October 24, 2011

Vertex Announces Presentation of New Data for Oral JAK3 Inhibitor VX-509 at Annual Meeting of the American College of Rheumatology

-Final data to be presented from Phase 2 study in rheumatoid arthritis-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that two abstracts related to its investigational oral JAK3 inhibitor, VX-509, will be presented at the 2011 Annual Meeting for the American College of Rheumatology (ACR) in Chicago, November 5-9, 2011. Presentations will include final data from a recently completed Phase 2 study of VX-509 in rheumatoid arthritis (RA) as well as preclinical data in models of inflammation that supported the Phase 2 study and provide a rationale for further evaluation of VX-509 in a number of immune-mediated inflammatory diseases. Vertex announced top-line results from the Phase 2 study in September, and final data will be presented at ACR.

"By selectively targeting JAK3, VX-509 represents a new approach to treating an underlying disease mechanism that triggers inflammation in a number of debilitating diseases, including RA," said Peter Mueller, Ph.D., Chief Scientific Officer and Executive Vice President of Global Research and Development at Vertex. "Based on the Phase 2 data to be presented at ACR, we plan to begin a larger and longer-duration study in RA early next year that will evaluate VX-509 in combination with methotrexate."

The accepted abstracts are now available online on the ACR conference website: http://acr.confex.com/acr/2011/webprogram/ACR.html.

Accepted VX-509 Abstracts:

Phase 2 Data:

Dose Ranging Study of VX-509, An Oral Selective JAK3 Inhibitor, As Monotherapy in Patients with Active Rheumatoid Arthritis (RA)

- Presentation Number L9: Tuesday, November 8, 2011: 9:00 a.m. - 6:00 p.m., Hall F2 - Poster Hall (McCormick Place West)
- Link: http://acr.confex.com/acr/2011/webprogram/Paper24549.html

Preclinical Data:

VX-509, An Orally Available Janus Kinase 3 (JAK3) Specific Inhibitor, Showed Robust Activity in Pre-Clinical Models of Aberrant Immune/Inflammatory Function

- Presentation Number 1136: Monday, November 7, 2011: 9:00 a.m. - 6:00 p.m., Hall F2 - Poster Hall (McCormick Place West)
- Link: http://acr.confex.com/acr/2011/webprogram/Paper20198.html

About VX-509

VX-509 is an investigational oral medicine being developed by Vertex that is designed to selectively inhibit Janus kinase 3, or JAK3, which is an essential part of the underlying disease mechanisms that cause inflammation in diseases such as RA. JAK3 specific inhibition represents a new approach to the treatment of a range of autoimmune diseases where it is an essential component of the signaling cascade that contributes to the abnormal immune response, which results in chronic inflammation and, in the case of RA, irreversible damage to cartilage and bones. JAK3 is one of four JAK family kinases (JAK1, JAK2, JAK3 and Tyk2).

Based on *in vitro* data, VX-509 has demonstrated a high degree of potency for JAK3 and a high level of selectivity for inhibition

of JAK3 compared to JAK1 and JAK2 dependent assays. VX-509 has been shown to be greater than 1000-fold more selective for JAK3 compared to non-JAK kinases and approximately 25- to 150-fold more selective for JAK3 compared to other JAK isotypes in cell-based assays. This high level of selectivity was confirmed in clinical studies where dose-related inhibition of a JAK3 dependent biomarker was observed while little to no effect was shown against a JAK2/JAK1 dependent biomarker.

Vertex completed a Phase 2a study of VX-509 in mid-2011 in which VX-509 was dosed twice daily as monotherapy for 12 weeks in people with moderate to severe active RA. In early 2012, Vertex plans to initiate a larger and longer-duration Phase 2b study that will evaluate VX-509 in combination with methotrexate, a commonly prescribed disease-modifying antirheumatic drug (DMARD) for RA that is frequently used in combination with other RA medicines. This study is expected to be six months in duration and will evaluate both once and twice-daily dosing of VX-509. VX-509 was discovered by Vertex scientists.

About Rheumatoid Arthritis

RA is a chronic inflammatory disease that affects 1 percent to 2 percent of the world's population, including 1.5 million adults in the United States.[1,9] The disease causes destruction of joint cartilage and erosion of adjacent bone, resulting in deformity, loss of function, substantial disability and, for many, the need for joint replacement. Many people with RA suffer progressive disability over time,[2,3] pain,[4] work loss,[5] substantial health care costs,[6] and premature death.[7] Approximately 80 percent of people with RA become disabled within 20 years of diagnosis.[8]

The treatment of RA focuses on reducing symptoms and inhibiting progression of the disease. Nonsteroidal anti-inflammatory drugs (NSAIDs) and corticosteroids may reduce the symptoms of pain, swelling and stiffness, but they do not alter the natural course of the disease. Disease-modifying antirheumatic drugs (DMARDs) have shown effects in slowing the progression of joint damage and time to disability and thus altering the natural history of RA. While recently approved medicines are effective in a portion of patients, a significant number of people with RA do not respond adequately or become refractory to these medicines, creating a significant need for new approaches to RA treatment.

About Vertex

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, rheumatoid arthritis, epilepsy and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, MA, we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 1,900 employees around the world, and *Science* magazine named Vertex number one on its 2011 list of Top Employers in the life sciences.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including statements regarding (i) Vertex's plan to present data regarding VX-509 at ACR; (ii) Vertex's plan to begin a larger and longer-duration Phase 2b study in RA early next year that will evaluate VX-509 in combination with methotrexate; (iii) the rationale for further evaluation of VX-509 in a number of immune-mediated diseases and (iv) the expected duration and dosing regimens for the study in RA to be initiated early next year. While Vertex believes the forward-looking statements contained in this press release are accurate, these statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the forward-looking statements. These risks and uncertainties include, among other things, the risks that efforts to develop VX-509 may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the Vertex's website at **www.vrtx.com**. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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[9] Arthritis Rheum. 2010 Jun;62(6):1576-82. [Data source: Patient Cohort, Minnesota]

(VRTX-GEN)

Vertex:
Media:
Zach Barber**,** 617-444-6992
mediainfo@vrtx.com
or
Investors:
Michael Partridge, 617-444-6108
or
Lora Pike, 617-444-6755
or
Matthew Osborne, 617-444-6057

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