

## Adakveo<sup>®</sup> (crizanlizumab) – New orphan drug approval

- On November 15, 2019, the [FDA announced](#) the approval of [Novartis' Adakveo \(crizanlizumab\)](#), 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, double-blind 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.