Kiniksa Announces U.S. Orphan Drug Designation for Rilonacept for the Treatment of Pericarditis

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HAMILTON, Bermuda, July 16, 2020 (GLOBE NEWSWIRE) -- Kiniksa Pharmaceuticals, Ltd. (Nasdaq: KNSA) ("Kiniksa"), a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients with significant unmet medical need, today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug designation for rilonacept for the treatment of pericarditis, which includes recurrent pericarditis. Rilonacept is a weekly, subcutaneously-injected, recombinant fusion protein that blocks interleukin-1 alpha (IL-1α) and interleukin-1 beta (IL-1β) signaling.

"Orphan Drug designation for rilonacept for the treatment of pericarditis, along with the Breakthrough Therapy designation for recurrent pericarditis and the recent highly statistically significant pivotal Phase 3 data, represent important advances toward bringing a potential treatment solution to patients suffering from this debilitating autoinflammatory disease," said Sanj K. Patel, Chief Executive Officer and Chairman of the Board of Kiniksa. "We look forward to submitting an sBLA in recurrent pericarditis to the FDA later this year and are actively preparing for commercialization."

Kiniksa recently reported positive data from RHAPSODY, a pivotal Phase 3 trial of rilonacept in recurrent pericarditis. RHAPSODY met its prespecified primary and all major secondary efficacy endpoints, showing that rilonacept improved clinically-meaningful outcomes associated with the unmet medical need in recurrent pericarditis.

Rilonacept was discovered and developed by Regeneron Pharmaceuticals, Inc. (Regeneron) and is approved by the FDA under the brand name ARCALYST® for the treatment of for Cryopyrin-Associated Periodic Syndromes (CAPS). Kiniksa licensed rilonacept from Regeneron in 2017 for evaluation in diseases believed to be mediated by both IL-1α and IL-1β, including recurrent pericarditis. The FDA granted Breakthrough Therapy designation to rilonacept for recurrent pericarditis in 2019. Based on the Phase 3 RHAPSODY data, the Biologic License Application (BLA) for CAPS will transfer to Kiniksa, and the company plans to submit a supplemental Biologic License Application (sBLA) in recurrent pericarditis to the FDA later this year. Upon receipt of FDA approval for rilonacept in recurrent pericarditis, Kiniksa would assume the sales and distribution of rilonacept for the approved indications in the United States and would evenly split profits on sales with Regeneron.

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 defines rare diseases as 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. If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years from the date of such approval, except in limited circumstances. Additionally, orphan drug designation waives the requirement to conduct pediatric studies for the product in the disease it is designated.

About Recurrent Pericarditis
Recurrent pericarditis is a painful and debilitating autoinflammatory cardiovascular disease that typically presents with chest pain and is often associated with changes in electrical conduction and sometimes buildup of fluid around the heart, called pericardial effusion. Patients with pericarditis are deemed recurrent if they have an additional episode after a symptom-free period of 4-6 weeks and chronic if symptoms from any one episode last longer than three months. Recurrent pericarditis symptoms impair qualify of life, limit physical activities, and lead to frequent emergency department visits and hospitalizations. There are currently no FDA-approved treatments for recurrent pericarditis.

About RHAPSODY
RHAPSODY is the global, randomized withdrawal design, pivotal Phase 3 clinical trial of rilonacept in recurrent pericarditis. Eligible patients presented at screening with at least a third pericarditis episode, defined as at least 1 day with pericarditis pain of ≥ 4 on the 11-point Numerical Rating Scale (NRS) and a C-reactive protein (CRP) value ≥ 1 mg/dL within the 7-day period prior to first study drug administration. Patients could have been receiving concomitant nonsteroidal anti-inflammatory drugs (NSAIDs) and/or colchicine and/or oral corticosteroid treatment in any combination. The study was comprised of 4 periods: a screening period; a single-blind run-in period during which patients received a loading dose of rilonacept 320 mg injected subcutaneously (SC) followed by 160 mg SC weekly while background pericarditis medications were tapered and discontinued; a double-blind, placebo-controlled randomized withdrawal period during which clinical responders to rilonacept were randomized 1:1 and received 160 mg SC weekly rilonacept or placebo; and a long-term extension treatment period with up to 24 months of open-label rilonacept 160 mg SC weekly. The primary efficacy endpoint was time-to-first pericarditis-recurrence in the randomized withdrawal period. The Clinical Endpoint Committee adjudicated all suspected pericarditis recurrences for inclusion in the primary efficacy endpoint analysis. The co-principal investigators are Dr. Allan Klein of Cleveland Clinic and Dr. Massimo Imazio of the University of Torino, Italy. For more information, refer to ClinicalTrials.gov Identifier: NCT03737110.

About Rilonacept
Rilonacept is a weekly, subcutaneously-injected, recombinant fusion protein that blocks IL-1α and IL-1β signaling. Rilonacept was discovered and developed by Regeneron and is approved by the FDA under the brand name ARCALYST® for the treatment of CAPS, specifically Familial Cold Autoinflammatory Syndrome and Muckle-Wells Syndrome. Rilonacept for the treatment of deficiency of the interleukin-1 receptor antagonist (DIRA) is currently pending FDA approval following the submission of an sBLA in June 2020. Rilonacept in recurrent pericarditis is an investigational drug. The

FDA granted Breakthrough Therapy designation to rilonacept for recurrent pericarditis in 2019 and Orphan Drug designation to rilonacept for pericarditis in 2020.

Important information about ARCALYST® (rilonacept) Injection
IL-1 blockade may interfere with immune response to infections. Serious, life-threatening infections have been reported in patients taking ARCALYST. ARCALYST should be discontinued if a patient develops a serious infection. Taking ARCALYST with TNF inhibitors is not recommended because this may increase the risk of serious infections.

Patients should not receive a live vaccine while taking ARCALYST. It is recommended that prior to initiation of therapy with ARCALYST patients receive all recommended vaccinations, as appropriate, including pneumococcal vaccine and inactivated influenza vaccine. In the initial development program for ARCALYST, six serious adverse reactions were reported by four patients: Mycobacterium intracellular infection, gastrointestinal bleeding and colitis, sinusitis and bronchitis and Streptococcus pneumoniae meningitis. The most commonly reported adverse reactions associated with ARCALYST were injection site reaction and upper respiratory tract infection. Patients should be monitored for changes in their lipid profiles and provided with medical treatment if warranted. Treatment with immunosuppressants, including ARCALYST, may result in an increase in risk of malignancies. Hypersensitivity reactions associated with ARCALYST administration in clinical studies have been rare. If a hypersensitivity reaction occurs, administration of ARCALYST should be discontinued and appropriate therapy initiated.

About Kiniksa
Kiniksa is a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need. Kiniksa's clinical-stage product candidates, rilonacept, mavrilimumab, vixarelimab and KPL-404, are based on strong biologic rationale or validated mechanisms, target underserved conditions and offer the potential for differentiation. These pipeline assets are designed to modulate immunological pathways that are implicated across a spectrum of diseases. For more information, please visit www.kiniksa.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. 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 expectation that the rilonacept BLA will be transferred to Kiniksa pursuant to the Regeneron license agreement; our plan to submit an sBLA for rilonacept in recurrent pericarditis to the FDA and the timing thereof; and the potential for rilonacept to be an treatment solution for patients suffering from recurrent pericarditis.

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: our potential inability to replicate in later clinical trials the positive final data from our earlier clinical trials or investigator-initiated protocols or studies; impact of additional data from us or other companies; potential undesirable side effects caused by our product candidates; our potential inability to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities; our reliance on third parties to manufacture our product candidates; drug substance and/or drug product shortages; and our reliance on third parties to conduct research, clinical trials, and/or certain regulatory activities for our product candidates; the potential impact of the COVID-19 pandemic and measures taken in response to the pandemic; changes in our operating plan and funding requirements; existing or new competition; and our ability to attract and retain qualified personnel.

These and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission ("SEC") on May 4, 2020 and our other reports subsequently filed with the SEC 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. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change. 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.

ARCALYST® is a registered trademark of Regeneron Pharmaceuticals, Inc.

Every Second Counts!™

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