Nice says no to new drug for dying children

A charity has condemned a decision by health officials to reject a drug to treat a rare genetic disorder which can mean that affected children do not live to their second birthday.

In new draft guidance, the National Institute for Health and Care Excellence (Nice) has not recommended Spinraza made by Biogen, a treatment for people with spinal muscular atrophy (SMA), for use across the NHS.

The condition causes loss of movement and difficulty breathing and swallowing — infants with severe SMA usually die before the age of two.

Muscular Dystrophy UK said the decision would cause heartbreak for families and that, without access to the drug, children with SMA, would lose the ability to crawl, walk and swallow.

Nice said there were uncertainties over its long-term benefits and that it was highly costly, but it would welcome discussions about access to Spinraza, also known as nusinersen.

Muscular Dystrophy UK said: "We call on Nice, NHS England and the drug company, Biogen, to urgently come together and find a solution to provision and pricing so families can get Spinraza before more lives are lost."

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