# Kiniksa Announces Positive Data from Phase 3 Trial of Rilonacept in Recurrent Pericarditis (RHAPSODY)

June 29, 2020

*- Primary and all major secondary efficacy endpoints were highly statistically significant -*
*- Rilonacept treatment resulted in a 96% reduction in risk of recurrent pericarditis events (primary efficacy endpoint: Hazard Ratio = 0.04, p<0.0001) -*
*- Safety results consistent with FDA-approved label for CAPS -*
*- sBLA submission expected later this year -*
*- Conference call and webcast scheduled for 8:30 a.m. EDT today -*

HAMILTON, Bermuda, June 29, 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, reported positive data from RHAPSODY, a pivotal Phase 3 trial of rilonacept, a weekly, subcutaneously-injected, recombinant fusion protein that blocks interleukin-1 alpha (IL-1α) and interleukin-1 beta (IL-1β) signaling, 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, a painful and debilitating autoinflammatory disease. The U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy designation to rilonacept for the treatment of recurrent pericarditis in 2019, and Kiniksa expects to submit a Supplemental Biologics License Application (sBLA) later this year.

"We are pleased to announce that RHAPSODY, our pivotal Phase 3 trial of rilonacept in recurrent pericarditis, met its primary and all major secondary efficacy endpoints," said Sanj K. Patel, Chief Executive Officer and Chairman of the Board of Kiniksa. "Combined with a well-tolerated safety profile and a weekly dosing regimen, these data are an important step forward for patients. We believe rilonacept has the potential to be the first FDA-approved therapy for recurrent pericarditis. We are committed to submitting an sBLA to the FDA later this year and look forward to bringing this potential treatment option to patients as soon as possible."

RHAPSODY is a global, randomized withdrawal design, pivotal Phase 3 clinical trial of rilonacept in recurrent pericarditis. The trial's primary analysis population included 61 actively symptomatic recurrent pericarditis patients who were failing standard of care treatment, including nonsteroidal anti-inflammatory drugs (NSAIDs), colchicine, or corticosteroids, initiated rilonacept treatment during a run-in period, discontinued background medications, and achieved and maintained clinical response (11-point pain Numerical Rating Scale (NRS) ≤ 2.0 and C-reactive protein (CRP) ≤ 0.5 mg/dL) on rilonacept monotherapy. Clinical responders were randomized 1:1 to receive continued weekly rilonacept (n=30) or placebo (n=31) in a blinded manner in the randomized withdrawal period.

The primary efficacy endpoint of time-to-first adjudicated pericarditis recurrence in the randomized withdrawal period was highly statistically significant.

- Median [95% CI] time to pericarditis recurrence for rilonacept recipients in the randomized withdrawal period could not be estimated due to the low number of recurrences in the rilonacept treatment arm. The median time-to-recurrence for placebo recipients was 8.6 [4.0-11.7] weeks (Hazard Ratio = 0.04, p<0.0001).

- Rilonacept recipients experienced a 96% reduction in risk of recurrent pericarditis events.

All major secondary efficacy endpoints in the randomized withdrawal period were also highly statistically significant.

- 81% of rilonacept recipients maintained clinical response at Week 16 of the randomized withdrawal period, compared to 20% of placebo recipients (p=0.0002). Consistent results were observed at Week 8 and Week 24 and were also highly statistically significant (p<0.0001 and p=0.0022, respectively).

- The proportion of rilonacept recipients with absent or minimal pericarditis symptoms at Week 16 of the randomized withdrawal period was 81% compared to 25% for placebo recipients (p=0.0006). Consistent results were observed at Week 8 and Week 24 and were also highly statistically significant (p<0.0001 and p=0.0002, respectively).

Rilonacept was well-tolerated in the study, with adverse events consistent with the FDA-approved label for the treatment of Cryopyrin-Associated Periodic Syndromes (CAPS). The most common adverse events were injection site reactions.

"The RHAPSODY data provide hope for patients suffering from recurrent pericarditis," said John F. Paolini, MD, PhD, Chief Medical Officer of Kiniksa. "In fact, rilonacept patients experienced no or minimal pericarditis pain for nearly 95% of study days through Week 16 compared to less than half of study days for placebo recipients, which was highly statistically significant. We believe that, by treating and preventing disease recurrence, rilonacept has the potential to be a transformational therapeutic advancement in the treatment of patients with recurrent pericarditis and to become the first FDA-approved therapy for this debilitating autoinflammatory disease."

Additional analyses of the RHAPSODY trial results are ongoing, and Kiniksa plans to present the data at a future medical meeting or in a publication.

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 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 announced today, the Biologic License Application (BLA) for CAPS will transfer to Kiniksa, and the company plans to submit an sBLA with the FDA in recurrent pericarditis 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 will evenly split profits on sales with Regeneron.

**Conference Call Information**
Kiniksa will host a conference call and webcast at 8:30 a.m. Eastern Time on Monday, June 29, 2020 to discuss top-line pivotal Phase 3 data for rilonacept in recurrent pericarditis. Individuals interested in participating in the call should dial (866) 614-0636 (U.S. and Canada) or (409) 231-2053 (international) using conference ID number 2089128. To access the webcast, please visit the Investors and Media section of Kiniksa's website at www.kiniksa.com. The archived webcast will be available on Kiniksa's website for 14 days beginning approximately one hour after the call has completed.

**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 NRS and a CRP value ≥ 1 mg/dL within the 7-day period prior to first study drug administration. Patients could be receiving concomitant 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 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 interleukin-1 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 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 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, which includes Familial Cold Autoinflammatory Syndrome and Muckle-Wells Syndrome. Rilonacept for the treatment of DIRA is currently pending FDA approval following the submission of a supplemental BLA in June 2020. Rilonacept in recurrent pericarditis is an investigational drug. The FDA has granted Breakthrough Therapy designation to rilonacept for recurrent pericarditis.

**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; the potential for rilonacept to be an advancement in treatment of pericarditis; our belief that rilonacept has the ability to become the first FDA-approved therapy for recurrent pericarditis; our expectation of presenting additional data from RHAPSODY; and the urgency in bringing an approved therapy to 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; delays or difficulty in activating sites or enrolling patients in our planned clinical trials; potential complications in coordinating among requirements, regulations and guidelines of regulatory authorities across a number of jurisdictions for our planned global clinical trials; 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.



***Every Second Counts!™***

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