Xenon Pharmaceuticals Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Corporate Update

February 29, 2024

XEN1101 Phase 3 epilepsy program continues to progress with X-TOLE2 and X-TOLE3 in focal onset seizures and X-ACKT in primary generalized tonic-clonic seizures; completion of patient enrollment in X-TOLE2 expected in late 2024 to early 2025

Planned "end-of-Phase 2" meeting with the FDA in April to support the initiation of XEN1101 Phase 3 program in major depressive disorder in the second half of the year

Strong financial position with approximately $930 million to fully support XEN1101 Phase 3 programs in epilepsy and major depressive disorder and expected cash runway into 2027

Conference call at 4:30 pm ET today

VANCOUVER, British Columbia, Feb. 29, 2024 (GLOBE NEWSWIRE) -- Xenon Pharmaceuticals Inc. (Nasdaq:XENE), a neurology-focused biopharmaceutical company, today reported financial results for the fourth quarter and full year ended December 31, 2023 and provided a corporate update.

Mr. Ian Mortimer, Xenon's President and Chief Executive Officer, stated, "I am proud of the progress made across our pipeline in 2023, culminating in the release of topline data from our XEN1101 Phase 2 proof-of-concept X-NOVA clinical trial, which demonstrated clinically meaningful drug activity in depression. We are advancing XEN1101 in major depressive disorder with plans to conduct an end-of-Phase 2 meeting with the FDA in April, and we expect to initiate our Phase 3 program in the second half of this year. We also continue to evaluate other neurological indications that present a potential fit for XEN1101's novel mechanism and product profile. Importantly, we believe the Kv7 mechanism of XEN1101 may have broad applicability, which supports our comprehensive strategy to pursue multiple streams of late-stage clinical development in epilepsy and depression."

Mr. Mortimer added, "We continue to make progress in our broad Phase 3 epilepsy programs, and we expect patient enrollment in X-TOLE2 to be completed in late 2024 to early 2025. We remain focused on executing our clinical development plans in epilepsy and are encouraged by the enthusiasm for XEN1101 from clinical investigators and the broader neurology community."

"We have also made significant advancements across our pre-clinical pipeline, leveraging our expertise in ion channel drug discovery to identify new product candidates across various targets including pain and seizure disorders. With a strong balance sheet to support our ambitious plans, we are looking forward to advancing and growing our broad pipeline, with the goal of improving outcomes for patients in areas of high unmet medical need."

Highlights and Anticipated Milestones

XEN1101

XEN1101 is a novel, potent Kv7 potassium channel opener being developed for the treatment of epilepsy, major depressive disorder, or MDD, and potentially other neurological disorders.

XEN1101 for Epilepsy (Focal Onset Seizures)

Xenon's XEN1101 Phase 3 epilepsy program includes two identical Phase 3 clinical trials, called X-TOLE2 and X-TOLE3, that are designed closely after the Phase 2b X-TOLE clinical trial. These multicenter, randomized, double-blind, placebo-controlled trials are evaluating the clinical efficacy, safety, and tolerability of 15 mg or 25 mg of XEN1101 administered with food as adjunctive treatment in approximately 360 patients per study with focal onset seizures, or FOS. Xenon anticipates patient enrollment in X-TOLE2 will be completed in late 2024 to early 2025.

XEN1101 for Epilepsy (Primary Generalized Tonic-Clonic Seizures)

Xenon's Phase 3 X-ACKT clinical trial is intended to support potential regulatory submissions in an additional epilepsy indication of primary generalized tonic-clonic seizures, or PGTCS. This multicenter, randomized, double-blind, placebo-controlled trial is evaluating the clinical efficacy, safety, and tolerability of 25 mg of XEN1101 administered with food as adjunctive treatment in approximately 160 patients with PGTCS.

XEN1101 for Epilepsy (Open-Label Extension)

Upon completion of the double-blind period in X-TOLE2, X-TOLE3, or X-ACKT, eligible patients may enter an open-label extension, or OLE, study for up to three years. In addition, the ongoing X-TOLE Phase 2b OLE has been extended from five

to seven years and continues to generate important long-term data for XEN1101.

XEN1101 for Major Depressive Disorder

In November 2023, Xenon reported topline results from the Phase 2 proof-of-concept X-NOVA clinical trial, which evaluated the clinical efficacy, safety, and tolerability of 10 mg and 20 mg of XEN1101 in 168 patients with moderate to severe MDD.

Xenon anticipates participating in an “end-of-Phase 2” meeting with the U.S. Food and Drug Administration in April to support the initiation of its late-stage XEN1101 clinical program in MDD, which will include three Phase 3 clinical trials, with the first Phase 3 study expected to initiate in the second half of 2024. Xenon is also evaluating other potential indications for the future development of XEN1101.

In addition, Xenon is collaborating with the Icahn School of Medicine at Mount Sinai to support an ongoing investigator-sponsored Phase 2 proof-of-concept, randomized, parallel-arm, placebo-controlled multi-site study of XEN1101 for the treatment of MDD in approximately 60 subjects.

Other Pipeline Opportunities

Xenon continues to leverage its extensive ion channel expertise and drug discovery capabilities to identify validated drug targets and develop new product candidates. The near-term focus is on development candidates targeting Kv7, Nav1.1 and Nav1.7, and Xenon expects multiple candidates will enter IND-enabling studies in 2024 and 2025.

Partnered Program: NBI-921352

Xenon has an ongoing collaboration with Neurocrine Biosciences to develop treatments for epilepsy. Neurocrine Biosciences has an exclusive license to XEN901, now known as NBI-921352, a selective Nav1.6 sodium channel inhibitor. A Phase 2 clinical trial is ongoing evaluating NBI-921352 in patients aged between 2 and 21 years with SCN8A developmental and epileptic encephalopathy.

Fourth Quarter and Full Year Financial Results

Cash and cash equivalents and marketable securities were $930.9 million as of December 31, 2023, compared to $720.8 million as of December 31, 2022. The increase was primarily the result of the completion of the Company’s public equity offering in December 2023. Based on current operating plans, including the completion of the XEN1101 Phase 3 epilepsy studies and fully supporting late-stage clinical development of XEN1101 in MDD, Xenon anticipates having sufficient cash to fund operations into 2027. As of December 31, 2023, there were 75,370,977 common shares and 2,173,081 pre-funded warrants outstanding.

Research and development expenses were $41.1 million for the fourth quarter of 2023, and $167.5 million for the year ended 2023, compared to $34.8 million and $105.8 million for the same periods in 2022, respectively. The increase in research and development expenses for the year was primarily attributable to expenses related to Xenon's enrollment of the XEN1101 Phase 3 epilepsy clinical trials, manufacturing to support current and future clinical trials and a potential NDA submission, the completion of the X-NOVA Phase 2 MDD clinical trial, personnel-related costs due to an increase in employee headcount, and higher stock-based compensation expense. These increases were partially offset by a decrease in expenses for the XEN496 program as a result of Xenon's decision in early 2023 to no longer pursue the clinical development of XEN496.

General and administrative expenses were $12.6 million for the fourth quarter of 2023, and $46.5 million for the year ended 2023, compared to $8.5 million and $32.8 million for the same periods in 2022, respectively. The increase in general and administrative expenses for the year was primarily attributable to personnel-related costs due to an increase in employee headcount and higher stock-based compensation expense, and an increase in professional and consulting fees.

Other income was $8.7 million for the fourth quarter of 2023, and $31.4 million for the year ended 2023, compared to $7.1 million and $3.9 million for the same periods in 2022. The increase in other income for the year was primarily attributable to higher interest income and an unrealized fair value gain on trading securities recognized in 2023 compared to an unrealized fair value loss for the same period in 2022.

Net loss was $44.7 million for the fourth quarter of 2023, and $182.4 million for the year ended 2023, compared to $37.4 million and $125.4 million for the same periods in 2022, respectively. The increase in net loss for the year was primarily attributable to higher research and development expenses driven by the ongoing XEN1101 Phase 3 epilepsy clinical trials, and increased personnel-related costs and stock-based compensation expense across the organization, partially offset by an increase in 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 fourth quarter and full year 2023 results. The audio webcast can be accessed on the Investors section of the Xenon website. Participants can access the live conference call by dialing (800) 715-9871, or (646) 307-1963 for international callers, and provide conference ID number 9376408. A replay of the webcast will be available on the website approximately one hour after the conclusion of the event and will be archived for approximately one month.

About Xenon Pharmaceuticals Inc.

Xenon Pharmaceuticals (Nasdaq:XENE) is a neuroscience-focused biopharmaceutical company committed to discovering, developing, and commercializing innovative therapeutics to improve the lives of people living with neurological and psychiatric disorders. We are advancing a novel product pipeline to address areas of high unmet medical need, including epilepsy and depression. 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 XEN1101 and other pipeline and development programs; the timing and results of our interactions with regulators; our ability to successfully develop and obtain regulatory approval of XEN1101 and our other product candidates; anticipated enrollment in our clinical trials of XEN1101 and the timing thereof; and our expectation that we will have sufficient cash to fund operations into 2027. 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 XEN1101, 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 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 pandemics, epidemics and other public health crises on our research and clinical development plans and timelines and results of operations, including impact on our clinical trial sites, collaborators, regulatory agencies and related review times, and contractors who act for or on our behalf; 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 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.

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

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents and marketable securities	$ 638,082	$ 592,087
Other current assets	6,880	8,211
Marketable securities, long-term	292,792	128,682
Other long-term assets	27,044	25,166
Total assets	$ 964,798	$ 754,146
Liabilities		
Current liabilities:		
Accounts payable and accrued expenses	$ 25,974	$ 22,214
Other current liabilities	1,299	488
Other long-term liabilities	9,604	9,947
Total liabilities	$ 36,877	$ 32,649
Shareholders' equity	$ 927,921	$ 721,497

Total liabilities and shareholders' equity	$ 964,798	$ 754,146

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 December 31,		Year Ended December 31,	
	2023	**2022**	**2023**	**2022**
Revenue	$ —	$ —	$ —	$ 9,434
Operating expenses:				
Research and development	41,076	34,830	167,512	105,767
General and administrative	12,619	8,501	46,542	32,810
	53,695	43,331	214,054	138,577
Loss from operations	(53,695)	(43,331)	(214,054)	(129,143)
Other income	8,747	7,075	31,369	3,888
Loss before income taxes	(44,948)	(36,256)	(182,685)	(125,255)
Income tax recovery (expense)	205	(1,139)	292	(118)
Net loss	(44,743)	(37,395)	(182,393)	(125,373)
Net loss attributable to preferred shareholders	—	—	—	(437)
Net loss attributable to common shareholders	$ (44,743)	$ (37,395)	$ (182,393)	$ (124,936)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	$ 2,876	$ (45)	$ 2,923	$ (2,010)
Comprehensive loss	$ (41,867)	$ (37,440)	$ (179,470)	$ (127,383)
Net loss per common share:				
Basic and diluted	$ (0.64)	$ (0.57)	$ (2.73)	$ (2.06)
Weighted-average common shares outstanding:				
Basic and diluted	69,968,038	65,657,784	66,889,005	60,542,142

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