NHS England News Release: 15th May 2019

NHS England to fund first ever treatment for children with rare muscle-wasting condition

NHS England will provide a promising new treatment which can prolong the lives of children with a rare genetic condition after negotiating a deal with the manufacturer.

Nusinersen, also called Spinraza and made by Biogen, is the first treatment that targets the underlying cause of spinal muscular atrophy (SMA).

The condition affects the nerves in the spinal cord, making muscles weaker and causing problems with movement, breathing and swallowing.

Where it develops in babies and toddlers, it can significantly reduce life expectancy.

Between 600 and 1,200 children and adults are currently living with the condition in England and Wales.

While not a cure, trials have shown that nusinersen can slow the effects of SMA in some cases, allowing babies and toddlers to develop stronger muscles and survive for longer without breathing support.

Working with NICE, NHS England has now successfully negotiated a deal with Biogen, meaning that this treatment will be available for those whose clinicians think they would benefit.

Simon Stevens, NHS England chief executive, said: "This promising treatment has the potential to be life changing for children and their families.

"The NHS has now reached one of the most comprehensive deals in the world, which allows us to assess real-world evidence of its long term benefits.

"This latest deal coming on the heels of a number of other recent successful negotiations demonstrates that there is no reason for other companies not to show equivalent flexibility in order to benefit NHS patients, taxpayers and indeed themselves."

NICE has previously not been able to recommend nusinersen for routine use because of uncertainties over its long-term effectiveness and its high cost.

The agreement that has been reached between NHS England and Biogen is known as a Managed Access Agreement, meaning that the NHS will fund treatment for a time-limited period, allowing further data to be collected on its effectiveness.

This paves the way for NICE to formally approve the treatment for use with eligible patients through publishing final guidance.

The treatment will be made available to the youngest and most severely-affected (SMA type 1) patients immediately by Biogen, with NHS England offering funding on NICE's publication of final guidance.

For older babies, children and young adults with less severe symptoms (SMA types 2 and 3), the NHS will begin to provide nusinursen shortly after NICE's guidance is published, once the services to deliver them are established. This is in line with the approach for making other brand new treatments available in a fair way for patients across the country, and is not expected to take more than a few weeks.

This is one of the most comprehensive deals in the world, meaning all relevant SMA patients (SMA 1,2,3a and 3b), including adults and siblings who are yet to show symptoms, will be able to benefit from this treatment.

Meindert Boysen, director of the Centre for Health Technology Evaluation at NICE, said: "We are very pleased that we can now recommend Spinraza for people with SMA.

"The committee has recognised that Spinraza is a promising treatment that has been shown to improve a range of outcomes important to patients. But it also recognised that there are significant uncertainties, particularly around its long-term benefits.

"All along we have felt it important to give all parties every opportunity to try to find a way to mitigate these uncertainties in order to make Spinraza available to patients in England.

"Today's announcement shows that, where companies show appropriate flexibility, it is possible to find a way to provide important treatments to patients in a way that is cost effective for the NHS and taxpayers."

Investment in world-class, cutting edge technologies and treatments is at the heart of the NHS Long Term Plan. The agreement is the latest in a string of 'smart deals' struck in recent months to drive value for the taxpayer and benefits for patients.

These include:

- a deal to make ocrelizumab, an innovative multiple sclerosis drug available;
- a ground-breaking deal which could eliminate Hepatitis C;
- a £300 million saving after negotiating deals with five manufacturers on low cost versions of the health service's most costly drug, adalimumab;
- striking the first full access deal in Europe for CAR-T therapy via the Cancer Drugs Fund, which can potentially cure some children and adults with blood cancers where other treatments have failed, and;
- reaching a deal to make the life-extending lung cancer drug pembrolizumab available for routine use on the NHS.

There are several types of SMA, which start at different ages, some more serious problems than others.

In most cases, a child can only be born with SMA if both of their parents have a faulty gene that causes the condition. People with the most severe forms of SMA usually die before the age of 2.

Nusinersen is the first treatment to address the cause of motor neurone degeneration in SMA – it is injected directly into the spine and is a lifelong treatment.