January 9, 2014

Vertex Announces Sustained Viral Response Rate (SVR4) Data from All-Oral Study of VX-135 in Combination with Daclatasvir in Hepatitis C

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the first data from the initial cohorts of an open-label Phase 2a study of VX-135, Vertex's nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor, in combination with daclatasvir, Bristol-Myers Squibb's NS5A replication complex inhibitor. In an intent-to-treat analysis, the sustained viral response rate four weeks after the completion of treatment (SVR4) was 83 percent (10 of 12) in treatment-naïve genotype 1 patients who received 200 mg of VX-135 in combination with daclatasvir. In this arm, one patient discontinued treatment after the first dose due to a serious adverse event of vomiting/nausea. The 11 other patients in this arm completed 12 weeks of treatment, and 91 percent (10 of 11) achieved SVR4. In the study, the majority of adverse events were mild.

"We are encouraged by these initial Phase 2a data for VX-135 in combination with another direct acting antiviral medicine," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer at Vertex. "We believe that VX-135 has the potential to play an important future role in the treatment of hepatitis C, and we are currently evaluating these data with BMS to determine the next steps for this combination in people with hepatitis C, including people with genotypes 1 and 3."

About the Phase 2a Study

The data announced today are from the first two cohorts of an open-label Phase 2a study of VX-135 in combination with daclatasvir. The initial two cohorts of the study evaluated 100 mg and 200 mg once-daily doses of VX-135 in combination with daclatasvir once daily (60 mg) for 12 weeks of total treatment. Twenty-three people with chronic genotype 1 hepatitis C who were new to treatment (treatment-naïve) and did not have liver cirrhosis were enrolled in these cohorts. More than 75 percent of all patients enrolled had genotype 1a HCV. The majority of adverse events observed in the study were mild. The most common adverse events observed in greater than 10 percent of patients across the study were fatigue, headache and nausea. Safety and efficacy data for the two arms of the study are provided below:

- **200 mg of VX-135 in Combination with Daclatasvir (60 mg):** In an intent-to-treat analysis, 58 percent (7 of 12) of patients had undetectable HCV RNA after 4 weeks of treatment and 83 percent (10 of 12) of patients had undetectable HCV RNA four weeks after the completion of treatment (SVR4). One patient in this arm experienced a serious adverse event of vomiting/nausea, discontinued treatment after the first dose and did not achieve SVR4. The 11 other patients in this arm completed 12 weeks of treatment, and 91 percent (10 of 11) achieved SVR4. One patient relapsed during the follow-up period and did not achieve SVR4.

- **100 mg of VX-135 in Combination with Daclatasvir (60 mg):** In an intent-to-treat analysis, 73 percent (8 of 11) of patients achieved undetectable HCV RNA after 4 weeks of treatment and 73 percent (8 of 11) of patients had undetectable HCV RNA four weeks after the completion of treatment (SVR4). Two patients in this arm experienced viral breakthrough while receiving the combination regimen, and one patient relapsed during the follow-up period.

Vertex expects to submit these data for presentation at a medical meeting in 2014.

About VX-135

VX-135 is a uridine nucleotide analogue pro-drug designed to inhibit the replication of the hepatitis C virus by acting on the NS5B polymerase. Vertex gained worldwide rights to ALS-2200, known as VX-135 in Phase 2 studies, through an exclusive licensing agreement signed with Alios BioPharma, Inc. in June 2011.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines 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 cystic fibrosis, hepatitis C, rheumatoid arthritis and other life-threatening diseases. In addition to our clinical development programs, Vertex has more than a dozen ongoing preclinical programs aimed at other serious and life-threatening diseases.
Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For four years in a row, Science magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

**Vertex 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, without limitation, Dr. Kauffman's statements in the second paragraph of the press release. While Vertex believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that there is a partial clinical hold on VX-135 in the United States, that the outcomes of Vertex's clinical studies of VX-135 may not be favorable or support further development of VX-135 due to safety, efficacy, or other reasons, and the 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.

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