# NHS should fund GSK's £526,000 "bubble baby" drug – NICE

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Richard Staines January 3, 2018


**NICE has recommended that the NHS should fund GlaxoSmithKline's Strimvelis gene therapy for the extremely rare immune deficiency condition known as 'bubble baby syndrome'.**

Costing €594,000 (£526,000) Strimvelis is the most expensive therapy cleared by NICE, but the drug is administered in a single curative dose for adenosine deaminase deficiency (ADA-SCID).

The condition only affects around three babies a year in England, leaving them without a properly functioning immune system.

If left untreated, infants die before school age and their quality of life is affected by development delay, chronic diarrhoea, failure to thrive and recurrent infections.

Patients who are not receiving treatment need to be kept in isolation to avoid infections, hence the colloquial term for the condition.

Strimvelis has to administered at a hospital in Milan, so patients will travel to Italy to have the treatment as part of the care provided by the NHS. Costs incurred to the NHS from the healthcare provider were commercially confidential.

Although ADA-SCID can also be treated with stem cell transplant, NICE said that Strimvelis seems to improve survival.

There were no deaths reported in trials in those with ADA-SCID treated with Strimvelis, although small patient numbers made results uncertain.

In comparison, stem cell transplants' overall survival was 67% (10/15) from a matched unrelated donor, and 71% for patients who had a stem cell transplant from a matched related or unrelated donor.

Assessed using NICE's Highly Specialised Technologies programme, the cost-effectiveness body said that, despite its high cost, the survival and quality of life benefits from Strimvelis mean it costs less than £100,000 per Quality Adjusted Life Year gained when compared with stem cell therapy.

This is in line with NICE's upper threshold for treatments for very rare diseases assessed through the scheme.

Professor Carole Longson, director of the centre for health technology at NICE, said: "Strimvelis represents an important development in the treatment of ADA-SCID, offering the potential to cure the immune aspects of the condition and avoid some of disadvantages of current treatments.

"This means that children born with ADA-SCID will now have a better chance of being able to lead as near normal a life as possible, going to school, mixing with friends, free from the constant threat of getting a potentially life-threatening infection.

"Our evaluation of Strimvelis is the first time that NICE has applied the higher cost effectiveness limits introduced last year for the assessment of treatments for these extremely rare conditions."