

FDA approves first oral drug for spinal muscular atrophy

August 11 2020



(HealthDay)—The U.S. Food and Drug Administration has approved the

first oral, at-home drug for spinal muscular atrophy (SMA), the agency announced Friday.

Evrysdi (risdiplam) was approved to treat [patients](#) ages 2 months and older with SMA and is the second [drug](#) approved for the disease. The drug is administered daily at home by mouth or feeding tube.

Approval was based on two [clinical studies](#), including FIREFISH, an open-label study of 21 patients aged 6.7 months on average at study initiation. At one year, 41 percent of patients met the efficacy end point of sitting independently for more than five seconds, and at nearly two years, 81 percent of patients were alive without permanent ventilation, which the FDA notes is a "noticeable improvement from typical disease development without treatment."

In the second study, SUNFISH, a randomized, placebo-controlled study of 180 patients ages 2 to 25 years, the Motor Function Measure-32 [total score](#) increased an average of 1.36 from baseline to one year for patients randomly assigned to Evrysdi compared with a decrease of 0.19 for patients randomly assigned to placebo.

The most commonly reported side effects of Evrysdi were fever, diarrhea, rash, ulcers in the mouth area, arthralgia, and urinary tract infections. Patients with infantile-onset SMA had additional side effects of upper respiratory tract infection, pneumonia, constipation, and vomiting. The FDA warns that patients taking Evrysdi should avoid taking it with drugs that are multidrug and toxin extrusion substrates because Evrysdi may increase plasma concentrations.

Approval was granted to Genentech.

More information: [More Information](#)

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Citation: FDA approves first oral drug for spinal muscular atrophy (2020, August 11) retrieved 27 April 2024 from <https://medicalxpress.com/news/2020-08-fda-oral-drug-spinal-muscular.html>

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