

## World's most expensive drug: £1.79 million one-off Zolgensma treatment approved by British National Health Service to treat spinal muscular dystrophy in children

Zolgensma, which costs £1.79m per dose, halts the progression of [spinal muscular atrophy](#) (SMA), which involves loss of movement, muscle weakness and paralysis, and is the leading genetic cause of death in infants.

Up to 80 infants a year in England are expected to benefit from the drug, which is administered as a single dose and replaces the function of the faulty SMN1 gene. The active ingredient, onasemnogene abeparvovec, restores the gene and produces proteins essential for muscle movement and nerve function.

Babies born with the most severe form of the muscle-wasting disease – SMA type 1 – have a life expectancy of two years. Zolgensma does not cure the disease but helps halt its progression, meaning babies breathe without a ventilator, sit up, crawl and even walk, with benefits lasting more than five years after treatment.

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“This deal is a life-changer for youngsters with this cruel disease and for their families,” said Sir Simon Stevens, the chief executive of [NHS](#) England. “SMA is the leading genetic cause of death among babies and young children, which is why [NHS](#) England has moved mountains to make this treatment available, while successfully negotiating hard behind the scenes to ensure a price that is fair to taxpayers.”

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