Xenon Outlines Key Corporate Milestone Opportunities for 2025

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– Topline data from the first Phase 3 FOS study anticipated in H2 2025 representing major milestone in support of NDA filing and potential launch of azetukalner

– First of three planned Phase 3 MDD studies now initiated

– Expanding ion channel portfolio includes Kv7 and Nav1.7 candidates advancing towards IND filings in 2025

VANCOUVER, British Columbia and BOSTON, Jan. 13, 2025 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq: XENE), a neuroscience-focused biopharmaceutical company dedicated to discovering, developing, and delivering life-changing therapeutics for patients in need, today outlined progress within its pipeline programs and key milestones for 2025.

“Reflecting on our accomplishments in 2024, we are proud of the advancements in our late-stage clinical programs, culminating in a strong presence at AES where we presented new long-term azetukalner data from our ongoing X-TOLE open-label extension study showing sustained monthly reduction in seizure frequency and impressive seizure freedom rates in patients with epilepsy,” said Ian Mortimer, President and Chief Executive Officer of Xenon. “We also made significant progress within our early-stage portfolio, progressing multiple drug candidates targeting Kv7 and Nav1.7 into IND-enabling studies.”

“Looking ahead to 2025, we anticipate a number of important milestones, particularly the first Phase 3 topline readout of azetukalner in focal onset seizures expected in the second half of 2025, representing a major inflection point for Xenon as we evolve from a clinical to commercial stage organization. Beyond epilepsy, we continue to advance azetukalner in MDD with the recent launch of our first Phase 3 study. Lastly, we expect multiple IND filings in 2025 in our early-stage programs, with the goal of initiating first-in-human trials across multiple targets,” stated Mr. Mortimer.

Program Updates and Anticipated Milestones

- Phase 3 azetukalner clinical studies in focal onset seizures (FOS) continue to advance, with the first topline data readout anticipated in the second half of 2025.
- Building upon more than 600 patient-years of data to date from the ongoing X-TOLE open-label extension study, Xenon continues to generate long-term scientific evidence supporting azetukalner’s compelling efficacy and safety profile.
- The Phase 3 X-ACKT clinical study continues to enroll patients and is intended to support potential regulatory submissions in an additional epilepsy indication of primary generalized tonic-clonic seizures (PGTCS).
- The first of three planned Phase 3 clinical trials evaluating azetukalner in patients with MDD, X-NOVA2, has been initiated to support indication expansion of azetukalner in neuropsychiatry. In addition, topline results from the Mount Sinai investigator-led study of azetukalner in MDD are anticipated in the first half of 2025.
- Xenon continues to expand its portfolio with pre-clinical candidates targeting Kv7, Nav1.7, and Nav1.1 across various indications and anticipates filing multiple INDs, or equivalent, in 2025.
- Within Xenon’s partnered program, Neurocrine Biosciences is now focusing its efforts on progressing a Nav1.2/1.6 inhibitor towards human clinical trials in 2025 as a potential treatment for epilepsy.

About Azetukalner Phase 3 Epilepsy Program

Xenon’s Phase 3 epilepsy program includes three Phase 3 clinical trials in focal onset seizures (FOS) and primary generalized tonic-clonic seizures (PGTCS). Designed closely after the Phase 2b X-TOLE clinical trial, the Phase 3 X-TOLE clinical trials are multicenter, randomized, double-blind, placebo-controlled studies evaluating the clinical efficacy, safety, and tolerability of 15 mg or 25 mg of azetukalner administered orally with food as adjunctive treatment in approximately 360 patients with FOS per study. The primary efficacy endpoint is the median percent change (MPC) in monthly seizure frequency from baseline through the 12-week double-blind period (DBP) of azetukalner compared to placebo. X-ACKT is a multicenter, randomized, double-blind, placebo-controlled study evaluating the clinical efficacy, safety, and tolerability of 25 mg of azetukalner administered with food as adjunctive treatment in approximately 160 patients with PGTCS. The primary efficacy endpoint is the MPC in monthly PGTCS frequency from baseline through the 12-week DBP of azetukalner compared to placebo. Upon completion of the DBP in the Phase 3 epilepsy studies, eligible patients may enter an OLE study for up to three years.

About Azetukalner Phase 3 MDD Program

Xenon’s Phase 3 MDD program includes three multicenter, randomized, double-blind, placebo-controlled clinical trials to evaluate the clinical efficacy, safety, and tolerability of 20 mg of azetukalner administered orally with food over the 6-week double-blind

period (DBP) as monotherapy treatment in approximately 450 patients with moderate-to-severe major depressive disorder (MDD) per study. The primary efficacy endpoint is the change from baseline in the HAM-D17 score at week 6 in patients who received azetukalner compared to placebo. Upon completion of the DBP, eligible patients may enter an OLE study for up to 12 months.

About Xenon Pharmaceuticals Inc.

Xenon Pharmaceuticals (Nasdaq: XENE) is a neuroscience-focused biopharmaceutical company dedicated to discovering, developing, and delivering life-changing therapeutics. We are advancing an ion channel product portfolio to address areas of high unmet medical need, including epilepsy and depression. Azetukalner, a novel, highly potent, selective Kv7 potassium channel opener, represents the most advanced, clinically validated potassium channel modulator in late-stage clinical development for multiple indications. 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; the potential efficacy, safety profile, future development plans in current and anticipated indications, addressable market, regulatory success and commercial potential of our and our partners' product candidates; the efficacy of our clinical trial designs; our ability to successfully develop and achieve milestones in our azetukalner and other pipeline and development programs, including the anticipated filing of INDs; the timing and results of our interactions with regulators; our ability to successfully develop and obtain regulatory approval of azetukalner and our other product candidates; and anticipated timing of topline data readout from our clinical trials of azetukalner. 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; 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 azetukalner, 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; the impact of market, industry, and regulatory conditions on clinical trial enrollment; the impact of competition; the impact of expanded product development and clinical activities on operating expenses; the impact of new or changing laws and regulations; the impact of unstable economic 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 U.S. 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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