Xilio Therapeutics Announces Initiation of Phase 2 Trial for Efarindodekin Alfa (XTX301), a Tumor-Activated IL-12, and Achievement of $17.5 Million Development Milestone Under Exclusive License Agreement with Gilead

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Phase 1 data for efarindodekin alfa (XTX301) demonstrate promising anti-tumor activity in patients with advanced solid tumors

Achievement of milestone extends anticipated cash runway into first quarter of 2027

WALTHAM, Mass., Sept. 09, 2025 (GLOBE NEWSWIRE) -- Xilio Therapeutics, Inc. (Nasdaq: XLO), a clinical-stage biotechnology company discovering and developing tumor-activated immuno-oncology therapies for people living with cancer, today announced the initiation of patient dosing in Phase 2 of an ongoing Phase 1/2 clinical trial evaluating efarindodekin alfa (XTX301), a tumor-activated IL-12, as a monotherapy in patients with certain advanced solid tumors. In addition, today Xilio announced the achievement of a $17.5 million development milestone under Xilio's license agreement with Gilead Sciences, Inc. (Gilead) and updated data from the ongoing Phase 1 trial for efarindodekin alfa.

"We are pleased to announce the initiation of the Phase 2 clinical trial for efarindodekin alfa (XTX301), a tumor-activated IL-12, in patients with advanced solid tumors," said René Russo, Pharm.D., president and chief executive officer of Xilio. "The achievement of this important milestone highlights the promising Phase 1 data demonstrated for efarindodekin alfa to date, including two partial responses in late-line patients with advanced solid tumors and a generally well-tolerated safety profile. These data also provide further clinical validation for our proprietary masking technology and approach, which we believe is best-in-class and has potential across a wide range of therapies and modalities."

"We are encouraged by the totality of data observed to date for efarindodekin alfa (XTX301) as a monotherapy in patients with advanced solid tumors, and we are excited for the potential that IL-12 has to treat a broad range of tumor types," said Bernard Fine, vice president, oncology early development at Gilead. "We look forward to advancing the efarindodekin alfa (XTX301) program in the Phase 2 trial in partnership with Xilio."

Efarindodekin alfa (XTX301): tumor-activated IL-12

Efarindodekin alfa (XTX301) is an investigational tumor-activated IL-12 designed to potently stimulate anti-tumor immunity and reprogram the tumor microenvironment (TME) of poorly immunogenic "cold" tumors towards an inflamed or "hot" state. In March 2024, Xilio entered into an exclusive license agreement with Gilead related to Xilio's tumor-activated IL-12 program, including efarindodekin alfa. Xilio is evaluating efarindodekin alfa as a monotherapy in an ongoing Phase 1/2 clinical trial in patients with advanced solid tumors.

- As of a data cutoff date of September 2, 2025, at dose levels up to the recommended Phase 2 dose (RP2D), efarindodekin alfa has been generally well-tolerated in Phase 1, and the majority of treatment-related adverse events were Grade 1 or 2.
- In Phase 1, as of the data cutoff date, efarindodekin alfa has also demonstrated encouraging anti-tumor activity, including two partial responses in patients with advanced solid tumors (one confirmed, one unconfirmed), as well as sustained interferon gamma (IFNɣ) signaling without evidence of tachyphylaxis throughout treatment cycles.
- Based on these promising Phase 1 data, Xilio recently selected an initial RP2D and schedule for efarindodekin alfa and initiated patient dosing in the Phase 2 portion of the trial evaluating efarindodekin alfa as a monotherapy in patients with certain advanced solid tumors. In connection with the initiation of Phase 2, Xilio achieved a development milestone of $17.5 million.
- Xilio recently completed enrollment in Phase 1A monotherapy dose escalation and evaluation of those patients is ongoing. In addition, Xilio continues to enroll patients in the Phase 1B monotherapy dose expansion portion of the ongoing Phase 1/2 clinical trial of efarindodekin alfa.

Efarindodekin alfa has not been approved by any regulatory agency and its efficacy and safety have not been established.

Financial Guidance

As of June 30, 2025, Xilio had cash and cash equivalents of $121.6 million. Based on its current operating plans, Xilio anticipates that its cash and cash equivalents as of June 30, 2025, together with the $17.5 million development milestone achieved under the license agreement with Gilead, will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the first quarter of 2027. Xilio expects to receive payment of the $17.5 million development milestone by the fourth quarter of 2025.

About the Gilead License Agreement

In March 2024, Xilio entered into an exclusive global license agreement with Gilead to develop and commercialize efarindodekin alfa (XTX301), a tumor-activated IL-12, and specified other molecules directed to IL-12.

Xilio is responsible for conducting clinical development for efarindodekin alfa through the initial Phase 2 portion of the ongoing Phase 1/2 clinical trial. Following the delivery by Xilio of a specified clinical data package for efarindodekin alfa related to the Phase 1/2 clinical trial, Gilead can elect to transition responsibilities for the development and commercialization of efarindodekin alfa to Gilead, subject to the terms of the license agreement and payment by Gilead of a $75.0 million transition fee.

If Gilead exercises its option for efarindodekin alfa, Xilio will be eligible to receive up to $500.0 million in specified development, regulatory and sales-based milestones and will be eligible to receive tiered royalties ranging from high single digits to mid-teens on annual global net product sales.

About Efarindodekin Alfa (XTX301) and the Phase 1/2 Clinical Trial

Efarindodekin alfa (XTX301) is an investigational masked IL-12 designed to potently stimulate anti-tumor immunity and reprogram the tumor microenvironment (TME) of poorly immunogenic "cold" tumors towards an inflamed or "hot" state. In March 2024, Xilio entered into an exclusive license agreement with Gilead Sciences, Inc. for Xilio's tumor-activated IL-12 program, including efarindodekin alfa. Xilio is currently evaluating the safety and tolerability of efarindodekin alfa as a monotherapy in patients with advanced solid tumors in the Phase 1 portion of a first-in-human, multi-center, open-label Phase 1/2 clinical trial and the safety and efficacy of efarindodekin alfa as a monotherapy in the Phase 2 portion in patients with advanced solid tumors. The Phase 2 portion of the trial is anticipated to enroll approximately 40 patients in specific tumor types at multiple sites in the United States. Please refer to NCT05684965 on www.clinicaltrials.gov for additional details.

About Xilio Therapeutics

Xilio Therapeutics is a clinical-stage biotechnology company discovering and developing tumor-activated, or masked, immuno-oncology (I-O) therapies with the goal of significantly improving outcomes for people living with cancer without the systemic side effects of current I-O treatments. The company is leveraging its proprietary platform to advance a pipeline of novel, tumor-activated I-O molecules that are designed to optimize the therapeutic index by localizing anti-tumor activity within the tumor microenvironment. Learn more by visiting www.xiliotx.com and follow us on LinkedIn (Xilio Therapeutics, Inc.).

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans, expectations, development timelines and anticipated milestones for Xilio's programs; the timing of data releases or program updates; the timing and receipt of future payments under Xilio's collaboration with Gilead; the potential benefits of efarindodekin alfa in any indication; the sufficiency of, and the period in which Xilio expects to have, cash to fund its operations; and Xilio's strategy, goals and anticipated financial performance, milestones, business plans and focus. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "seek," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of important risks, uncertainties and other factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks related to general market conditions and geopolitical uncertainties; risks and uncertainties related to ongoing and planned research and development activities, including initiating, conducting or completing preclinical studies and clinical trials and the timing and results of such preclinical studies or clinical trials; the delay of any current or planned preclinical studies or clinical trials or the development of Xilio's current or future product candidates; Xilio's ability to obtain and maintain sufficient preclinical and clinical supply of current or future product candidates; Xilio's ability to advance multiple early stage masked T cell engager programs; initial, preliminary or interim preclinical or clinical data or results may not be replicated in or predictive of future preclinical or clinical data or results; Xilio's ability to successfully demonstrate the safety and efficacy of its product candidates and gain approval of its product candidates on a timely basis, if at all; results from preclinical studies or clinical trials for Xilio's product candidates may not support further development of such product candidates; actions of regulatory agencies may affect the initiation, timing and progress of current or future clinical trials; Xilio's ability to obtain, maintain and enforce patent and other intellectual property protection for current or future product candidates; Xilio's need to obtain additional cash resources to advance its pipeline of tumor-activated I-O molecules; the impact of international trade policies on Xilio's business, including U.S. and China trade policies; and Xilio's ability to maintain its collaboration or partnership agreements with AbbVie, Gilead and Roche. These and other risks and uncertainties are described in greater detail in the sections entitled "Risk Factor Summary" and "Risk Factors" in Xilio's filings with the U.S. Securities and Exchange Commission ("SEC"), including Xilio's most recent Quarterly Report on Form 10-Q and any other filings that Xilio has made or may make with the SEC in the future. Any forward-looking statements contained in this press release represent Xilio's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Xilio explicitly disclaims any obligation to update any forward-looking statements.

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