

## **FDA News**

**FOR IMMEDIATE RELEASE**

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### **FDA Approves New Orphan Drug for Treatment of Rare Inflammatory Syndromes**

***Arcalyst is first treatment for extremely rare condition called Cryopyrin-Associated Periodic Syndrome or CAPS***

The U.S. Food and Drug Administration today approved a drug to help ease the suffering faced by those with certain chronic inflammatory diseases. Arcalyst (rilonacept, an Interleukin-1 blocker) is now approved for the long term treatment of two Cryopyrin-Associated Periodic Syndromes (CAPS) disorders: Familial Cold Auto-Inflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS).

Symptoms of both of these disorders include inflammation such as joint pain, rash or skin lesions, fever and chills, eye redness or pain, and fatigue in both children and adults; however MWS is associated with more severe inflammation and may include hearing loss or deafness. In addition, some MWS patients may also be affected by the buildup of a protein substance that damages organs and tissue (amyloidosis). The FCAS and MWS disorders affect about 300 people in the United States. CAPS disorders are inherited. Fifty percent of CAPS cases are associated with a gene mutation in the CIAS 1 gene.

"Arcalyst offers new promise for this small patient population suffering disorders associated with Cryopyrin-Associated Periodic Syndromes," said Curt Rosebraugh, M.D., M.P.H., acting director of the FDA's Office of Drug Evaluation II. "The Orphan Drug Act—now in its 25th year—has been tremendously successful in delivering effective treatments to patients with extremely rare, but serious, diseases."

Arcalyst blocks interleukin-1 which is a signaling protein secreted by certain immune-related cells in the body. Interleukin-1 acts as a messenger to regulate inflammatory responses, but in excess it can be harmful and has been shown to be key in the inflammation seen in CAPS sufferers with FCAS or MWS.

The FDA based its approval on a clinical study conducted by the manufacturer, which demonstrated the drug's safety and effectiveness. Using a daily diary questionnaire, 47 patients rated the following five signs and symptoms of CAPS: joint pain, rash, feeling of fever/chills, eye redness/pain, and fatigue, each on a scale of zero (none/no severity) to 10 (very severe). Patients noted initial onset of relief of symptoms in their diaries within several days.

The most commonly reported side effects associated with use of Arcalyst were injection-site reactions and upper respiratory infections.

The FDA granted the drug a priority review, which speeds the review process for patients who have unmet medical needs.

Arcalyst is manufactured by Regeneron Pharmaceuticals Inc., Tarrytown, N.Y.

**For more information** on the Orphan Drug Act, visit: [www.fda.gov/orphan/](http://www.fda.gov/orphan/).