Rilonacept

Phase 3

Therapeutic Area

Recurrent pericarditis (RP) is a painful and debilitating autoinflammatory cardiovascular disease.

Mechanism of Action

Inhibitor of interleukin-1α (IL-1α) and interleukin-1β (IL-1β).

US Prevalence

Health plan claims analysis (cross-validated with published estimates) supports that there are approximately 40,000 patients with RP in the United States seeking and receiving medical treatment for RP.

Breakthrough Therapy designation

Breakthrough Therapy designation granted by the US Food and Drug Administration (FDA) for rilonacept for the treatment of RP.

Orphan Drug designation

Orphan Drug designation granted by the FDA for rilonacept for the treatment of pericarditis.

KINIKSA

Rationale

We reported data from RHAPSODY, a global, randomized withdrawal (RW) design, pivotal Phase 3 clinical trial of rilonacept in recurrent pericarditis in June 2020. RHAPSODY met its prespecified primary and all major secondary efficacy endpoints.

Status

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 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 Biologic License Application (BLA) for CAPS transferred to Kiniksa, and the FDA accepted the supplemental Biologic License Application (sBLA) for 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. 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.

Unmet Need

We are not aware of any current therapies approved by the FDA for the treatment of RP.

- We believe RP presents a significant clinical and humanistic burden and imposes a significant financial burden on the healthcare system.
- A patient's initial acute episode of pericarditis is typically treated with over-the-counter or prescription nonsteroidal anti-inflammatory drugs (NSAIDs) or colchicine, both of which are used off-label. Recurrent episodes are treated in a similar manner or by adding systemic corticosteroids, which are also used off-label.
- We believe the greatest demand in the market is for a product that not only treats the symptoms of a pericarditis recurrence but also reduces the risk of future recurrences while on treatment.
- Within the estimated diagnosed and treated RP population of 40,000, we believe that there are initially 14,000 patients who may benefit from rilonacept — ~8,000 refractory, which includes those not suitable for steroids, ~1,000 steroid dependent, and ~5,000 multiple relapsing.
- Refractory and steroid-dependent patients have the highest recurrence burden, with 30% to 40% of patients experiencing greater than 2 episodes per year.
    - They also have a higher incidence of severe complications, such as cardiac tamponade and constrictive pericarditis, often use opioids to manage their pain, and deal with anxiety and depression due to the unpredictability and severity of recurrences.