Vitae Pharmaceuticals Announces Positive Top-Line Results From Initial Phase 1 Study of First-in-Class RORγt Inhibitor VTP-43742 in Autoimmune Disorders

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- *Single ascending doses of VTP-43742 safe and generally well-tolerated, demonstrated once-daily pharmacokinetics*
- *Robust ex vivo biomarker response, suppressing pro-inflammatory IL-17A by more than 90 percent*

FORT WASHINGTON, Pa., Sept. 8, 2015 (GLOBE NEWSWIRE) -- Vitae Pharmaceuticals, Inc. (NASDAQ:VTAE), a clinical-stage biotechnology company, today announced positive top-line results from its Phase 1 single ascending dose clinical study of VTP-43742 in autoimmune disorders. VTP-43742 is Vitae's first-in-class, wholly owned RORγt inhibitor being developed for the treatment of a range of autoimmune disorders, potentially including psoriasis, psoriatic arthritis, rheumatoid arthritis, multiple sclerosis and irritable bowel disease (IBD), as well as numerous orphan diseases.

In this double-blind, randomized, placebo-controlled study that evaluated the safety, tolerability, pharmacokinetic (PK), and pharmacodynamic (PD) profile of single oral doses of VTP-43742 in 53 healthy human volunteers, VTP-43742 was safe and generally well tolerated at all dose levels across a 60-fold dose range. No serious adverse events were reported and there were no drug-related clinical laboratory or electrocardiogram (ECG) abnormalities.

VTP-43742 was also evaluated in an *ex vivo* assay for its ability to inhibit the production of pro-inflammatory cytokine IL-17A in blood obtained from study subjects. Subjects receiving VTP-43742 showed a dose-dependent suppression of RORγt dependent IL-17A production by more than 90 percent, with the effect largely sustained over the full 24-hour measurement period.

In animal studies, steady inhibition of RORγt was necessary to achieve full therapeutic efficacy, indicating the importance of a relatively long plasma half-life. The plasma half-life of VTP-43742 was observed to be approximately 30 hours in this clinical trial, supporting the potential for effective once-a-day dosing in humans.

"VTP-43742's robust and sustained lowering of IL-17A production observed in the *ex vivo* blood assay, paired with its favorable safety, tolerability and PK profile, demonstrate that this first-in-class drug candidate has the potential to safely and effectively treat a range of autoimmune conditions," said Dr. Richard Gregg, Chief Scientific Officer of Vitae. "We are extremely encouraged by the PK and PD data, and look forward to reporting additional clinical results, including top-line proof-of-concept data in psoriasis patients, by the end of the year."

Vitae is currently conducting a Phase 1 multiple ascending dose clinical trial of VTP-43742, which was initiated in August 2015. This trial includes both healthy human volunteers and patients with moderate to severe psoriasis. The Company plans to begin dosing psoriatic patients in the second half of 2015, with top-line clinical efficacy results expected by the end of 2015.

About Autoimmune Disorders

Autoimmune disorders, where a patient's own immune system attacks normal tissue, make up a large number of human disorders. Increased activity of a class of lymphocytes called Th17 cells, and the subsequent excess production of pro-inflammatory cytokines, including IL-17, by those cells are critical parts of the pathophysiology of many human autoimmune disorders. RORγt is a nuclear hormone receptor that is essential for the formation and function of Th17 cells. Vitae believes inhibition of excess RORγt activity in immune cells will be beneficial for the treatment of multiple autoimmune disorders, potentially including psoriasis, psoriatic arthritis, rheumatoid arthritis, multiple sclerosis and / or IBD, as well as numerous orphan diseases.

About VTP-43742

VTP-43742 is Vitae's first-in-class, wholly owned product candidate for the potential treatment of a variety of

autoimmune disorders and orphan diseases. In preclinical studies, VTP-43742 inhibited the activity of RORγt and has demonstrated potent inhibition of multiple Th17 cell-dependent pro-inflammatory cytokines, including IL-17. It is highly selective versus other ROR isotypes, and has a plasma half following single oral dosing that is consistent with a once a day dosing schedule. VTP-43742 has demonstrated superior efficacy in an animal model of multiple sclerosis in direct comparison to an IL-17A monoclonal antibody. Vitae initiated Phase 1 testing with VTP-43742 in June of 2015 with this Phase 1 single ascending dose clinical trial in healthy human volunteers. A Phase 1 multiple ascending dose clinical trial in healthy human volunteers was initiated in August 2015.

About Vitae Pharmaceuticals

Vitae Pharmaceuticals is a clinical-stage biotechnology company focused on discovering and developing first-in-class, small molecule drugs for difficult-to-drug disease targets that can potentially address significant unmet medical needs, including programs in autoimmune disorders, atopic dermatitis, Alzheimer's disease and diabetes. This robust and growing portfolio of novel product candidates is generated internally by Contour®, Vitae's proprietary structure-based drug discovery platform.

For additional information, please visit the company's website at www.vitaepharma.com.

Safe Harbor Statement

This release contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, including statements regarding the clinical development of VTP-43742. In some cases, you can identify forward-looking statements by terms such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "target," "design," "estimate," "predict," "potential," "plan," "impending" or the negative of these terms, and similar expressions intended to identify forward-looking statements. Such forward- looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. Vitae is at an early stage of development and may not ever have any products that generate significant revenue. Important factors that could cause actual results to differ materially from those reflected in Vitae's forward-looking statements include, among others, the timing and success of preclinical studies and clinical trials conducted by Vitae and its collaborative partners; the ability to obtain and maintain regulatory approval of Vitae's product candidates, and the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing Vitae's product candidates; the size and growth of the potential markets for Vitae's product candidates and the ability to serve those markets; Vitae's expectations regarding Vitae's expenses and revenue, the sufficiency of Vitae's cash resources and needs for additional financing; Vitae's ability to attract or retain key personnel; and other factors that are described in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of Vitae's Annual Report on Form 10-K for the year ended December 31, 2014 and Vitae's Quarterly Report on Form 10-Q for the quarter ended June 30, 2015 which have been filed with the Securities and Exchange Commission (SEC). In addition to the risks described above and in Vitae's other filings with the SEC, other unknown or unpredictable factors also could affect Vitae's results. No forward-looking statements can be guaranteed and actual results may differ materially from such statements. The information in this release is provided only as of the date of this release, and Vitae undertakes no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.

CONTACT: INVESTORS:
Vitae Pharmaceuticals, Inc.
Richard S. Morris, CPA
Chief Financial Officer
(215) 461-2000
rmorris@vitaerx.com

Westwicke Partners
John Woolford
(443) 213-0506
john.woolford@westwicke.com

MEDIA:
6 Degrees PR
Tony Plohoros
(908) 591-2839
tplohoros@6degreespr.com

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