Xenon Pharmaceuticals Provides Update on Additional Positive Data from the XEN1101 Program

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New XEN1101 Phase 2b X-TOLE sub-group analysis demonstrates statistically significant efficacy results at all doses at Week 1, differentiating XEN1101 with rapid onset to seizure reduction

New XEN1101 OLE data demonstrates continued seizure reduction exceeding 70% at 3 months in OLE and exceeding 80% at 12 months in OLE

BURNABY, British Columbia, June 22, 2022 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq:XENE), a neurology-focused biopharmaceutical company, today announced new, compelling efficacy data supporting the late-stage, Phase 3 development of XEN1101.

Mr. Ian Mortimer, Xenon's President and Chief Executive Officer, stated, "We have generated additional efficacy data from sub-group analyses of our Phase 2b X-TOLE trial, which further support our Phase 3 development plans for XEN1101. A 'time course to efficacy' analysis shows that all doses of XEN1101 rapidly reduced the frequency of focal onset seizures within one week compared to placebo, suggesting that XEN1101 may offer a compelling and differentiated option for patients seeking to quickly reduce seizure frequency."

Dr. Christopher Kenney, Xenon's Chief Medical Officer, commented, "Based on these Phase 2b efficacy data, we are including the secondary endpoint of 'Week 1 median percent change in seizure frequency' within the statistical hierarchy of the Phase 3 focal onset seizure trials to build upon the differentiated profile of XEN1101. Additionally, within our analysis of the open label extension (OLE) population, we are seeing seizure frequency continuing to improve after the double-blind period with patients experiencing increased periods of seizure freedom. At the request of study investigators and based on the potential to continue to provide significant benefit to patients, we are extending the X-TOLE OLE from three to five years."

Summary of New XEN1101 Data from Phase 2b X-TOLE and Ongoing OLE

- XEN1101 rapidly reduced focal onset seizure (FOS) frequency within one week for all doses compared with placebo. At Week 1, the median percent reduction in monthly focal onset seizure frequency was 55.4% in the 25 mg group ($p<0.001$), 41.5% in the 20 mg group ($p=0.039$), and 39.1% in the 10 mg group ($p=0.002$) compared to 20.2% in the placebo group. Based on the strength of data from this time course to efficacy analysis, a key secondary endpoint in the Phase 3 trials will include the median percent change of weekly FOS at Week 1.
- Approximately 96% of patients who completed the randomized phase of the XEN1101 Phase 2b X-TOLE study rolled over into the OLE, with 231, 193 and 54 patients having now been treated in the trial for at least 6 months, 12 months, and 2 years, respectively.
- Seizure frequency continued to improve for the OLE population during the first month after the 8-week double-blind period (DBP), suggesting that the efficacy signal has the potential to persist, and may potentially improve, in the planned 12-week DBP of the XEN1101 Phase 3 trials.
- Subjects remaining in the X-TOLE OLE for at least 3 months and 12 months experienced a greater than 70% and 80% reduction, respectively, in median monthly seizure frequency when compared to the DBP baseline.
- 54 (19.6%) and 26 (9.5%) of subjects in the OLE experienced a ≥6 and a ≥12 consecutive months of seizure freedom, respectively. This analysis uses the denominator of all patients transitioning to the OLE (N=275) even though not all patients have been in the study long enough to be treated for at least 12 months.
- XEN1101 continues to be generally well-tolerated in the OLE with adverse events (AEs) consistent with other anti-seizure medicines and the X-TOLE double-blind period. There have been no treatment emergent AEs of pigmentary abnormalities reported during the DBP or OLE. As was the case in the DBP, two AEs of urinary retention occurred in the OLE possibly related to study drug; both patients continued in the study without requiring intervention. Weight changes in the OLE were 1.4 ± 4.5 kg at the 6-month visit and 0.9 ± 6.2 kg at the 12-month visit.

About Xenon Pharmaceuticals Inc.
Xenon Pharmaceuticals (NASDAQ:XENE) is 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 potential results from clinical trials, including those related to XEN1101; the potential efficacy, safety profile, future development plans, addressable market, regulatory success and commercial potential of XEN1101; the efficacy of our clinical trial designs; our ability to successfully develop and achieve milestones in the XEN1101 programs; the timing and results of our interactions with regulators; our ability to successfully develop and obtain regulatory approval of XEN1101; 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 ongoing COVID-19 pandemic on our research and clinical development plans and timelines and results of operations, including impact on our clinical trial sites, collaborators, and contractors who act for or on our behalf, 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; promising results from pre-clinical development activities or early clinical trial results may not be replicated in later clinical trials; 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, including XEN 1101 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 expanded product development and clinical activities on operating expenses; impact of new or changing laws and regulations; the impact of the COVID-19 pandemic on our business, adverse conditions in the general domestic and global economic markets; adverse conditions from geopolitical events; 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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