Viridian Therapeutics Announces Positive Topline Results from Elegrobart Phase 3 REVEAL-1 Clinical Trial in Active Thyroid Eye Disease

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- REVEAL-1 met the primary endpoint of Q4W proptosis responder rate (PRR) with a highly statistically significant treatment effect -

- Elegrobart Q4W and Q8W achieved clinically meaningful 54% and 63% PRR versus 18% placebo at week 24 -

- Complete resolution of diplopia in 51% of patients treated Q4W versus 16% placebo at week 24 -

- Elegrobart was generally well tolerated in both dose groups with low rates of hearing impairment -

- REVEAL-2, a phase 3 clinical trial evaluating elegrobart in patients with chronic TED, is on track for topline readout in Q2 2026; BLA submission anticipated in Q1 2027 -

- Viridian ended Q4 2025 with $875 million in cash; the company anticipates existing cash, potential near-term milestones from its 2025 royalty agreement, and anticipated commercial revenues from veligrotug and elegrobart if approved, to fund current business plans through profitability -

- Conference call and webcast to be held today, March 30th 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 serious and rare diseases, today announced positive topline data from the elegrobart REVEAL-1 phase 3 clinical trial in patients with active 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-1 evaluated two dosing regimens, every four weeks (Q4W) and every eight weeks (Q8W), compared with placebo.

“We are excited to report these results from REVEAL-1, the largest pivotal clinical trial conducted in active TED to date, which position elegrobart as potentially the first ever subcutaneous autoinjector treatment for TED,” said Steve Mahoney, President and Chief Executive Officer of Viridian Therapeutics. “REVEAL-1 met its primary endpoint with high statistical significance. Elegrobart treatment drove robust proptosis responses in a treatment regimen comprised of as few as three subcutaneous doses. Further, in the Q4W arm, we saw clinically meaningful diplopia responses and diplopia resolution. Currently, the only marketed treatment for TED requires eight intravenous infusions and, despite low market penetration, annualized in 2025 to approximately $2B in revenues. We believe there is a significant opportunity with subcutaneous elegrobart in TED, including the potential to expand the market as an at-home and self-administered treatment option, if approved.”

“Subcutaneous elegrobart showed rapid and clinically meaningful reductions in proptosis and diplopia in REVEAL-1 with a highly convenient, well-tolerated dosing profile,” said Prem Subramanian, MD, PhD, professor of ophthalmology at Colorado University Anschutz, and chief of neuro-ophthalmology at the Sue Anschutz-Rodgers Eye Center. “Patients are seeking more treatment choices for TED, and there remains a clear need for a more conveniently administered therapy. I am very encouraged to see the data for elegrobart and believe it has the potential to reach more TED patients than an intravenous therapy and to provide them with an attractive treatment option.”

Elegrobart REVEAL-1 Phase 3 Topline Results

REVEAL-1 assessed the efficacy and safety of subcutaneous Q4W or Q8W elegrobart versus placebo in patients with active TED. The study enrolled 132 patients, randomized 1:1:1 to elegrobart Q4W (n=44), elegrobart Q8W (n=44), and placebo (n=44).

REVEAL-1 Efficacy

REVEAL-1 was highly statistically significant on its primary endpoint of Q4W proptosis responder rate and the key secondary endpoint of Q4W proptosis mean change from baseline, each at week 24 as measured by exophthalmometry. REVEAL-1 also assessed elegrobart Q8W versus placebo and additional clinically relevant endpoints for both the Q4W and Q8W dosing arms. Detailed results are presented below:

			Elegrobart (*n* = 44 per arm)	**Placebo** (*n* = 44)	**p-value**
Primary Endpoint	**Q4W**	**FDA:** Proptosis responder rate (exophthalmometry)	54%	18%	*p < 0.0001**
		EMA: Overall responder rate (ORR)	51%	16%	*p = 0.0001**
Key Secondary Endpoints	**Q4W**	Proptosis mean change from baseline (exophthalmometry)	-2.33 mm	-0.81 mm	*p < 0.0001**
		Clinical activity score (CAS) reduction to 0 or 1	57%	50%	*p = 0.24*
		Diplopia responder rate	71%	32%	*p = 0.0009*

		Diplopia complete resolution	51%	16%	*p = 0.0013*
	Q8W	Proptosis responder rate (exophthalmometry)	63%	18%	*p < 0.0001*
		EMA: Overall responder rate (ORR)	58%	16%	*p < 0.0001*
		Proptosis mean change from baseline (exophthalmometry)	-2.50 mm	-0.81 mm	*p < 0.0001*
		Clinical activity score (CAS) reduction to 0 or 1	69%	50%	*p = 0.03*
		Diplopia responder rate	54%	32%	*p = 0.05*
		Diplopia complete resolution	28%	16%	*p = 0.14*
Other Secondary Endpoints	**Q4W**	Proptosis responder rate (MRI)	50%	2%	*p < 0.0001*
		Proptosis mean change from baseline (MRI)	-2.04 mm	-0.22 mm	*p < 0.0001*
	Q8W	Proptosis responder rate (MRI)	36%	2%	*p < 0.0001*
		Proptosis mean change from baseline (MRI)	-1.99 mm	-0.22 mm	*p < 0.0001*

* Statistically significant. Key secondary endpoints below Q4W “CAS Reduction to 0 or 1” in the prespecified testing hierarchy and other secondary endpoints are nominally significant if below the statistically significant threshold of 0.025.

REVEAL-1 Safety

Elegrobart was generally well-tolerated 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 (11.3% and 2.3% placebo-adjusted rates, respectively), and all reports were of tinnitus, none of which were associated with reductions in hearing.

Elegrobart on Track with a BLA Submission anticipated in Q1 2027

- Topline data from REVEAL-2 for patients with chronic TED, the second pivotal phase 3 clinical trial of elegrobart, remains on track to read out in Q2 2026.
- Viridian anticipates submitting a Biologics License Application (BLA) to the U.S. FDA for elegrobart in Q1 2027.

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

- The veligrotug BLA is under Priority Review at the FDA with a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2026. Veligrotug also received both Breakthrough Therapy Designation and Priority Review from the FDA in 2025.
- Viridian is approaching full launch-readiness and is on track to support the PDUFA action date next quarter, with experienced teams in place across field sales, field medical affairs, market access, and patient services.
- Viridian anticipates the veligrotug commercial and medical affairs infrastructure will support a potential elegrobart launch, if approved, with limited incremental investment, providing an infrastructure to support multiple treatment options for patients within the Viridian TED portfolio.

Conference call and webcast information

Viridian will host a conference call today at 8:00 a.m. ET to discuss the REVEAL-1 topline data. The dial-in number for the conference call is (800) 715-9871 for domestic participants and +1 (646) 307-1963 for international participants. The conference ID is 7373356.

A live webcast of the conference call can be accessed through the “Events” page in the Investors section 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 biopharmaceutical company focused on discovering, developing, and commercializing potential best-in-class medicines for patients with serious 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. Both THRIVE and THRIVE-2 reported positive topline data, meeting the primary and all secondary endpoints of each study. Viridian is also advancing elegrobart as the potential first subcutaneous autoinjector for the treatment of TED, including two ongoing 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.

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; anticipated data results and timing of their disclosure, including elegrobart topline data from the REVEAL-2 trial; 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; elegrobart’s potential to be the potential first subcutaneous autoinjector for the treatment of TED; that the veligrotug commercial infrastructure will support a potential elegrobart launch, if approved; elegrobart’s potential to expand the market for products in TED, if approved; Viridian’s product candidates potentially being best-in-class; and that Viridian’s cash, potential near-term milestones from its 2025 royalty agreement and anticipated commercial revenues, if veligrotug and elegrobart are approved, will be sufficient to fund its business plans through profitability.

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, including as a result of a prolonged government shutdown; 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.

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