The NHS is to introduce a revolutionary new treatment to tackle the leading genetic cause of death among babies and young children.

About 1,500 patients in England with certain types of spinal muscular atrophy (SMA) are expected to benefit from risdiplam, after a recommendation from the health watchdog. The drug, also called Evrysdi and made by Roche, is a syrup that can be taken at home and is the first non-injectable treatment for the condition.

SMA is a progressive neuromuscular condition affecting the nerves in the spinal cord controlling movement and can cause paralysis, muscle weakness and progressive loss of mobility.

The NHS England chief executive, Amanda Pritchard, said: “In the last three years the NHS has revolutionised care for people with SMA, by securing access to a trio of innovative treatments – Spinraza, Zolgensma and now risdiplam – where three years ago clinicians had no effective medicines at all.

“Spinal muscular atrophy is a cruel disease and the leading genetic cause of death among babies and young children, which is why NHS England has been determined to make these treatments available to people as soon as possible to help transform the lives of patients and their families.”

Risdiplam has initially been recommended for limited use for people with certain types of SMA as part of a deal known as a managed access agreement between NHS England and Roche, the National Institute for Health and Care Excellence (Nice) said. Babies as young as two months old will be among the first to benefit.

The list price of risdiplam is £7,900 per 80ml vial but a cut-price deal has been struck to make it available to the NHS.

Dr Elizabeth Wraige, a consultant paediatric neurologist at Evelina London children’s hospital, part of Guy’s and St Thomas’ NHS foundation trust, said the development was “excellent news”.

“This will be especially important for those with SMA who cannot receive either of the two existing treatments, Spinraza and Zolgensma. These are very exciting times and I am sure this news will be welcomed by those families and individuals affected by SMA as well as by their clinicians.”

Meindert Boysen, the deputy chief executive of Nice, said the watchdog was pleased to recommend a “convenient oral treatment for people with SMA that can be administered at home”.

He said: “This will not only be less burdensome, and therefore have a positive impact on the lives of both people with SMA and their caregivers, but it will also reduce the treatment administration requirements for the NHS.

“In practical terms, the availability of an oral drug should lead to greater adherence to treatment,

along with giving access to a treatment to those who aren't able to have other currently recommended options."

The mother of a nine-year-old boy from London who was the first patient from the UK to receive risdiplam through a clinical trial said the change she had seen in him was "absolutely amazing".

Melvil Vedrenne-Cloquet, who started taking the drug when he was five, had shown "really positive results" and could look forward to "a brighter future", his mother, Eve Chirdkiatisak, said.

She added: "It's fantastic for the other children and their families out there that it will now be available on the NHS, because when it comes to a diagnosis of SMA, time is of the essence. Children will never get what they have lost back, so the faster they can get the right medicines the better."

Source: **Oral drug for spinal muscular atrophy to be available on NHS in England**