Kiniksa Announces U.S. FDA Acceptance of sBLA and Priority Review for Rilonacept in Recurrent Pericarditis

November 23, 2020

- PDUFA goal date of March 21, 2021 -
- Filing based on positive data from RHAPSODY, which achieved its primary and all major secondary endpoints -
- Rilonacept BLA for CAPS transferred to Kiniksa from Regeneron -

HAMILTON, Bermuda, Nov. 23, 2020 (GLOBE NEWSWIRE) -- Kiniksa Pharmaceuticals, Ltd. (Nasdaq: KNSA) ("Kiniksa"), a biopharmaceutical company with a pipeline of assets designed to modulate immunological pathways across a spectrum of diseases, today announced that the U.S. Food and Drug Administration (FDA) accepted the supplemental Biologics License Application (sBLA) for rilonacept in recurrent pericarditis. The FDA granted priority review to the application and assigned a Prescription Drug User Fee Act (PDUFA) goal date of March 21, 2021. Rilonacept is a weekly, subcutaneously-injected, recombinant dimeric fusion protein that blocks interleukin-1 alpha (IL-1α) and interleukin-1 beta (IL-1β) signaling. The FDA granted Breakthrough Therapy designation to rilonacept for the treatment of recurrent pericarditis in 2019 and Orphan Drug designation to rilonacept for the treatment of pericarditis in 2020.

The regulatory submission was based on 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 treatment in the trial improved clinically meaningful outcomes associated with the significant unmet medical need in recurrent pericarditis. RHAPSODY data were recently published in *The New England Journal of Medicine* simultaneously with a late-breaking scientific presentation at the American Heart Association's Scientific Sessions 2020.

"We are thrilled to receive the FDA's acceptance of the sBLA submission for rilonacept in recurrent pericarditis with priority review, said Sanj K. Patel, Chief Executive Officer and Chairman of the Board of Kiniksa. "Rilonacept has the potential to become the first FDA-approved therapy for this painful and debilitating autoinflammatory disease. We are committed to bringing this potential treatment option to patients as soon as possible."

Rilonacept was discovered and developed by Regeneron Pharmaceuticals, Inc. (Regeneron) and is approved by the FDA for the treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) under the brand name ARCALYST®. 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. Based on positive RHAPSODY data, the Biologic License Application (BLA) for CAPS transferred to Kiniksa. If approved by the FDA for recurrent pericarditis, Kiniksa will take responsibility for sales and distribution of rilonacept for all the approved indications in the United States and evenly split profits with Regeneron, as described in the Rilonacept License Agreement.

Kiniksa is obligated to pay regulatory milestones to Regeneron of up to an aggregate of $27.5 million through the time of a potential approval of rilonacept in recurrent pericarditis, of which $7.5 million is expected to be paid in the fourth quarter of 2020.

Kiniksa continues to prepare for the potential commercial launch of rilonacept in recurrent pericarditis. The company has been generating evidence on disease burden, building disease awareness with payers, physicians, and advocacy groups, and establishing core capabilities such as distribution, patient services and data management.

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 be 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. Kiniksa will continue to follow patients in the long-term extension treatment period for up to 24 months. 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 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 the Rilonacept License Agreement with Regeneron

In 2017, Regeneron granted Kiniksa an exclusive license to develop and commercialize rilonacept worldwide, aside from Israel, Egypt, Turkey and select countries in the Middle East and North Africa. In the United States and Japan, Kiniksa's license is initially for all indications other than those involving local administration to the eye or ear, oncology, deficiency of the interleukin1 receptor antagonist (DIRA) and CAPS. If Kiniksa is successful in receiving marketing approval for rilonacept in the United States for a new indication, the scope of the license granted to Kiniksa will automatically expand to include DIRA, if approved, and CAPS in the United States and Japan, and Kiniksa will assume the sales and distribution of rilonacept in these additional indications. Outside the United States and Japan, Kiniksa's license is for all indications other than local application to the eye or ear, oncology, CAPS, DIRA and certain periodic fever syndromes. Kiniksa made an upfront payment of $5.0 million to Regeneron and is obligated to make regulatory milestone payments of up to $27.5 million in the aggregate. Thereafter, Kiniksa and Regeneron will evenly split profits on sales of rilonacept after deducting certain commercialization expenses subject to specified limits.

About Rilonacept
Rilonacept is a weekly, subcutaneously-injected, recombinant dimeric 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 DIRA is currently under FDA review 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 the treatment of recurrent pericarditis in 2019 and Orphan Drug designation to rilonacept for the treatment of 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 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 belief that rilonacept has the potential to become the first FDA-approved therapy for recurrent pericarditis; our urgency in bringing this potential treatment option to patients suffering from recurrent pericarditis; our expectation regarding the timing of payments to Regeneron in connection with regulatory milestones; and our belief that all of our product candidates offer 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: impact of additional data from us or other companies; potential undesirable side effects caused by rilonacept; our potential inability to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities; the potential for applicable regulatory authorities to delay or deny approval of the sBLA for rilonacept in recurrent pericarditis; our potential inability to demonstrate that the clinical data integrity and quality of the biologic manufacturing processes and facilities are sufficient for the FDA to approve rilonacept in recurrent pericarditis; our reliance on third parties to manufacture our product candidates, including our reliance on Regeneron to manufacture the clinical and commercial supply of rilonacept; the potential impact of the COVID-19 pandemic and measures taken in response to the pandemic; and changes in our operating plan and funding requirements.

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 November 5, 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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