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SEG101 therapy by Novartis gets FDA approval

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Treatments that make blood cells and blood vessels less sticky may help reduce the number of days patients experience VOCs

Novartis has announced that the US Food and Drug Administration has granted crizanlizumab (SEG101) Breakthrough Therapy designation for the prevention of vaso-occlusive crises (VOCs) in patients of all genotypes with sickle cell disease (SCD).

Also known as sickle cell pain crises, VOCs are unpredictable and extremely painful events that can lead to serious acute and chronic complications2. VOCs happen when multiple blood cells stick to each other and to blood vessels, causing blockages.

Treatments that make blood cells and blood vessels less sticky may help reduce the number of days patients experience VOCs.

Samit Hirawat, Head, Novartis Oncology Global Drug Development said, "Painful sickle cell crises matter because they can disrupt patients' lives, and often require hospital visits and medical attention. We look forward to working closely with the FDA over the coming months toward making crizanlizumab, a therapy that has the potential to prevent sickle cell pain crises, available in the US as soon as possible."

According to FDA guidelines, treatments that receive Breakthrough Therapy designation are those that treat a serious or life-threatening disease or condition and demonstrate a substantial improvement over existing therapies on one or more significant end points based on preliminary clinical evidence.

The FDA granted Breakthrough Therapy designation for crizanlizumab based on positive results of the Phase II SUSTAIN trial, which compared the P-selectin inhibitor crizanlizumab with placebo in patients with sickle cell disease.

SUSTAIN showed that crizanlizumab reduced the median annual rate of VOCs leading to health care visits by 45.3% compared to placebo (1.63 vs 2.98, P=0.010) in patients with or without hydroxyurea therapy. The study also demonstrated that crizanlizumab significantly increased the percentage of patients who did not experience any VOCs vs placebo (35.8% vs

16.9%, P=0.010) during treatment.

Patients taking crizanlizumab (5 mg/kg) experienced a similar incidence of treatment-emergent adverse events (AEs) (86.4% vs 88.7%) and serious AEs (25.8% vs 27.4%) compared to placebo, and a low incidence of discontinuations (3%) due to adverse events.

Adverse events that occurred in 10% or more of the patients in either active-treatment group (2.5 mg/kg; 5 mg/kg) and at a frequency that was at least twice as high as that in the placebo group included arthralgia, diarrhea, pruritus, vomiting, and chest pain. There were no apparent increases in infections with crizanlizumab treatment.