



Resource impact statement

Resource impact

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NICE has recommended risdiplam as an option for treating 5q spinal muscular atrophy (SMA) in people of all ages, with a clinical diagnosis of SMA types 1, 2 or 3 or with pre-symptomatic SMA and 1 to 4 SMN2 copies, only if the conditions of the [managed access agreement](#) are followed.

Risdiplam will be available to the NHS in line with the managed access agreement with NHS England. As part of this, NHS England and Roche have a commercial access agreement that makes risdiplam available to the NHS at a reduced cost. The financial terms of the agreement are commercial in confidence. The managed access agreement includes the collection of more data to address uncertainties in the evidence.

It is estimated around 1,500 children and adults in the UK are currently living with types 1, 2 and 3 SMA or pre-symptomatic SMA and 1 to 4 SMN2 copies SMA. The NICE guidance does not restrict use from the licence, therefore all patients covered by the marketing authorisation for risdiplam are eligible for treatment.

Risdiplam is an oral treatment which provides an alternative for people who cannot have other treatments for SMA and also means they can have the treatment at home.

When NICE recommends a treatment as an option for use within a managed access agreement, NHS England will make it available according to the conditions in the managed access agreement. This means that, if a person has types 1, 2 or 3 SMA or pre-symptomatic SMA with 1 to 4 SMN2 copies, and the doctor responsible for their care thinks that risdiplam is the right treatment, it should be available for use, in line with NICE's recommendations and the criteria in the managed access agreement.

The guidance will be reviewed when the primary analysis from FIREFISH and SUNFISH have both completed. The review is anticipated to begin in 2024. After this, NICE will decide whether or not to recommend it for use in the NHS and update the guidance.

This technology is commissioned by NHS England. Providers are NHS hospital trusts.