

Adakveo® (crizanlizumab) – New orphan drug approval

- On November 15, 2019, the <u>FDA announced</u> the approval of <u>Novartis</u> <u>Adakveo (crizanlizumab)</u>, to reduce the frequency of vasoocclusive crises (VOCs) in adults and pediatric patients aged 16 years and older with sickle cell disease (SCD).
- SCD is an inherited blood disorder in which the red blood cells are abnormally shaped (in a crescent
 or "sickle" shape), which restricts the flow in blood vessels and limits oxygen delivery to the body's
 tissues, leading to severe pain (VOCs) and organ damage.
 - According to the Centers for Disease Control and Prevention, SCD affects approximately 100,000 individuals in the U.S. The disease occurs most often in African-Americans, where the incidence is 1 out of every 365 babies born.
- Adakveo is a first-in-class monoclonal antibody that binds to P-selectin a cell adhesion protein that
 plays a central role in the multicellular interactions that can lead to vaso-occlusion.
- The efficacy of Adakveo was established in SUSTAIN, a randomized, placebo-controlled, doubleblind study in 198 patients with SCD. Patients were randomized Adakveo 5 mg/kg, Adakveo 2.5 mg/kg, or placebo. The primary endpoint was the annual rate of VOCs leading to a healthcare visit.
 - Patients who received Adakveo 5 mg/kg had a lower median annual rate of VOC vs. patients who received placebo (1.63 vs. 2.98; p = 0.010).
 - Thirty-six percent (36%) of patients treated with Adakveo 5 mg/kg did not experience a VOC vs. 17% of placebo-treated patients. The median time to first VOC from randomization was 4.1 months in the Adakveo 5mg/kg arm vs. 1.4 months in the placebo arm.
- Warnings and precautions for Adakveo include infusion-related reactions and laboratory test interference.
- The most common adverse reactions (> 10%) with Adakveo use were nausea, arthralgia, back pain, and pyrexia.
- The recommended dose of Adakveo is 5 mg/kg administered via intravenous infusion over a period of 30 minutes at week 0, week 2, and every 4 weeks thereafter.
 - Adakveo should be prepared and administered by a healthcare professional.
- The estimated list price for Adakveo is \$85,000 to \$113,000 per year, depending on dosing.
- Novartis plans to launch Adakveo in the coming weeks. Adakveo will be available as a 100 mg/10 mL (10 mg/mL) solution in single-dose vials.



OptumRx® specializes in the delivery, clinical management and affordability of prescription medications and consumer health products. We are an Optum® company — a leading provider of integrated health services. Learn more at **optum.com**.

All Optum® trademarks and logos are owned by Optum, Inc. All other brand or product names are trademarks or registered marks of their respective owners.

This document contains information that is considered proprietary to OptumRx and should not be reproduced without the express written consent of OptumRx.

RxNews® is published by the OptumRx Clinical Services Department.

©2019 Optum, Inc. All rights reserved.