# Xenon Pharmaceuticals Receives FDA Feedback and is on Track to Initiate XEN496 Phase 3 Clinical Trial for the Treatment of KCNQ2-DEE Before Year-End

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**Positive Opinion Received Supporting Orphan Medicinal Product Designation in Europe for XEN496 for the Treatment of KCNQ2-DEE**

BURNABY, British Columbia, Oct. 08, 2020 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq:XENE), a neurology-focused biopharmaceutical company, today provided regulatory updates on its proprietary pediatric neurology program, XEN496, a Kv7 potassium channel modulator that is a proprietary pediatric formulation of the active ingredient ezogabine being developed for the treatment of KCNQ2 developmental and epileptic encephalopathy (KCNQ2-DEE). With the U.S. Food and Drug Administration (FDA) having completed its review of the clinical trial protocol, Xenon is on track to initiate the XEN496 Phase 3 clinical trial in pediatric patients with KCNQ2-DEE before year-end.

In addition, Xenon has received a positive opinion from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), which recommends the granting of an orphan medicinal product designation for XEN496 for the treatment of KCNQ2-DEE. This European designation is in addition to the FDA granting Fast Track designation for XEN496 for the treatment of seizures associated with KCNQ2-DEE as well as Orphan Drug Designation (ODD) for the treatment of KCNQ2-DEE.

Dr. Simon Pimstone, Xenon's Chief Executive Officer, said, "This marks an extremely important milestone for Xenon, with the first of our proprietary product candidates now poised to enter a pivotal Phase 3 clinical trial. With feedback from the FDA, pharmacokinetic data supportive of our proprietary pediatric formulation, and considerable progress made in site selection and other preparations, we are excited to move forward with our 'EPIK' Phase 3 clinical trial studying XEN496 in pediatric patients with KCNQ2-DEE. In addition, receiving an orphan medicinal product designation for XEN496 in Europe underscores that KCNQ2-DEE is a severe, early onset epilepsy disorder and further validates Xenon's novel, 'precision medicine' approach to develop treatments for pediatric epilepsies. We continue to work closely with the medical community, genetic testing companies, and patient advocacy groups to identify potential patients for our EPIK study, which we expect to initiate before year-end."

The XEN496 Phase 3 "EPIK" clinical trial is titled "A Phase 3 Study of Adjunctive XEN496 in Pediatric Subjects with KCNQ2 Developmental and Epileptic Encephalopathy." This study is designed as a randomized, double-blind, placebo-controlled, parallel group, multicenter clinical trial to evaluate the efficacy, safety and tolerability of XEN496 administered as adjunctive treatment in approximately 40 pediatric patients aged one month to less than 6 years with KCNQ2-DEE. After screening, patients will enter a baseline period to assess the frequency of seizures. Eligible subjects will be randomized on a 1:1 basis to receive either XEN496 or placebo for approximately 15 weeks (titration and a 12-week maintenance period). At the end of treatment, there will be a period of tapering off of study drug, followed by a 28-day safety monitoring period. Patients may be considered for an open-label extension if they meet all requirements. The primary endpoint is the percent change from baseline in monthly countable motor seizure frequency during the blinded treatment period, as recorded by caregivers in a daily seizure diary. Key secondary endpoints include the proportion of patients experiencing greater than or equal to 50 percent reduction in monthly seizure frequency from baseline, caregiver global impression of change (CaGI-C) scores, and caregiver global impression of severity (CaGI-S) scores.

**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. 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, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, 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 other development activities; the potential efficacy, safety profile, future development plans, addressable market, regulatory success and commercial potential of XEN496; the anticipated timing of the initiation of future clinical trials for XEN496; the efficacy of our clinical trial designs; our ability to successfully develop and achieve milestones in the XEN496 development programs; the timing and results of our interactions with regulators; and anticipated enrollment in our clinical trials and the timing thereof. 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: the impact of the COVID-19 pandemic on our business, research and clinical development plans and timelines and results of operations may be more severe and more prolonged than currently anticipated;

clinical trials may not demonstrate safety and efficacy of any of our or our collaborators' 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 impose additional requirements or delay the initiation of clinical trials; regulatory agencies may be delayed in reviewing, commenting on or approving any of our or our collaborators' clinical development plans as a result of the COVID-19 pandemic, which could further delay development timelines; the impact of competition; the impact of new or changing laws or regulations; 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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