at home could be an attractive option for many patients living with chronic disease.”

Elegrobart REVEAL-2 Phase 3 Topline Results

REVEAL-2 assessed the efficacy and safety of subcutaneous Q4W or Q8W elegrobart versus placebo in patients with chronic TED. The clinical trial enrolled 204 patients, randomized 1:1:1 to elegrobart Q4W (n = 70), elegrobart Q8W (n = 68), and placebo (n = 66).

REVEAL-2 Efficacy

REVEAL-2 met its primary endpoint for both the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) with high statistical significance (p < 0.0001). In addition, REVEAL-2 met all its proptosis key secondary endpoints in the Q4W and Q8W treatment arms with high statistical significance, and the Q4W treatment arm showed a statistically significant diplopia responder rate at week 24. Efficacy was generally consistent regardless of baseline Clinical Activity Score (CAS). Results from primary and all key secondary endpoints at week 24 are presented below:

		Elegrobart Q4W (*n* = 70)	**Elegrobart Q8W** (*n* = 68)	**Placebo** (*n* = 66)
Proptosis	Proptosis responder rate (exophthalmometry)	**50% (FDA Primary Endpoint)**	54%	15%
	p-value	*p < 0.0001*	*p < 0.0001*	*n/a*
	Overall responder rate (ORR)[1]	**47% (EMA Primary Endpoint)**	54%	15%
	p-value	*p < 0.0001*	*p < 0.0001*	*n/a*
	Proptosis mean change from baseline (exophthalmometry)	-1.9 mm	-2.1 mm	-0.5 mm
	p-value	*p < 0.0001*	*p < 0.0001*	*n/a*

Diplopia	Diplopia responder rate	61%	55%	38%
	p-value	*p = 0.0118*	*p = 0.0419*	*n/a*
	Diplopia complete resolution	44%	36%	25%
	p-value	*p = 0.0295*	*p = 0.1304*	*n/a*

Results with p < 0.025 are statistically significant.

[1]Participants with both proptosis and CAS response; CAS response defined as no worsening in CAS from baseline in study eye, without deterioration in fellow eye (≥2-point increase)

REVEAL-2 Safety

Elegrobart was generally well tolerated in REVEAL-2 with a safety profile consisting of adverse events generally expected from the anti-IGF-1R class, the vast majority of which were mild. Rates of hearing impairment were low in both the Q4W and Q8W treatment arms (4.1% and 8.8% placebo-adjusted rates, respectively). 91% of elegrobart-treated patients completed the full course of treatment, and there were no treatment-related serious adverse events (SAEs).

Elegrobart BLA Submission Expected in Q1 2027

- REVEAL-2 is the second successful pivotal phase 3 clinical trial for elegrobart, following positive results from REVEAL-1 in active TED.
- Viridian remains on track to submit a Biologics License Application (BLA) to the U.S. FDA for elegrobart in Q1 2027.
- If approved, elegrobart has the potential to offer a convenient, at-home treatment in as few as three doses for both active and chronic patients.

Veligrotug on Track with a PDUFA Target Action Date of June 30, 2026

- Viridian is prepared for the planned U.S. commercial launch for veligrotug, its lead program for TED. Viridian anticipates the veligrotug commercial and medical affairs infrastructure will support a potential elegrobart launch, if approved, with limited incremental investment.
- Veligrotug was granted Breakthrough Therapy Designation from the FDA, and its BLA is under Priority Review at the FDA with a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2026.

Conference call and webcast information

Viridian will host a conference call today at 8:00 a.m. ET to discuss the REVEAL-2 topline data.

- Dial-in (U.S.): (800) 715-9871
- Dial-in (International): +1 (646) 307-1963
- Conference ID: 7373356

A live webcast of the conference call can be accessed through the “Events” section in the Investors page of the Viridian Therapeutics website. Following the live webcast, an archived version of the call will also be available on the website.

About Viridian Therapeutics

Viridian is a biotechnology company focused on discovering, developing, and commercializing potential best-in-class medicines for patients with autoimmune and rare diseases. Viridian’s expertise in antibody discovery and protein engineering enables the development of differentiated therapeutic candidates for validated drug targets and disease-driving mechanisms in autoimmune and rare diseases.

Viridian is advancing multiple late-stage, anti-insulin-like growth factor-1 receptor (IGF-1R) candidates in the clinic for the treatment of patients with thyroid eye disease (TED). The company conducted a pivotal program for veligrotug, including two global phase 3 clinical trials, THRIVE and THRIVE-2, to evaluate its efficacy and safety in patients with active and chronic TED. THRIVE and THRIVE-2 reported positive topline data, meeting their primary endpoints and all secondary endpoints. Viridian is also advancing elegrobart as the potential first subcutaneous autoinjector for the treatment of TED. Viridian is conducting an ongoing pivotal program for elegrobart, including two global phase 3 pivotal clinical trials, REVEAL-1 and REVEAL-2, to evaluate the efficacy and safety of elegrobart in patients with active and chronic TED. REVEAL-1 and REVEAL-2 reported positive topline data, meeting their primary endpoints and multiple secondary endpoints.

In addition to its IGF-1R inhibitor portfolio, Viridian is developing an anti–thyroid-stimulating hormone receptor (TSHR) program designed as a potential therapy for TED and Graves’ disease.

Viridian is also advancing a novel portfolio of neonatal Fc receptor (FcRn) inhibitors, including VRDN-006 and VRDN-008, which have the potential to be developed in multiple autoimmune diseases.

Viridian is based in Waltham, Massachusetts. For more information, please visit www.viridiantherapeutics.com. Follow Viridian on LinkedIn and X.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of words such as, but not limited to, “anticipate,” “believe,” “become,” “continue,” “could,” “design,” “estimate,” “expect,” “intend,” “may,” “might,” “on track,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” or “would” or other similar terms or expressions that concern our expectations, plans and intentions. Forward-looking statements are neither historical facts nor assurances of future performance. Instead,

they are based on our current beliefs, expectations, and assumptions. Forward-looking statements include, without limitation, statements regarding: preclinical development, clinical development, and anticipated commercialization of Viridian's product candidates; Viridian's expectations regarding the anticipated timing or likelihood of regulatory submissions and approvals, including the anticipated approval of the BLA for veligrotug and the anticipated submission of a BLA for elegrobart in Q1 2027; elegrobart's potential to be the potential first subcutaneous therapy for the treatment of TED and its potential to launch commercially with an at-home autoinjector; the potential benefits of elegrobart for patients, including its viability as a compelling solution for patients living with both active and chronic disease and provide meaningful improvement in the signs and symptoms of TED in as few as three doses; Viridian's expectations with the market size and position; that the veligrotug commercial infrastructure will support a potential elegrobart launch, if approved, with limited incremental investment; elegrobart's potential to expand the market for products in TED, if approved; and Viridian's product candidates potentially being best-in-class.

New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements. Such forward-looking statements are subject to a number of material risks and uncertainties including but not limited to: potential utility, efficacy, potency, safety, clinical benefits, clinical response, and convenience of Viridian's product candidates; that results or data from completed or ongoing clinical trials may not be representative of the results of ongoing or future clinical trials; that the results of ongoing or future clinical trials may not support submission for regulatory approvals; the timing, progress and plans for our ongoing or future research, preclinical, and clinical development programs; changes to trial protocols for ongoing or new clinical trials; expectations and changes regarding the timing for regulatory filings; expectations and changes regarding the timing for enrollment and data; uncertainty and potential delays related to clinical drug development; the duration and impact of regulatory delays in our clinical programs; the timing of and our ability to obtain and maintain regulatory approvals for our therapeutic candidates; manufacturing risks; competition from other therapies or products; estimates of market size; other matters that could affect the sufficiency of existing cash, cash equivalents, and short-term investments to fund operations; our financial position; our future operating results and financial performance; Viridian's intellectual property position; that our product candidates may not be commercially successful, if approved; and other risks described from time to time in the "Risk Factors" section of our filings with the Securities and Exchange Commission (SEC), including those described in our most recent Annual Report on Form 10-K or Quarterly Report on Form 10-Q, as applicable, and supplemented from time to time by our Current Reports on Form 8-K. Any forward-looking statement speaks only as of the date on which it was made. Neither the company, nor its affiliates, advisors, or representatives, undertake any obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law. These forward-looking statements should not be relied upon as representing the company's views as of any date subsequent to the date hereof.

Investors
Greg Rossino
grossino@viridiantherapeutics.com

Media
Lisa Lopez
llopez@viridiantherapeutics.com

Source: Viridian Therapeutics, Inc.