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Vertex Announces Completion of Phase 2 Study of VX-765 in People with Epilepsy who did not Respond to Previous Treatment

-Results support continued development of VX-765 in people with treatment-resistant epilepsy-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced results from a recently completed Phase 2 study of VX-765 in 60 people with treatment-resistant epilepsy. The primary endpoint of the study was safety and tolerability, and results from the study showed a similar safety profile for VX-765 as compared to placebo. Secondary endpoints and additional analyses evaluated the clinical activity of VX-765, and results support the initiation of a larger and longer-duration Phase 2b study of VX-765 in people with treatment-resistant epilepsy. Vertex expects to begin this trial as early as the fourth quarter of 2011.

"The use of anti-inflammatory medicines is a completely new way to approach the treatment of epilepsy, and this study was an important first step toward understanding whether VX-765 could help people with this disease," said Peter Mueller, Ph.D., Executive Vice President, Global Research and Development, and Chief Scientific Officer for Vertex. "People who do not respond to standard medicines for epilepsy are often severely debilitated by frequent seizures that limit daily activities and independence. The results observed in this study provide support for the continued evaluation of VX-765 as part of a larger and longer-duration study in epilepsy planned for later this year."

About the Study

The double-blind, randomized, placebo-controlled Phase 2a study of VX-765 enrolled 60 people with treatment-resistant partial onset epilepsy, which is a type of epilepsy where the seizures start and occur in a specific part of the brain. The study enrolled and dosed people who did not benefit from the use of at least two currently available medicines for partial epilepsy. Patients received six weeks of treatment with VX-765 or placebo following a six-week baseline period to monitor seizure frequency. All patients in the study had at least six partial seizures during the baseline period. Patients were followed for six weeks after the treatment phase to collect additional safety and seizure information. Patients continued to receive standard medicines for epilepsy throughout the study, in addition to VX-765 or placebo. In the study, 48 people received 900 mg of VX-765 three-times-daily and 12 people received placebo three-times-daily.

Preliminary Analysis

The primary endpoint of the study was the safety and tolerability of VX-765. In the study, the safety profile for VX-765 was similar to that for placebo. The most common adverse events observed across both treatment arms were headache, dizziness, fatigue and gastrointestinal disorders, and the majority of these adverse events were mild to moderate. The only adverse event that was 10 percent or greater in frequency among those treated with VX-765 compared to placebo was dizziness. One person discontinued treatment due to adverse events during the study and was in the VX-765 treatment group.

Key secondary endpoints focused on the clinical activity of VX-765 during the study based on the following:

(1) Percent reduction in seizure rate
(2) Percent of patients with a 50 percent or greater reduction in seizure frequency, known as the responder-rate
(3) Percent of patients who were seizure-free in the last two weeks of treatment

In addition to the endpoints specified in the study protocol, a number of additional analyses were conducted to evaluate the clinical activity of VX-765 during the last two weeks of the treatment phase and first two weeks of the follow-up period. These evaluations included:

(1) Percent of patients who were seizure-free during the first two weeks of the follow-up period only
(2) Percent reduction in seizure rate during the last two weeks of the treatment phase and first two weeks of the follow-up period only
(3) Percent of patients with a 50 percent or greater reduction in seizure frequency, known as the responder-rate, during the last two weeks of the treatment phase and first two weeks of the follow-up period only

Results for these endpoints and additional evaluations are as follows:

Key Secondary Endpoints:	VX-765	Placebo	Treatment Difference
-- Percent reduction in seizure rate -- Percent of patients with a 50 percent or greater reduction in seizure frequency, known as the responder-rate -- Percent of patients who were seizure-free in the last two weeks of treatment	13% - 19%	0% - 9%	**9% - 13%**
Additional Analyses:			
-- Percent of patients who were seizure-free during the first two weeks of the follow-up period only -- Percent reduction in seizure rate during the last two weeks of the treatment phase and first two weeks of the follow-up period only -- Percent of patients with a 50 percent or greater reduction in seizure frequency, known as the responder-rate, during the last two weeks of the treatment phase and first two weeks of the follow-up period only	19% - 31%	0% - 9%	**19% - 23%**

While none of the observed differences outlined above were statistically significant, the improvements observed in the last two weeks of the treatment phase and first two weeks of the follow-up period suggest that the clinical activity observed for VX-765 was greater at the end of the six-week treatment phase and continued during the start of the follow-up phase. These results indicate that a longer-duration study may be needed to characterize any additional or more robust clinical activity of VX-765. Vertex plans to further evaluate VX-765 as part of a larger Phase 2b study that will evaluate people with treatment-resistant epilepsy for a treatment period longer than six weeks. The company intends to initiate this study as early as the fourth quarter of 2011.

About VX-765

VX-765 is an oral medicine in development that is designed to inhibit Caspase-1, an enzyme involved in the production of IL-1 beta and linked to a wide range of immune and inflammatory responses. VX-765 has been shown to inhibit acute partial seizures in preclinical models and has shown activity in preclinical models of chronic partial epilepsy that do not respond to currently available medicines for epilepsy. Vertex holds worldwide rights to VX-765.

About Epilepsy

Epilepsy is a chronic neurological disorder characterized by recurrent seizures resulting from overactive neurons in the brain. Recent studies suggest that inflammation and overproduction of IL-1 beta may be associated with epileptic seizures.[1] The Centers for Disease Control and Prevention (CDC) estimate that about 2 million people in the United States have epilepsy and nearly 140,000 Americans develop the condition each year.[2] While there are a number of approved epilepsy medicines, more than 30 percent of people are not well-controlled on current treatments and continue to have seizures.[3] Epilepsy can have a significant impact on people's quality of life and independence. The CDC estimates that the total indirect and direct cost of epilepsy in the United States is $15.5 billion.[2]

About Vertex

Vertex creates new possibilities in medicine. Our team aims to discover, develop and commercialize 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, 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.

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) the results from the completed Phase 2 study supporting continued development of VX-765 in people with treatment-resistant epilepsy; (ii) the expectation that Vertex will begin a larger and longer-duration Phase 2b study of VX-765 as early as the fourth quarter of 2011; (iii) the suggestion that the clinical activity observed for VX-765 was

greater at the end of the six-week treatment phase and continued during the start of the follow-up phase; and (iv) the possibility that a longer-duration study may be needed to characterize any additional or more robust clinical activity. 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-765 may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned, that larger and longer-duration clinical trials of VX-765 may not show any or any additional or more robust clinical activity, that an adverse event profile for VX-765 could be revealed in further nonclinical or clinical studies that could put further development of VX-765 in jeopardy or adversely impact its therapeutic value, 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 Company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

[1] *Current Opinion in Investigational Drugs.* 2010; 11(1): 34-50

[2] Hirtz D, Thurman DJ, Gwinn-Hardy K, Mohamed M, Chaudhuri AR, Zalutsky R: How common are the "common" neurologic disorders? *Neurology* 2007, 68:326-337

[3] *Current Opinion in Investigational Drugs.* 2010; 11(1): 34-50

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