

# Treosulfan with fludarabine before allogeneic stem cell transplant for people aged 1 month to 17 years with non-malignant diseases (terminated appraisal)

Technology appraisal guidance

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[www.nice.org.uk/guidance/ta945](https://www.nice.org.uk/guidance/ta945)

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## Advice

NICE is unable to make a recommendation about the use in the NHS of treosulfan with fludarabine before allogeneic stem cell transplant for babies, children and young people aged 1 month to 17 years with non-malignant diseases. This is because Medac Pharma has confirmed that it does not intend to make an evidence submission for the appraisal. Medac Pharma considers that because of the low price and small patient population, it cannot find an economically viable route to submission.

## Information

If NHS organisations wish to consider treosulfan with fludarabine for this indication, they should follow the advice on rational local decision making in the [NHS Constitution for England](#) and the [NHS Commissioning Board and Clinical Commissioning Groups \(Responsibilities and Standing Rules\) Regulations 2012](#). This outlines the approach that should be taken when there is no NICE guidance.

NICE will review the position if the company decides that it wants to make an evidence submission.

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