NHS fears new drug for children could cost £60m

Chris Smyth Health Editor

A £200,000-a-year drug that can "transform childhoods" has been approved for NHS use, but patients must wait for a battle over costs.

Children with Duchenne muscular dystrophy have no other treatment to slow the progression of the disease and have pleaded to get the drug while they are still able to walk.

The National Institute for Health and Care Excellence ruled yesterday that patients should receive Translarna, but NHS England has demanded a further discount before funding the drug, which could cost the health service £60 million a year.

Health chiefs are increasingly concerned about the rising treatment costs for rare conditions at a time when the NHS is struggling to keep to its budget. Last year it approved a £340,000-a-year treatment for people with a rare kidney disorder — a decision that could cost £10 million per patient.

About 2,500 people in Britain, almost all male, have Duchenne muscular dystrophy, a genetic condition in which the body produces little dystrophin, a substance crucial for muscle functioning. Patients grow weaker and more disabled, often dying before they reach 30.

Translarna is the first drug shown to slow the progression of the disease and could benefit 10-15 per cent of sufferers. A study suggested that it would keep patients walking for an extra seven years.

After months of discussions, Nice finally ruled that patients should get the drug, also known as ataluren, subject to conditions including agreement on a discount on its £220,256 list price.

A spokesman for NHS England said: "It is now for the manufacturer to agree to terms which allow us to make ataluren available without a disproportionate impact on our ability to fund new treatments for other patients."

Robert Meadowcroft, chief executive of Muscular Dystrophy UK, greeted the decision as a "chance to transform childhoods". He said: "Parents of children eligible for Translarna have fought courageously for this outcome, and to give their children the chance to keep walking for longer. This announcement comes as wonderful news and a true victory for the families."

Patients are only eligible for the drug if they are over the age of five and still able to walk, and Mr Meadowcroft urged NHS England to reach a deal to avoid further delays which could mean children missing out on the drug. "Having waited 18 months for a decision, this is a delay boys and their families can ill afford," he said.

Stuart Peltz, chief executive of PTC Therapeutics, which makes the drug, said he was "extremely pleased" by the Nice ruling and looked forward to working with NHS England to finalise a deal.

The Nice decision will initially fund treatment for the 50 children currently eligible, who will be reviewed after five years to decide if the drug has made enough difference. Charities hope that more patients will gradually become eligible.

Sir Andrew Dillon, chief executive of Nice, said the drug would benefit people suffering from a "cruel disease" but acknowledged that it "represents a significant cost to the NHS at a time of increased pressure on funding and [Nice] has considered this carefully against the uncertainties of its potential long-term benefits".

Louisa Hill, whose 10-year-old son Archie met David Cameron last year to appeal for the drug, said: "This is amazing, life-changing news. Duchenne muscular dystrophy brings so many challenges into his life, and this will become even greater in the future. Yet now, for the first time, there is something in our corner. We can have real hope that when, one day, there may be a drug to not only slow down the condition, but completely stop it, our son will be strong enough to benefit."