Macitentan in the treatment of pulmonary arterial hypertension (PAH)

Macitentan **WILL BE** funded as an option in the treatment of pulmonary arterial hypertension in line with the following criteria:

- The patient’s management is overseen by a specialist centre designated by NHS England
- The decision to offer macitentan **HAS BEEN** made by a consultant at the specialist centre (arrangements for provision of the treatment **CAN BE** made through a home care provider or arranged locally as part of a shared care arrangement)
- Macitentan **CAN BE** accessed at the discount agreed with NHS England
- Macitentan (or bosentan or ambrisentan) is an option for first line therapy **ONLY WHERE** a PDE51 drug (the usual first line therapy) is inappropriate
- Macitentan (or bosentan or ambrisentan) is an option for second line therapy in patients who have initially responded to a first line therapy but then deteriorated despite dose escalation (if appropriate) and patients who have had a sub-optimal response to first line therapy (with dose escalation where appropriate)
- Macitentan (or bosentan or ambrisentan) is an option for dual therapy (in combination with a PDE51 drug) in patients with progressive disease who have failed to respond to first and second line monotherapy or who have initially responded to monotherapy but subsequently deteriorated or who have had a sub-optimal response to monotherapy.

Provided macitentan can be accessed at the NHS England agreed discount price, the impact of this policy will be cost neutral as it adds an additional (similar cost) option to an existing point in the pathway at which two options (bosentan and ambrisentan) are already available. This policy is in line with NHS England commissioning policy for PAH.
<table>
<thead>
<tr>
<th>Strength of evidence</th>
<th>Clinical Effectiveness</th>
<th>Cost Effectiveness</th>
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<tbody>
<tr>
<td>Comments</td>
<td>The effectiveness of macitentan has been demonstrated in what is to date the largest and longest randomised controlled trial in PAH using the recommended outcomes of morbidity and mortality. The evidence is more robust than that for any of the other drugs included in the PAH pathway. In the trial setting, macitentan showed a 45% reduction in risk for morbidity and mortality outcomes that was both statistically and clinically relevant. The benefit was shown early in treatment and persisted throughout an average of more than two years’ follow up. Based on trial data, macitentan is the only PAH therapy licensed for long term use. Evidence for cost effectiveness is currently lacking.</td>
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**Summary of evidence**


**Reason for Requesting a policy recommendation:**

Reviewed following a request from Nobles Pharmacy, local consultants and the Sheffield PAH specialist centre to consider addition of macitentan to the treatment options available within the existing PAH pathway.

Where a patient is considered to have exceptional need for and capacity to benefit from a treatment that is not routinely funded, a request for individual funding may be made to the Individual Funding Requests Panel. The patient must be made aware that the Panel may not support the request and must not be given any expectation that they will be able to have the treatment until a decision to fund has been received in writing from the Panel.

For further information contact:

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