



Pacira BioSciences Presents New Data on the Effects of Clinical Immunogenicity on Locally Administered PCRX-201 in Patients with Moderate to Severe Osteoarthritis of the Knee

May 15, 2025

-- Early clinical data indicates that Pre-existing and treatment-induced anti-Ad5 neutralizing antibodies do not impact the safety and effectiveness of PCRX-201 --

-- An oral presentation of the data to be featured at the ASGCT Annual Meeting --

BRISBANE, Calif., May 15, 2025 (GLOBE NEWSWIRE) -- Pacira BioSciences, Inc. (NASDAQ: PCRX), the industry leader in the delivery of innovative, non-opioid pain therapies to transform the lives of patients, today announced new preliminary data which suggests that clinical immunogenicity does not reduce sustained improvements in knee pain, stiffness and function provided by its gene therapy candidate, PCRX-201 (enekinragene inzadenovec), following local administration in patients with mild, moderate, as well as severe osteoarthritis of the knee. The research findings are being presented during a podium session at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting in New Orleans on May 15, 2025, at 2:15 CT.

"These are encouraging results supporting the potential of PCRX-201 as an effective and durable treatment option for patients with osteoarthritis of the knee, many of whom currently rely on therapies that deliver only short-term, limited relief," said Mijeong Kim, immunology and pharmacology leader at Pacira BioSciences and lead investigator of the research. "Natural immune responses are a major obstacle for gene therapies, since they can hinder the effectiveness of the treatment and increase adverse reactions. This preliminary data indicates that neither pre-existing nor treatment-induced immunogenicity compromise the safety or long-lasting efficacy of PCRX-201."

PCRX-201 features an innovative design based on the company's proprietary high-capacity adenovirus, or HCAd, gene therapy vector platform. The HCAd vector is based on adenovirus serotype 5 (Ad5), a well understood serotype that is very common in community circulation. PCRX-201 is injected locally into the knee joint to boost cellular production of interleukin-1 receptor antagonist (IL-1Ra) and block interleukin-1 pathway activation to improve chronic inflammation, pain, and function. PCRX-201's unique design also features an inducible promoter to mimic the body's natural response to inflammation by "turning on" the expression of IL-1Ra when inflammation is present in the joint and turning off expression once inflammation is quelled.

Study Details

The open-label, phase 1 trial evaluated the effect that pre-existing or treatment-induced anti-Ad5 neutralizing antibodies (NABs) have on PCRX-201 and its impact on dosing and redosing strategy. The study enrolled 72 participants aged 30 to 80 who received a single low, middle or high dose of PCRX-201. One of the two study cohorts (N=36) was pretreated with an intraarticular corticosteroid (methylprednisolone 40 mg) prior to the PCRX-201 injection. The presence of NAB titers in patients' blood and synovial fluid (SF), as well as the levels of IL-1Ra in SF, were measured over 52 weeks.

The data indicates that pre-existing NABs did not affect the efficacy or safety of PCRX-201 at any of the three doses studied. All doses achieved improved pain, stiffness and function as measured by WOMAC-A and WOMAC-B (The Western Ontario and McMaster Universities Osteoarthritis Index). The findings also demonstrated that using a corticosteroid treatment before PCRX-201 administration appears to help reduce treatment-induced NAB titers and dose-related swelling of the knee joint. Additionally, most serum NAB titers fell below the highest observed baseline levels within 38 to 52 weeks.

No serious treatment-emergent AEs related to the treatment or procedure were reported regardless of steroid pretreatment or dose level administered. Treatment-related joint effusions (swelling) were the most common AE, occurring in 36% of patients who received steroid pretreatment vs 61% of patients who were not pretreated. The majority of effusions were mild to moderate in severity and resolved in a median of 33 days among patients in the pretreated group.

"Advancing gene therapy as an approach to treating pain is a natural progression of Pacira's deep commitment to innovation in non-opioid therapies and delivers on our 5x30 strategy for growth and value creation," said Frank D. Lee, chief executive officer of Pacira Biosciences. "With our Phase 2 study of PCRX-201 currently underway, we see great potential for offering a long-lasting pain relief option to the 14 million patients living with osteoarthritis of the knee, an often debilitating condition that can severely impact mobility and quality of life."

In March 2024, PCRX-201 became the first-ever gene therapy product candidate in osteoarthritis to receive Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA).

RMAT designation provides the benefits of intensive FDA guidance on efficient drug development, including the ability for early interactions with the FDA to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the Biologics License Application (BLA), and other opportunities to

expedite development and review. PCRX-201 was also granted Advanced Therapy Medicinal Products (ATMP) designation by the European Medicines Agency in May 2023.

Given the promising Phase 1 results, dosing is underway in a [Phase 2 study](#) of PCRX-201 ([the ASCEND study](#)) for the treatment of osteoarthritis of the knee.

About PCRX-201 (enekenragene inzadenovec)

PCRX-201 (enekenragene inzadenovec) features an innovative design based on the company's proprietary high-capacity adenovirus vector platform. It is currently being studied in the fundamental, underlying chronic inflammatory processes that contribute to "wear and tear" over time in osteoarthritis of the knee, a condition that affects more than 14 million individuals in the U.S. today.

In November 2024, Pacira reported promising data from a large Phase 1 study in which PCRX-201 provided sustained improvements in knee pain, stiffness, and function through two years following local administration, with a well-tolerated safety profile. PCRX-201 has received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration and Advanced Therapy Medicinal Products (ATMP) designation from the European Medicines Agency. PCRX-201 is the first gene therapy to achieve these clinical results and earn these regulatory designations in osteoarthritis of the knee – a testament to its promise and potential.

About Pacira

Pacira delivers innovative, non-opioid pain therapies to transform the lives of patients. Pacira has three commercial-stage non-opioid treatments: EXPAREL® (bupivacaine liposome injectable suspension), a long-acting local analgesic currently approved for infiltration, fascial plane block, and as an interscalene brachial plexus nerve block, an adductor canal nerve block, and a sciatic nerve block in the popliteal fossa for postsurgical pain management; ZILRETTA® (triamcinolone acetone extended-release injectable suspension), an extended-release, intra-articular injection indicated for the management of osteoarthritis knee pain; and iovera®, a novel, handheld device for delivering immediate, long-acting, drug-free pain control using precise, controlled doses of cold temperature to a targeted nerve. The Company is also advancing the development of PCRX-201 (enekenragene inzadenovec), a novel, locally administered gene therapy with the potential to treat large prevalent diseases like osteoarthritis. To learn more about Pacira, visit www.pacira.com.

Forward-Looking Statements

Any statements in this press release about Pacira's future expectations, plans, trends, outlook, projections and prospects, and other statements containing the words "believes," "anticipates," "plans," "estimates," "expects," "intends," "may," "will," "would," "could," "can" and similar expressions, constitute forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and the Private Securities Litigation Reform Act of 1995, including, without limitation, statements related to: the settlement described herein, '5x30', our growth and business strategy; our future outlook, our intellectual property and patent terms, our growth and future operating results and trends, our strategy, plans, objectives, expectations (financial or otherwise) and intentions, future financial results and growth potential, including our plans with respect to the repayment of our indebtedness, anticipated product portfolio, development programs, development of products, strategic alliances and other statements that are not historical facts. For this purpose, any statement that is not a statement of historical fact should be considered a forward-looking statement. We cannot assure you that our estimates, assumptions and expectations will prove to have been correct. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including risks relating to, among others: the failure to realize the anticipated benefits and synergies from the acquisition of GQ Bio Therapeutics GmbH; risks associated with acquisitions, such as the risk that the acquired businesses will not be integrated successfully, that such integration may be more difficult, time-consuming or costly than expected or that the expected benefits of the transaction will not occur; our manufacturing and supply chain, global and U.S. economic conditions (including inflation and rising interest rates), and our business, including our revenues, financial condition, cash flow and results of operations; the success of our sales and manufacturing efforts in support of the commercialization of EXPAREL, ZILRETTA and iovera®; the rate and degree of market acceptance of EXPAREL, ZILRETTA and iovera®; the size and growth of the potential markets for EXPAREL, ZILRETTA and iovera® and our ability to serve those markets; our plans to expand the use of EXPAREL, ZILRETTA and iovera® to additional indications and opportunities, and the timing and success of any related clinical trials for EXPAREL, ZILRETTA and iovera®; the commercial success of EXPAREL, ZILRETTA and iovera®; the related timing and success of U.S. Food and Drug Administration supplemental New Drug Applications and premarket notification 510(k)s; the related timing and success of European Medicines Agency Marketing Authorization Applications; our plans to evaluate, develop and pursue additional product candidates utilizing our proprietary multivesicular liposome ("pMVL") drug delivery technology; the approval of the commercialization of our products in other jurisdictions; clinical trials in support of an existing or potential pMVL-based product; our commercialization and marketing capabilities; our ability to successfully complete capital projects; the outcome of any litigation; the recoverability of our deferred tax assets; assumptions associated with contingent consideration payments; assumptions used for estimated future cash flows associated with determining the fair value of the Company; the anticipated funding or benefits of our share repurchase program; and factors discussed in the "Risk Factors" of our most recent Annual Report on Form 10-K and in other filings that we periodically make with the Securities and Exchange Commission (the "SEC"). In addition, the forward-looking statements included in this press release represent our views as of the date of this press release. Important factors could cause actual results to differ materially from those indicated or implied by forward-looking statements, and as such we anticipate that subsequent events and developments will cause our views to change. Except as required by applicable law, we undertake no intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, and readers should not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this press release.

These forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from those expressed or implied by these statements. These factors include the matters discussed and referenced in the "Risk Factors" of our most recent Annual Report on Form 10-K and in other filings that we periodically make with the SEC.

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