

FDA OKs first treatment for rare genetic disorder

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The Food and Drug Administration on Friday approved the first treatment for children and adults with spinal muscular atrophy, a rare genetic disorder marked by progressive muscle weakness that's the most common genetic cause of death in infants.

The agency that it approved the drug, Spinraza, after granting it fast-track status. It was developed by Ionis Pharmaceuticals Inc. of Carlsbad, California, and Biogen Inc. of Cambridge, Massachusetts. Biogen will market it.

The organization Cure SMA says [spinal muscular atrophy](#) affects about 1 in 10,000 babies, and about 1 in every 50 Americans is a genetic carrier. The FDA noted the disorder, which affects the motor nerve cells in the [spinal cord](#), can impact people of any age. Its symptoms and rate of progression also vary.

Spinraza is injected into the fluid surrounding the spinal cord. Biogen said that in a clinical study, the drug brought about "meaningful improvement in motor function compared to untreated study participants."

With the FDA approval, Ionis will receive a \$60 million milestone payment. It's also in line for royalties on Spinraza sales. Its shares rose \$3.69, or 7 percent, to \$57.10 in after-market trading following the announcement of the drug's approval.

Biogen rose \$9.47, or 3.3 percent, to \$297.

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