



Xenon Provides Key Regulatory Updates on XEN007 and XEN1101

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FDA Grants Rare Pediatric Disease Designation to XEN007 for Alternating Hemiplegia of Childhood (AHC); Xenon Eligible to Apply for Priority Review Voucher

IND for XEN1101 Accepted; Site Selection and Patient Enrollment Underway in Canada and the United States for XEN1101 Phase 2b Clinical Trial

BURNABY, British Columbia, Jan. 28, 2019 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq:XENE), a clinical stage biopharmaceutical company, today provided regulatory updates on XEN007 and XEN1101, two therapeutic candidates from its CNS pipeline.

The U.S. Food and Drug Administration (FDA) has granted rare pediatric disease (RPD) designation for the treatment of Alternating Hemiplegia of Childhood (AHC) with XEN007 (active ingredient flunarizine). The FDA grants the RPD designation for serious or life-threatening diseases that primarily affect children 18 years old or younger and affect fewer than 200,000 people nationwide. The designation allows recipient companies, upon approval of the subject treatment, to be eligible for a priority review voucher, which may be used to obtain what is referred to as priority review for a future submission of a New Drug Application (NDA).

Dr. Simon Pimstone, Xenon's Chief Executive Officer, said, "Receipt of the RPD designation from the FDA is an important milestone for our XEN007 program and underscores the need to find treatments for AHC, a rare, severe and debilitating neurological movement disorder that presents within the first 18 months of life and causes lifetime morbidity and increased mortality. There are no FDA-approved drugs for AHC, and Xenon has the support of key physicians and patient advocacy groups to pursue the development of XEN007 as a treatment of AHC. We look forward to continued collaboration with the FDA as we further develop our clinical plans for XEN007, including the opportunity to define a regulatory path for XEN007 in AHC and other neurological conditions. We plan to engage with FDA and receive feedback on our development plans for XEN007 over the next two quarters."

XEN007 (active ingredient flunarizine) is a CNS-acting calcium channel inhibitor, which is available in certain countries outside of the United States, and has been reported to have clinical benefit in migraine and other neurological disorders, including hemiplegic migraine (HM), AHC, and other childhood neurological disorders. Although the efficacy and safety of flunarizine has not been well studied in a randomized, placebo-controlled trial as a treatment for AHC, it has been evaluated in numerous AHC patients. The published literature suggests that flunarizine may be an effective treatment for AHC and appears to be safe and well tolerated by AHC patients. In addition to the RPD for AHC, Xenon previously received orphan drug designations (ODD) from the FDA for XEN007 for the treatment of both AHC and HM. In addition, Xenon entered into key exclusive licensing agreements in order to access regulatory files and to manufacture the final product, both of which may enable advanced clinical development of XEN007. Xenon is currently evaluating various development strategies for XEN007, including the support of at least one Phase 2 (or later stage) clinical trial in an orphan neurological indication, with initiation anticipated in 2019.

Dr. Pimstone added, "We are also pleased to announce that an investigational new drug (IND) application has been accepted by the FDA for XEN1101, a Kv7 potassium channel modulator that we are developing for the treatment of epilepsy and potentially other neurological disorders. Site selection and patient enrollment are now underway for the XEN1101 Phase 2b clinical trial in Canada and the United States. Additionally, we have filed applications in multiple European countries, which we anticipate will be accepted in the near term in order to expand the trial to European clinical trial sites."

The XEN1101 Phase 2b clinical trial is designed as a randomized, double-blind, placebo-controlled, multicenter study to evaluate the clinical efficacy, safety and tolerability of XEN1101 administered as adjunctive treatment in adult patients with focal epilepsy. Approximately 300 patients will be randomized in a blinded manner to one of three active treatment groups or placebo in a 2:1:1:2 fashion (XEN1101 25 mg : 20 mg : 10 mg : Placebo). The primary endpoint is the median percent change in monthly focal seizure frequency from baseline compared to treatment period of active versus placebo. Depending upon the rate of enrollment, top-line results from the XEN1101 Phase 2b clinical trial are anticipated in the second half of 2020.

About Alternating Hemiplegia of Childhood (AHC)

AHC is a devastating and life-threatening neurological disease associated with significant morbidity that has substantial impact on day-to-day functioning. AHC presentation includes repeated attacks of hemiplegia involving either side of the body or episodes of quadriplegia, paroxysmal disturbances including tonic or dystonic spells, and developmental deterioration and neurologic abnormalities, which all initially present before 18 months of age. Oculomotor features have been documented to occur within one or two days after birth, with onset of hemiplegia and dystonia occurring as early as one month after birth. AHC children suffer from debilitating painful paroxysmal events that can last for days or weeks and fixed non-paroxysmal symptoms. A recent study conducted nationwide in Denmark identified 10 patients ≤16 years old with AHC, thus estimating the Danish prevalence to be

approximately one per 100,000 children. According to the reported U.S. birth incidence, it is estimated that there are potentially up to 3,200 patients with AHC in the U.S. Despite the severity of AHC, there are no drugs approved for its treatment.

About Xenon Pharmaceuticals Inc.

We are a clinical stage biopharmaceutical company committed to developing innovative therapeutics to improve the lives of patients with neurological disorders, including rare central nervous system (CNS) conditions. We are advancing a novel product pipeline of neurology therapies to address areas of high unmet medical need, with a focus on epilepsy. For more information, please visit www.xenon-pharma.com.

Safe Harbor Statement

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995 and Canadian securities laws. These forward-looking statements are not based on historical fact, and include statements regarding the timing of and results from clinical trials and pre-clinical development activities, including those related to XEN1101, XEN007 and our other product candidates, the potential efficacy, safety profile, future development plans, addressable market, regulatory success and commercial potential of XEN1101, XEN007 and our other product candidates, the anticipated timing of IND, or IND equivalent, submissions and the initiation of future clinical trials for XEN1101, XEN007 and our other product candidates, the efficacy of our clinical trial designs, our ability to successfully develop and achieve milestones in the XEN1101, XEN007 and other development programs, the timing of results of our interactions with regulators, the potential to advance certain of our product candidates directly into Phase 2 or later stage clinical trials, anticipated enrollment in our clinical trials, the progress and potential of our other ongoing development programs, and the timing of potential publication or presentation of future clinical data. These forward-looking statements are based on current assumptions that involve risks, uncertainties and other factors that may cause the actual results, events or developments to be materially different from those expressed or implied by such forward-looking statements. These risks and uncertainties, many of which are beyond our control, include, but are not limited to: clinical trials may not demonstrate safety and efficacy of any of our or our collaborators' product candidates; our assumptions regarding our planned expenditures and sufficiency of our cash to fund operations may be incorrect; our ongoing discovery and pre-clinical efforts may not yield additional product candidates; any of our or our collaborators' product candidates may fail in development, may not receive required regulatory approvals, or may be delayed to a point where they are not commercially viable; we may not achieve additional milestones in our proprietary or partnered programs; regulatory agencies may not permit certain of our product candidates to advance directly into a Phase 2 or later clinical trials or may delay the initiation thereof; the impact of competition; the impact of expanded product development and clinical activities on operating expenses; adverse conditions in the general domestic and global economic markets; as well as the other risks identified in our filings with the Securities and Exchange Commission and the securities commissions in British Columbia, Alberta and Ontario. These forward-looking statements speak only as of the date hereof and we assume no obligation to update these forward-looking statements, and readers are cautioned not to place undue reliance on such forward-looking statements.

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