NICE National Institute for Health and Care Excellence

# Single Technology Appraisal

# Treosulfan with fludarabine for malignant disease before allogeneic stem cell transplant [ID1508]

# Committee Papers

NICE National Institute for Health and Care Excellence

**NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE**

**SINGLE TECHNOLOGY APPRAISAL**

# Treosulfan with fludarabine for malignant disease before allogeneic stem cell transplant [ID1508]

**Contents:**

The following documents are made available to consultees and commentators:

1. **Response to consultee, commentator and public comments on the Appraisal Consultation Document (ACD)**

2. **Comments on the Appraisal Consultation Document from Medac GmbH**

3. **Comments on the Appraisal Consultation Document received through the NICE website**

*Any information supplied to NICE which has been marked as confidential, has been redacted. All personal information has also been redacted.*

# Treosulfan with fludarabine for malignant disease before allogeneic stem cell transplant

## Single Technology Appraisal

## Response to consultee, commentator and public comments on the Appraisal Consultation Document (ACD)

**Type of stakeholder:**

**Consultees –** Organisations that accept an invitation to participate in the appraisal including the companies, national professional organisations, national patient organisations, the Department of Health and Social Care and the Welsh Government and relevant NHS organisations in England. Consultees can make a submission and participate in the consultation on the appraisal consultation document (ACD; if produced). All non-company consultees can nominate clinical experts and/or patient experts to verbally present their personal views to the Appraisal Committee. Company consultees can also nominate clinical experts. Representatives from NHS England and clinical commissioning groups invited to participate in the appraisal may also attend the Appraisal Committee as NHS commissioning experts. All consultees have the opportunity to consider an appeal against the final recommendations, or report any factual errors, within the final appraisal document (FAD).

**Clinical and patient experts and NHS commissioning experts** – The Chair of the Appraisal Committee and the NICE project team select clinical experts and patient experts from nominations by consultees and commentators. They attend the Appraisal Committee meeting as individuals to answer questions to help clarify issues about the submitted evidence and to provide their views and experiences of the technology and/or condition. Before they attend the meeting, all experts must either submit a written statement (using a template) or indicate they agree with the submission made by their nominating organisation..

**Commentators –** Commentators can participate in the consultation on the ACD (if produced), but NICE does not ask them to make any submission for the appraisal. Non-company commentator organisations can nominate clinical experts and patient experts to verbally present their personal views to the Appraisal Committee. Commentator organisations representing relevant comparator technology companies can also nominate clinical experts. These organisations receive the FAD and have opportunity to report any factual errors. These organisations include comparator technology companies, Healthcare Improvement Scotland any relevant National Collaborating Centre (a group commissioned by NICE to develop clinical guidelines), other related research groups where appropriate (for example, the Medical Research Council and National Cancer Research Institute); other groups such as the NHS Confederation, the NHS Commercial Medicines Unit, the Scottish Medicines Consortium, the Medicines and Healthcare Products Regulatory Agency, the Department of Health and Social Care, Social Services and Public Safety for Northern Ireland).

**Public –** Members of the public have the opportunity to comment on the ACD when it is posted on the Institute's web site 5 days after it is sent to consultees and commentators. These comments are usually presented to the appraisal committee in full, but NICE reserves the right to summarise and edit comments received during consultations, or not to publish them at all, where in the reasonable opinion of NICE, the comments are voluminous, publication would be unlawful or publication would be otherwise inappropriate.

**Please note:** Comments received in the course of consultations carried out by NICE are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the submissions that NICE has received, and are not endorsed by NICE, its officers or advisory committees.

| Comment number | Type of stakeholder | Organisation name | Stakeholder comment<br>Please insert each new comment in a new row | NICE Response<br>Please respond to each comment |
|---|---|---|---|---|
| 1 | Consultee-Company | Medac | medac GmbH (MAH for Trecondi/treosulfan) confirms that they have no plans to submit any further evidence or data for this appraisal. | Comment noted. |
| 1 | Public – Web comment | NHS professional | *Has all of the relevant evidence been taken into account?*<br>Not considered because I'm not familiar with the evidence for this medicine | Comment noted. |
| 2 | Public – Web comment | NHS professional | *Are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?*<br>The summaries appear to be reasonable interpretations. | Comment noted. |
| 3 | Public – Web comment | NHS professional | *Are the recommendations sound and a suitable basis for guidance to the NHS?*<br>The recommendation in this TA refers to 'people', but should it just refer to adults? I found it confusing because people generally means both adults and children, but the text below the recommendation states that insufficient evidence is available for children (or people who could tolerate a high intensity regimen, who are not included in the recommendation). Please could this be clarified? | Thank you for your comment. The company did not present enough evidence on the use of treosulfan in children so the committee could not make a recommendation about children. Most people who receive a reduced intensity regimen are adults. It was not considered necessary to amend the guidance. |
| 4 | Public – Web comment | NHS professional | *Are there any aspects of the recommendations that need particular consideration to ensure we avoid unlawful discrimination against any group of people on the grounds of race, gender, disability, religion or belief, sexual orientation, age, gender reassignment, pregnancy and maternity?*<br>Not that I am aware of. | Comment noted. |

NICE National Institute for Health and Care Excellence

**Treosulfan with fludarabine for malignant disease before allogeneic stem cell transplant [ID1508]**

**Consultation on the appraisal consultation document – deadline for comments** 5pm on Tuesday 4 February 2020 **email:** NICE DOCS

| | |
|---|---|
| | Please read the checklist for submitting comments at the end of this form. We cannot accept forms that are not filled in correctly.<br><br>The Appraisal Committee is interested in receiving comments on the following:<br>• has all of the relevant evidence been taken into account?<br>• are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?<br>• are the provisional recommendations sound and a suitable basis for guidance to the NHS?<br><br>NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the preliminary recommendations may need changing in order to meet these aims. In particular, please tell us if the preliminary recommendations:<br>• could have a different impact on people protected by the equality legislation than on the wider population, for example by making it more difficult in practice for a specific group to access the technology;<br>• could have any adverse impact on people with a particular disability or disabilities.<br><br>Please provide any relevant information or data you have regarding such impacts and how they could be avoided or reduced**.** |
| **Organisation name – Stakeholder or respondent** (if you are responding as an individual rather than a registered stakeholder please leave blank): | **medac GmbH** |
| **Disclosure** Please disclose any past or current, direct or indirect links to, or funding from, the tobacco industry. | **n/a** |
| **Name of commentator person completing form:** | [redacted] |

| Comment number | Comments<br><br>Insert each comment in a new row. |
|---|---|

Please return to: **NICE DOCS**

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**Treosulfan with fludarabine for malignant disease before allogeneic stem cell transplant [ID1508]**

**Consultation on the appraisal consultation document – deadline for comments** 5pm on Tuesday 4 February 2020 **email:** NICE DOCS

| | Do not paste other tables into this table, because your comments could get lost – type directly into this table. |
|---|---|
| Example 1 | We are concerned that this recommendation may imply that …….. |
| 1 | medac GmbH (MAH for Trecondi/treosulfan) confirms that they have no plans to submit any further evidence or data for this appraisal. |
| 2 | |
| 3 | |
| 4 | |
| 5 | |
| 6 | |

Insert extra rows as needed

**Checklist for submitting comments**

- Use this comment form and submit it as a Word document (not a PDF).
- Complete the disclosure about links with, or funding from, the tobacco industry.
- Combine all comments from your organisation into 1 response. We cannot accept more than 1 set of comments from each organisation.
- Do not paste other tables into this table – type directly into the table.
- Please underline all confidential information, and separately highlight information that is submitted under 'commercial in confidence' in turquoise and all information submitted under 'academic in confidence' in yellow. If confidential information is submitted, please also send a 2nd version of your comment with that information replaced with the following text: 'academic / commercial in confidence information removed'. See the Guide to the processes of technology appraisal (section 3.1.23 to 3.1.29) for more information.
- Do not include medical information about yourself or another person from which you or the person could be identified.
- Do not use abbreviations
- Do not include attachments such as research articles, letters or leaflets. For copyright reasons, we will have to return comments forms that have attachments without reading them. You can resubmit your comments form without attachments, it must send it by the deadline.
- If you have received agreement from NICE to submit additional evidence with your comments on the appraisal consultation document, please submit these separately.

**Note:** We reserve the right to summarise and edit comments received during consultations, or not to publish them at all, if we consider the comments are too long, or publication would be unlawful or otherwise inappropriate.

Comments received during our consultations are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the comments we received, and are not endorsed by NICE, its officers or advisory committees.

Please return to: **NICE DOCS**

# Comments on the ACD received from the public through the NICE Website

| | |
|---|---|
| **Name** | [redacted] |
| **Role** | |
| **Organisation** | [redacted] |
| **Location** | |
| **Notes** | |
| **Comments on the ACD:** | |

*Has all of the relevant evidence been taken into account?*
Not considered because I'm not familiar with the evidence for this medicine

*Are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?*
The summaries appear to be reasonable interpretations.

*Are the recommendations sound and a suitable basis for guidance to the NHS?*
The recommendation in this TA refers to 'people', but should it just refer to adults? I found it confusing because people generally means both adults and children, but the text below the recommendation states that insufficient evidence is available for children (or people who could tolerate a high intensity regimen, who are not included in the recommendation). Please could this be clarified?

*Are there any aspects of the recommendations that need particular consideration to ensure we avoid unlawful discrimination against any group of people on the grounds of race, gender, disability, religion or belief, sexual orientation, age, gender reassignment, pregnancy and maternity?*
Not that I am aware of.