



Viridian Presents Positive Clinical Data from Ongoing VRDN-001 Phase 1/2 Trial in Active Thyroid Eye Disease (TED) Patients During Late-Breaking Presentations at the American Thyroid Association (ATA) 91st Annual Meeting

October 22, 2022

- *Clinical and in vitro data presented in three late-breaking presentations at ATA provide emerging evidence of VRDN-001 efficacy and differentiation -*
- *Full 10 mg/kg cohort data presented at ATA, 20 mg/kg and 3 mg/kg cohort data on track for 4Q22 -*
- *Pivotal program for VRDN-001 in TED patients set to begin this quarter -*

WALTHAM, Mass., Oct. 22, 2022 (GLOBE NEWSWIRE) -- Viridian Therapeutics, Inc. (NASDAQ: VRDN), a biotechnology company advancing new treatments for patients suffering from serious diseases underserved by current therapies, presented positive proof-of-concept data from the 10 mg/kg cohort in its ongoing Phase 1/2 clinical trial of VRDN-001, an anti-IGF-1R antibody, in patients with active thyroid eye disease (TED). These data, as well as new in vitro data further characterizing and differentiating the pharmacological profile of VRDN-001, were included as part of three late-breaking poster presentations at the American Thyroid Association (ATA) 91st Annual Meeting. The abstract describing new in vitro data on the distinct anti-IGF-1R profile of VRDN-001 was also selected as an oral highlighted late breaking presentation. The three posters are available on the Viridian website ([click here](#)).

"Patients suffering from TED would benefit from additional therapeutic options," said Raymond Douglas, M.D., Ph.D., director of the Orbital and Thyroid Eye Disease Program, Cedars-Sinai Medical Center and an investigator in the VRDN-001 trial. "The rapid and near complete resolution of key signs and symptoms of TED in the majority of patients at six weeks following two infusions of 10 mg/kg VRDN-001 in a cohort of patients with active TED suggests VRDN-001, with further study, may provide an important new option for TED patients."

ATA Poster #535: VRDN-001, a Full Antagonist Antibody to the Insulin-Like Growth Factor Receptor-1 (IGF-1R) for Thyroid Eye Disease (TED): Phase 1/2 Proof of Concept in Patients with TED

Poster #535 presents proof-of-concept data from the first cohort of patients with active TED treated in the ongoing Phase 1/2 clinical trial. In this cohort, a total of 8 patients were randomized to receive two infusions of 10 mg/kg dose of VRDN-001 or placebo intravenously; 6 patients received VRDN-001 and 2 patients received placebo. In patients receiving VRDN-001, proptosis response was achieved by 83% of patients, with a mean reduction of 2.4 mm from baseline. Clinical Activity Score (CAS) of 0 or 1 was achieved by 83% of patients, with a mean reduction of 4.3 points from baseline. Complete resolution of diplopia was achieved by 75% of patients who presented with diplopia at baseline. VRDN-001 demonstrated a favorable safety and tolerability profile with no reported SAEs, no hyperglycemia, and no infusion reactions.

Poster #535 was authored by Shoaib Ugradar, UCLA Stein Eye Institute, Barrett Katz, Viridian Therapeutics, Denis O'Shaughnessy, Viridian Therapeutics, Rochelle Summerfelt, Viridian Therapeutics, Angela She, Viridian Therapeutics, and Raymond Douglas, Cedars Sinai Medical Center.

ATA Poster #568: VRDN-001, A Potent and Selective Insulin-Like Growth Factor-1 Receptor (IGF-1R) Antagonist Antibody for Thyroid Eye Disease (TED): Phase 1 Safety and Pharmacodynamic Results in Healthy Volunteers

Poster #568 presents full safety and pharmacodynamic data from the completed healthy volunteer portion of the ongoing Phase 1/2 trial of VRDN-001. A total of 13 subjects were randomized to receive two infusions of either 3, 10, or 20 mg/kg dose of VRDN-001 or placebo. Twelve subjects completed the trial; one of the subjects in the 20 mg/kg group withdrew for personal reasons after the first infusion and was followed through Day 35.

Plasma levels of IGF-1, a biomarker for IGF-1R antagonism, increased five- to seven-fold above baseline indicating maximal target engagement at all doses. All doses studied were generally safe and well tolerated, with no cases of hearing impairment or treatment related hyperglycemia and no infusion reactions.

Poster #568 was authored by Angela She, Barrett Katz, Rochelle Summerfelt, Denis O'Shaughnessy, Brent Dickinson, Kelly Foster, and Vahe Bedian of Viridian Therapeutics

ATA Poster #132: VRDN-001, a Full Antagonist Antibody to the Insulin-Like Growth Factor-1 Receptor (IGF-1R) in Development for Thyroid Eye Disease (TED), Binds to a Distinct Epitope from Teprotumumab

Poster #132 presents preclinical data from in vitro studies of VRDN-001. The data show that VRDN-001 binds the same region of the IGF-1R as teprotumumab but engages a distinct epitope. This difference in binding translated to differences in functional effects: unlike the anti-IGF-1R antibody teprotumumab, which incompletely antagonized IGF-1R function, VRDN-001 fully antagonized ligand binding, receptor autophosphorylation, and downstream signaling.

These pharmacological differences may provide a mechanistic basis for the initial data reported from the ongoing VRDN-001 Phase 1/2 study, including the pharmacodynamic responses in the healthy volunteer cohort presented in Poster #568 and favorable clinical responses seen in TED patients as presented in Poster #535.

Poster #132 was authored by Yang Zhao, Jordan Tsai, Rachel Newell, and Vahe Bedian of Viridian Therapeutics.

Upcoming Clinical Milestones for the Ongoing VRDN-001 Phase 1/2 Proof-of-Concept Trial

The Company remains on track to present additional updates from the VRDN-001 Phase 1/2 study this quarter. These updates will include top-line data for the 20 mg/kg cohort of the ongoing Phase 1/2 trial of VRDN-001 in TED, followed later this quarter by top-line data from the currently enrolling

3 mg/kg cohort.

This ongoing trial is evaluating two infusions of VRDN-001, three weeks apart, with efficacy measured six weeks after the first dose. Each dose is evaluated in a cohort of eight patients, randomized so that six patients receive VRDN-001 and two patients receive placebo. [The first cohort evaluated a dose of 10 mg/kg, with initial clinical data reported on August 15, 2022.](#) The Company expects to initiate the pivotal program for VRDN-001 by the end of 2022.

This quarter the Company also will report final pharmacokinetic and pharmacodynamic data from Viridian's first-in-human trial of VRDN-002, a half-life extended IGF-1R antibody, which build upon the recently presented interim results. The Company expects data from a proof-of-concept trial of subcutaneously administered VRDN-002 in the second half of 2023.

About Viridian Therapeutics, Inc.

Viridian Therapeutics is a biotechnology company advancing new treatments for patients suffering from serious diseases underserved by current therapies. Viridian's most advanced program, VRDN-001, is a differentiated monoclonal antibody targeting insulin-like growth factor-1 receptor (IGF-1R), a clinically and commercially validated target for the treatment of thyroid eye disease (TED). VRDN-002 is a distinct anti-IGF-1R antibody and incorporates half-life extension technology. VRDN-003 is an extended half-life version of VRDN-001. Both VRDN-002 and VRDN-003 are designed for administration as convenient, low-volume, subcutaneous injections. TED is a debilitating autoimmune disease that causes inflammation and fibrosis within the orbit of the eye which can cause double vision, pain, and potential blindness. Viridian is based in Waltham, Massachusetts.

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," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," or "would" or other similar terms or expressions that concern the Company's expectations, plans and intentions. Forward-looking statements include, without limitation, statements regarding the Company's expectations, strategies, plans and intentions. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on the Company's current beliefs, expectations, and assumptions. 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: the potential efficacy and safety of VRDN-001 and VRDN-002 or the treatment of TED; the timing, progress and plans for the Company's ongoing and future research and clinical development programs; expectations regarding the timing for data, including the expected timing of additional data from the ongoing Phase 1/2 clinical trial of VRDN-001 and the first-in-human Phase 1 clinical trial of VRDN-002, including those risks set forth under the caption "Risk Factors" in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 11, 2022 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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