Kiniksa Pharmaceuticals Announces U.S. Orphan Drug Designation for KPL-387 for the Treatment of Pericarditis

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LONDON, Oct. 17, 2025 (GLOBE NEWSWIRE) -- Kiniksa Pharmaceuticals International, plc (Nasdaq: KNSA) (Kiniksa), a biopharmaceutical company developing and commercializing novel therapies for diseases with unmet need, with a focus on cardiovascular indications, today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to KPL-387 for the treatment of pericarditis, which includes recurrent pericarditis. KPL-387 is an independently developed monoclonal antibody that binds human interleukin-1 receptor 1 (IL-1R1), inhibiting the signaling activity of the cytokines interleukin-1α (IL-1α) and interleukin-1β (IL-1β).

"We are pleased to announce that yesterday the FDA granted Orphan Drug Designation to KPL-387 for the treatment of pericarditis," said John F. Paolini, M.D., Ph.D., FACC, Chief Medical Officer of Kiniksa. "We are committed to helping patients with this rare and debilitating disease and believe that KPL-387 could provide an additional treatment option for patients by potentially enabling dosing with a single monthly subcutaneous self-injection in a liquid formulation. Data from the Phase 2 dose-focusing portion of the KPL-387 Phase 2/3 recurrent pericarditis trial are expected in the second half of 2026."

About Orphan Drug Designation
The FDA grants Orphan Drug Designation status to products that treat rare diseases, providing incentives to sponsors developing drugs or biologics. The FDA's definition of rare diseases includes those affecting fewer than 200,000 people in the U.S. at the time of designation. Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. Additionally, Orphan Drug Designation waives the requirement to conduct pediatric studies for the product in the disease it is designated.

About Kiniksa
Kiniksa is a biopharmaceutical company dedicated to improving the lives of patients suffering from debilitating diseases by discovering, acquiring, developing, and commercializing novel therapies for diseases with unmet need, with a focus on cardiovascular indications. Kiniksa's portfolio of assets is based on strong biologic rationale or validated mechanisms and offers the potential for differentiation. For more information, please visit www.kiniksa.com.

About KPL-387
KPL-387 is an independently developed, investigational, fully human immunoglobulin IgG2 monoclonal antibody that binds human IL-1R1, inhibiting the signaling of the cytokines IL-1α and IL-1β. Kiniksa believes KPL-387 could expand the treatment options for recurrent pericarditis patients by potentially enabling dosing with a single monthly subcutaneous self-injection in a liquid formulation. In October 2025, the FDA granted Orphan Drug Designation to KPL-387 for the treatment of pericarditis.

Forward-Looking Statements
This press release contains forward-looking statements. In some cases, you can identify forward looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these identifying words. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation, statements regarding: our belief that KPL-387 could provide an additional treatment option for patients by potentially enabling dosing with a single monthly subcutaneous self-injection in a liquid formulation; our expectation to have data from the Phase 2 dose-focusing portion of the KPL-387 Phase 2/3 recurrent pericarditis trial in the second half of 2026; and our belief that our portfolio of assets offers the potential for differentiation.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including without limitation, the following: delays or difficulty in enrollment of patients in, and activation or continuation of sites for, our clinical trials; delays or difficulty in completing our clinical trials as originally designed; our inability to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities; potential for applicable regulatory authorities to not accept our filings, delay or deny approval of any of our product candidates or require additional data or trials to support approval; our reliance on third parties as the sole source of supply of the drug substance and drug product used in our product candidates; raw material, important ancillary product and drug substance and/or drug product shortages; our reliance on third parties to conduct research, clinical trials, and/or certain regulatory activities for our product candidates; complications in coordinating requirements, regulations and guidelines of regulatory authorities across jurisdictions for our clinical trials; changes in our operating plan or funding requirements; and the impact of global economic policy, including any uncertainty in national and international markets.

These and other important factors discussed in our filings with the U.S. Securities and Exchange Commission, including under the caption "Risk Factors" contained therein, could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. Except as required by law, we disclaim any intention or obligation to update or revise any forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

Every Second Counts! ®

Kiniksa Investor & Media Contact
Jonathan Kirshenbaum
(781) 829-3949
jkirshenbaum@kiniksa.com