Viridian Therapeutics Announces Positive Topline Results from Veligrotug Phase 3 THRIVE-2 Clinical Trial in Patients with Chronic Thyroid Eye Disease

- Veligrotug met all primary and secondary endpoints with high statistical significance in THRIVE-2, achieving a week 15 proptosis responder rate (PRR) of 56% (placebo-adjusted PRR of 48%, p < 0.0001) -

- THRIVE-2 is the first global phase 3 study in patients with chronic TED to demonstrate a statistically significant and clinically meaningful 56% diplopia responder rate (placebo-adjusted rate of 31%, p = 0.0006) and 32% rate of diplopia complete resolution (placebo-adjusted rate of 18%, p = 0.0152) -

- Veligrotug was generally well-tolerated with 94% of patients completing their treatment course and a 9.6% placebo-adjusted rate of hearing impairment -

- BLA submission for veligrotug on track for second half of 2025; potential to transform the standard of care in TED with differentiated clinical profile demonstrated in the broadest population studied in a global phase 3 study across active and chronic TED patients to date -

- REVEAL-1 and REVEAL-2, global phase 3 clinical trials of subcutaneous VRDN-003 evaluating every four-week (Q4W) or every eight-week (Q8W) regimens, are both currently dosing and on track for topline data in 1H 2026; VRDN-003 is a half-life-extended anti-IGF-1R antibody with the same binding domain as veligrotug -

- Strong cash position of $753 million as of September 30, 2024; provides cash runway into the second half of 2027 through the anticipated commercial launch of veligrotug, topline results and BLA submission for subcutaneous VRDN-003, and multiple FcRn inhibitor clinical catalysts -

- Conference call and webcast to be held today, December 16th at 8:00 a.m. ET -

WALTHAM, Mass., December 16, 2024 — Viridian Therapeutics, Inc. (NASDAQ: VRDN), a biopharmaceutical company focused on discovering and developing potential best-in-class medicines for serious and rare diseases, today announced positive topline data from the THRIVE-2 phase 3 clinical trial of veligrotug (veli), an intravenously (IV) delivered anti-insulin-like growth factor-1 receptor (IGF-1R) antibody, in patients with chronic thyroid eye disease (TED). TED is an autoimmune condition characterized by inflammation, growth, and damage to tissues around and behind the eyes.

“We are extremely pleased to announce better-than-expected THRIVE-2 results generated in the broadest population of chronic TED patients studied in a global phase 3 study to date. We believe that these efficacy and safety results in only five infusions, combined with our compelling data from THRIVE, confirm the potential of veli to be the treatment-of-choice for all forms of active and chronic TED,” said Steve Mahoney, Viridian’s President and CEO. “The robustness and consistency of our data, similar to THRIVE, showed strong and rapid responses in categories that we believe matter most to patients including proptosis reduction, diplopia resolution and improvements in Clinical Activity Scores. This is the first product candidate to demonstrate a diplopia response and resolution rate in a global chronic TED phase 3 study. Our BLA preparation work is underway, and, if approved, we now believe veli will offer a differentiated commercial product profile to patients. The combined results of THRIVE and THRIVE-2, give us even higher conviction that our subcutaneous VRDN-003 program will deliver positive topline data in the first half of 2026, which would enable a BLA submission in the second half of 2026.”

“These data represent an incredible step forward for TED patients. I’ve been treating TED for over 30 years, and these results in the broadest population of TED patients are highly encouraging. Resolving double vision or even improving it in chronic TED patients can really change their lives,” said Steven Leibowitz, M.D., Associate Clinical Professor of Ophthalmology, Stein Eye Institute, University of California Los Angeles and THRIVE-2 investigator. “I see veligrotug’s potential product profile as highly compelling with a rapid onset of treatment effect, diplopia benefit across a broad TED population, shorter dosing regimen, and favorable safety profile.”

THRIVE-2 Phase 3 Topline Results

THRIVE-2 Clinical Activity Data

THRIVE-2 met all primary and secondary endpoints at the 15-week primary analysis timepoint after five infusions of veligrotug, showing statistically significant responses on all of the measured signs and symptoms of TED: proptosis, CAS, and diplopia. THRIVE-2 enrolled a total of 188 patients, randomized to veligrotug (n = 125) and placebo (n = 63). The mean time since onset of TED in this patient population was 69.8 months.

The key data at the primary efficacy analysis timepoint of 15 weeks are as follows:

Proptosis:

- Proptosis Responder Rate (PRR): 56% in veligrotug patients, compared with 8% in placebo patients (48% placebo-adjusted, $p < 0.0001$). PRR was statistically significant at all time points, including as early as 3 weeks after just one infusion, demonstrating a rapid onset of response. PRR is defined as at least a 2-millimeter (mm) reduction in proptosis from baseline in the study eye without worsening in the fellow eye (≥ 2 mm increase), as measured by exophthalmometry. PRR results as measured by MRI/CT were consistent with those measured by exophthalmometry at the primary efficacy analysis timepoint.

- Proptosis Mean Reduction: 2.34mm mean reduction in proptosis from baseline in veligrotug patients, compared with 0.46mm reduction in placebo patients (1.9mm placebo-adjusted, $p < 0.0001$).

Diplopia:

- Diplopia Response: 56% of veligrotug patients achieved a diplopia response, compared with 25% of placebo patients (31% placebo-adjusted, $p = 0.0006$). Rapid onset was observed as early as 6 weeks after just two infusions. Diplopia response is defined as patients achieving a reduction of at least 1 on the Gorman subjective diplopia scale at week 15, for those patients with diplopia at baseline (n = 102).

- Diplopia Complete Resolution: 32% of veligrotug patients achieved complete resolution of diplopia, compared with 14% of placebo patients (18% placebo-adjusted, $p = 0.0152$). Rapid onset was observed as early as 6 weeks after just two infusions. Diplopia resolution is defined as patients achieving a score of 0 on the Gorman subjective diplopia scale at week 15, for those patients with diplopia at baseline.

Clinical Activity Score (CAS):

CAS measures inflammatory signs and symptoms of TED, providing a composite score of pain, as well as redness and swelling of the eyelids and conjunctiva, on a scale from 0 to 7.

- CAS Reduction to 0 or 1: 54% of veligrotug patients achieved maximal or near-maximal therapeutic effect on CAS, compared with 24% of placebo patients (29% placebo-adjusted, $p = 0.006$), defined as reaching a CAS of 0 or 1, among patients with a CAS of ≥ 3 at baseline (n = 104).

- CAS Mean Reduction: 2.9-point mean reduction in CAS from baseline in veligrotug patients, compared with 1.3-point reduction in placebo patients (1.6-point placebo-adjusted, $p < 0.0001$), among patients with a CAS of $\geqslant 3$ at baseline.

Overall Response:

- Overall Responder Rate: 56% of veligrotug patients achieved an overall response, compared with 7% of placebo patients (50% placebo-adjusted, $p < 0.0001$). Overall Responder Rate is defined as achieving a proptosis response without worsening of CAS from baseline (≥ 1 point increase) and without worsening in the fellow eye in either proptosis (2 mm increase) or CAS.

THRIVE-2 Safety Data

- Generally Well-Tolerated: Veligrotug was generally well-tolerated with a safety profile consistent with previous veligrotug studies including THRIVE. The majority of adverse events (AEs) were mild, and 94% of veligrotug-treated patients completed their treatment course.

- Low Rate of Hearing Impairment: There was a 9.6% placebo-adjusted rate of hearing impairment AEs (12.8% incidence in veligrotug patients, compared with 3.2% incidence in placebo patients).

“Seeing the strong data presented today demonstrating that diplopia can in fact be improved in patients with long standing chronic TED is exciting. Together with the robust activity in both THRIVE and THRIVE-2, favorable safety profile, and a shorter dosing regimen, we believe veli is positioned to become a market leading TED therapeutic,” said Tony Casciano, Chief Commercial Officer. “Veli has the potential to be the only approved therapy with data in chronic patients included in the labeling. We believe the strength and completeness of veli’s pivotal program, the largest to date in TED, could not only ensure advantageous market access, but also expand utilization in patients unaddressed by current therapies. Veli’s profile itself is inspiring, and if approved, our commercial team is eager to get veli to patients.”

Subcutaneous VRDN-003, a Potential Best-In-Class Anti-IGF-1R, On Track to Report Topline 1H 2026

VRDN-003 is an IGF-1R antibody with the same binding domain as veligrotug and is believed to be the only anti-IGF-1R in development with an extended half-life.

Based on this shared binding domain, Viridian believes the topline results from THRIVE-2, in addition to the previously reported THRIVE results, reinforces the potential best-in-class profile of VRDN-003 to deliver clinical activity and safety consistent with veligrotug in a low-volume, infrequent, self-administered, subcutaneous injection that patients take at home.

Viridian is currently dosing patients in two global phase 3 clinical trials for VRDN-003, REVEAL-1 and REVEAL-2, in active and chronic TED, respectively. Viridian anticipates reporting topline data from these studies in the first half of 2026 and submitting a BLA for VRDN-003 for the treatment of TED by year-end 2026.

Conference Call and Webcast Information

Viridian will host a conference call today at 8:00 a.m. ET to discuss the THRIVE-2 topline data. The dial-in number for the conference call is (800) 715-9871 for domestic participants and (646) 307-1963 for international participants. The conference ID is 9934051.

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 engineering and developing 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 previously validated drug targets in commercially established disease areas.

Viridian is advancing multiple candidates in the clinic for the treatment of patients with thyroid eye disease (TED). The company is conducting a pivotal program for veligrotug (VRDN-001), 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 all the primary and secondary endpoints of each study. Viridian is also advancing VRDN-003 as a potential best-in-class subcutaneous therapy 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 VRDN-003 in patients with active and chronic TED.

In addition to its TED portfolio, Viridian is advancing a novel portfolio of neonatal Fc receptor (FcRn) inhibitors, including VRDN-006 and VRDN-008, which has 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.

Cautionary Note Regarding 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,” “estimate,” “expect,” “intend,” “may,” “might,” “on track,” “plan,” “potential,” “predict,” “project,” “design,” “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 and clinical development of Viridian’s product candidates veligrotug (formerly VRDN-001), VRDN-003, VRDN-006 and VRDN-008; the anticipated VRDN-003 topline data from THRIVE-2 in the first half of 2026; anticipated BLA submissions for veligrotug in the second half of 2025 and VRDN-003 in the second half of 2026, pending data; Viridian’s expectation that its data package will support a BLA for VRDN-003; the potential utility, efficacy, potency, safety, clinical benefits, clinical response, convenience and number of indications of veligrotug, VRDN-003, VRDN-006 and VRDN-008; veligrotug’s potential to be a market-leading therapeutic and the only approved therapy with data in chronic patients included in the labeling; Viridian’s product candidates potentially being best-in-class; whether veligrotug will serve an unmet need; Viridian’s expectations regarding the potential commercialization of veligrotug and VRDN-003, if approved; and that Viridian’s cash, cash equivalents and short-term investments will be sufficient to fund its operations into the second half of 2027.

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 preliminary data may not be representative of final data; 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; regulatory interactions 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 and projected cash runway; our future operating results and financial performance; Viridian's intellectual property position; the timing of preclinical and clinical trial activities and reporting results from same; that our product candidates may not be commercially successful, if approved and those risks set forth under the caption "Risk Factors" in our most recent quarterly report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on November 12, 2024 and other subsequent disclosure documents filed with the SEC. 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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