Xenon Reports Second Quarter 2025 Financial Results & Business Update

August 11, 2025

– Phase 3 azetukalner X-TOLE2 FOS study patient recruitment complete, with topline data anticipated in early 2026
– Phase 3 azetukalner neuropsychiatric studies underway with X-NOVA3 in MDD and X-CEED in BPD recently initiated
– Phase 1 healthy volunteer study initiated for lead Nav1.7 development candidate for pain
– Darren Cline appointed as Chief Commercial Officer to lead commercial build and anticipated azetukalner launch
– Conference call at 4:30 pm ET today

VANCOUVER, British Columbia and BOSTON, Aug. 11, 2025 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq: XENE), a neuroscience-focused biopharmaceutical company dedicated to drug discovery, clinical development, and commercialization of life-changing therapeutics for patients in need, today reported financial results for the second quarter ended June 30, 2025, and provided a business update.

“The completion of patient recruitment for our Phase 3 X-TOLE2 study is a significant milestone in the development of azetukalner and keeps us on track to report topline results in early 2026 in anticipation of our first potential approval and commercial product as a company. We remain highly encouraged by the potential value proposition of azetukalner as a novel, next-generation treatment option for people living with the debilitating effects of uncontrolled seizures,” said Ian Mortimer, President and Chief Executive Officer of Xenon. “In addition, we remain excited about azetukalner’s potential beyond epilepsy, with Phase 3 programs underway across multiple neuropsychiatric indications, with the initiation of our Phase 3 X-CEED study in bipolar depression, alongside two clinical studies now underway in our ongoing Phase 3 X-NOVA program in major depressive disorder.”

“We also continue to make significant progress within our early-stage portfolio, advancing multiple drug candidates targeting promising sodium and potassium channel targets, and we are currently conducting two first-in-human studies within our Kv7 and Nav1.7 programs. As we enter this catalyst-rich period, we remain focused on advancing and delivering innovative therapeutics to patients living with epilepsy, depression, and pain.” continued Mr. Mortimer.

Business Highlights and Anticipated Milestones

Azetukalner Clinical Development

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 the treatment of multiple indications that include epilepsy, including focal onset seizures (FOS) and primary generalized tonic-clonic seizures (PGTCS), as well as neuropsychiatric disorders including major depressive disorder (MDD) and bipolar depression (BPD).

Epilepsy Programs

- Phase 3 X-TOLE2 clinical study of azetukalner in FOS has now completed patient recruitment, with topline data anticipated in early 2026.
- Phase 3 X-TOLE3 clinical study of azetukalner in FOS continues to enroll patients and is intended to support potential regulatory submissions in ex-U.S. jurisdictions.
- Phase 3 X-ACKT clinical study of azetukalner in PGTCS continues to enroll patients and is intended to support regulatory submissions for an additional epilepsy indication.
- The Company had four abstracts accepted to present at the 36th International Epilepsy Congress (IEC) taking place August 30 to September 3, 2025 in Lisbon, Portugal.

Neuropsychiatric Programs

- X-NOVA2 and X-NOVA3, the first two of three planned Phase 3 clinical trials evaluating azetukalner in patients with MDD, are now underway and screening patients.
- X-CEED, the first of two planned Phase 3 clinical studies evaluating azetukalner in patients with BPD I and BPD II depression, has been initiated.

Early-Stage Pipeline: Next-Generation Ion Channel Modulators

Xenon continues to expand its portfolio by leveraging its extensive expertise to discover and develop potassium and sodium channel therapeutics, with the goal of filing multiple INDs, or equivalent, in 2025.

- Phase 1 study in healthy adult participants is underway for XEN1120, a Kv7 channel opener in development for pain. Work remains ongoing with additional Kv7 development molecules and chemistries with the hypothesis that Kv7 may have utility in a broad range of therapeutic indications including seizure disorders, pain, and neuropsychiatric disorders, such as MDD and BPD.
- Phase 1 study was initiated for XEN1701, the Company's lead Nav1.7 development candidate for pain. IND-enabling work remains ongoing for additional Nav1.7 candidates. Nav1.7 is an important pain-related target, based on strong human genetic validation, which may represent a new class of medicines without the limitations of opioids.
- Nav1.1 lead candidate is expected to enter IND-enabling studies in 2025, with pre-clinical data suggesting that targeting Nav1.1 could potentially address the underlying cause and symptoms of Dravet Syndrome.
- Xenon plans to host multiple R&D webinars with focus on early-stage pipeline programs. The first webinar will take place on October 2, 2025, and focus on our approach to treating pain with drug candidates targeting Nav1.7 and Kv7.

Partnered Program

- As part of Xenon's ongoing collaboration with Neurocrine Biosciences, a Phase 1 study is underway for NBI-921355, an investigational, selective inhibitor of voltage-gated sodium channels Nav1.2 and Nav1.6 in development for the potential treatment of certain types of epilepsy.

Corporate

- The Company appointed Darren Cline to Chief Commercial Officer (CCO). Cline brings extensive commercial expertise to lead the transition of Xenon to a commercial-stage company with the anticipated launch of azetukalner across three potential indications.

Second Quarter Financial Results

- Cash and cash equivalents and marketable securities were $624.8 million as of June 30, 2025, compared to $754.4 million as of December 31, 2024. Based on current operating plans, including the completion of the azetukalner Phase 3 epilepsy studies and supporting late-stage clinical development of azetukalner in MDD and BPD, Xenon anticipates having sufficient cash to fund operations into 2027. As of June 30, 2025, there were 76,939,811 common shares and 2,173,081 pre-funded warrants outstanding.
- Research and development expenses for the quarter ended June 30, 2025 were $75.0 million, compared to $49.7 million for the same period in 2024. The increase of $25.3 million was primarily attributable to the ongoing azetukalner Phase 3 clinical trials in epilepsy and MDD, as well as start-up costs for the azetukalner Phase 3 BPD clinical trial, and increased personnel-related costs due to an increase in employee headcount.
- General and administrative expenses for the quarter ended June 30, 2025 were $19.2 million, compared to $19.4 million for the same period in 2024.
- Other income for the quarter ended June 30, 2025 was $8.9 million, compared to $10.8 million for the same period in 2024. The decrease of $2.0 million was primarily attributable to lower interest income, partially offset by an increase in foreign exchange gain.
- Net loss for the quarter ended June 30, 2025 was $84.7 million, compared to $57.9 million for the same period in 2024. The increase in net loss was primarily attributable to higher research and development expenses driven by the azetukalner program, increased personnel-related costs, and lower interest income.

Conference Call Information

Xenon will host a conference call and webcast today at 4:30 pm Eastern Time (1:30 pm Pacific Time) to discuss its second quarter 2025 results. A listen-only webcast can be accessed on the Investors section of the Xenon website, with a replay available following the event. Participants can access the conference call by dialing (800) 715-9871 or (646) 307-1963 for international callers and referencing conference ID 4102397.

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 X-NOVA 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 open-label extension (OLE) study for up to 12 months.

About Azetukalner Phase 3 BPD X-CEED Program

Xenon's Phase 3 BPD program includes two 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 400 patients per study with bipolar depression (BPD) I or II. The primary efficacy endpoint is the change from baseline in the MADRS 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 drug discovery, clinical development, and commercialization of life-changing therapeutics for patients in need. Xenon's lead molecule, azetukalner, is a novel, potent, selective Kv7 potassium channel opener in Phase 3 clinical trials for the treatment of epilepsy, major depressive disorder (MDD) and bipolar depression (BPD). Xenon is also advancing an early-stage portfolio of multiple promising potassium and sodium channel modulators, including Kv7 and Nav1.7 programs in Phase 1 development for the potential treatment of pain. Xenon has offices in Vancouver, British Columbia, and Boston, Massachusetts. For more information, visit www.xenon-pharma.com and follow us on LinkedIn and X.

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 and NDAs; 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 or completion 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.

Contacts:

For Investors:
Chad Fugere
Vice President, Investor Relations
(857) 675-7275
investors@xenon-pharma.com

For Media:
Colleen Alabiso
Senior Vice President, Corporate Affairs
(617) 671-9238
media@xenon-pharma.com

XENON PHARMACEUTICALS INC.

Condensed Consolidated Balance Sheets
(Expressed in thousands of U.S. dollars)

	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents and marketable securities	$ 487,545	$ 626,905
Other current assets	12,707	8,359
Marketable securities, long-term	137,297	127,496
Other long-term assets	36,732	35,379
Total assets	$ 674,281	$ 798,139
Liabilities		
Current liabilities:		
Accounts payable and accrued liabilities	$ 31,588	$ 34,221
Other current liabilities	1,459	1,369
Other long-term liabilities	7,256	7,646
Total liabilities	$ 40,303	$ 43,236
Shareholders' equity	$ 633,978	$ 754,903
Total liabilities and shareholders' equity	$ 674,281	$ 798,139

XENON PHARMACEUTICALS INC.

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Expressed in thousands of U.S. dollars except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue	$ —	$ —	$ 7,500	$ —
Operating expenses:				
Research and development	74,985	49,702	136,185	93,952
General and administrative	19,244	19,402	38,282	34,193
Total operating expenses	94,229	69,104	174,467	128,145
Loss from operations	(94,229)	(69,104)	(166,967)	(128,145)

Other income	8,897	10,847	17,015	22,369
Loss before income taxes	(85,332)	(58,257)	(149,952)	(105,776)
Income tax recovery (expense)	626	333	199	(79)
Net loss	$ (84,706)	$ (57,924)	$ (149,753)	$ (105,855)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	949	(443)	1,725	(2,135)
Comprehensive loss	$ (83,757)	$ (58,367)	$ (148,028)	$ (107,990)
Net loss per common share:				
Basic and diluted	$ (1.07)	$ (0.75)	$ (1.90)	$ (1.36)
Weighted average common shares outstanding:				
Basic and diluted	78,953,445	77,671,128	78,820,474	77,632,864

Source: Xenon Pharmaceuticals Inc.