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sent by email:

cc.

Dr Pamela McHenry and colleagues
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14 June 2018

Dear Dr McHenry and colleagues

Final Evaluation Determination: Afamelanotide for treating erythropoietic protoporphyria (EPP)

Thank you for your letter of 6 June, lodging the Association's appeal against the above Final Evaluation Determination.

Introduction

The Institute's appeal procedures provide for an initial scrutiny of points that an appellant wishes to raise, to confirm that they are at least arguably within the permitted grounds of appeal ("valid"). The permitted grounds of appeal are:

- 1(a) NICE has failed to act fairly, or
- 1(b) NICE has exceeded powers;
- (2) the recommendation is unreasonable in the light of the evidence submitted to NICE

This letter sets out my initial view of the points of appeal you have raised: principally whether they fall within any of the grounds of appeal, or whether further clarification is required of any

point. Only if I am satisfied that your points contain the necessary information and arguably fall within any one of the grounds will your appeal be referred to the Appeal Panel.

You have the opportunity to comment on this letter in order to elaborate on or clarify any of the points raised before I make my final decision as to whether each appeal point should be referred on to the Appeal Panel.

I can confirm that there will be an oral hearing of the appeal.

Initial View

Ground 2

2.1 "clinical trials suggest small benefits with afamelanotide"

A valid appeal point, though I should explain that an appeal panel cannot receive new evidence.

2.2 "This is despite [NICE] taking into account the impact the condition and technology have on QoL, disability etc"

NICE committees have the benefit of clinical and patient experts to supply an expert perspective on the condition and treatment in question. Of course if the outcome of the committee's work is unreasonable, that is an appeal point, but a lack of specific expertise on the committee is not a standalone ground of appeal.

I would not be minded to refer this point to an appeal panel.

2.3 "cannot be recommended for routine funding in the NHS"

It seems to me that NICE's obligation under the HST process is to be clear about what uncertainties an MAA might be able to address. The evaluation documents do seem to do that. I am not sure NICE is responsible, or it is a valid ground of appeal, for the progress or otherwise of discussions about an MAA between the company and third parties.

I would not be minded to refer this point to an appeal panel.

2.4 "The committee took into account the full range of factors"

A valid appeal point.

2.5 "The committee concluded that the trials had shown relatively small benefits"

A valid appeal point.

2.6 "it [DLQI] has been shown to be sensitive to the impact of EPP on people with the condition"

It seems to me from FED 4.10-4.11 that the committee were aware of limitations with DLQI in EPP, and used it with caution alongside EPP-QoL. It is clear that there were drawbacks to all QoL measures available and the committee does not appear to have relied on any one to the exclusion of others. I do not think that can be said to be an unreasonable approach.

I would not be minded to refer this point to an appeal panel.

2.7 "the committee considered that an MAA would not have the plausible potential to reduce the uncertainties identified..."

For the reason given above I would not be minded to refer this point to an appeal panel.

2.8 "It (the company) was willing to enter into discussions with NHS England..."

Capping financial risk would be relevant to budget impact rather than to cost effectiveness. As the patient numbers are small, it is unlikely that this would be a case where NICE would be asked to consider budget impact. If a discussion between the company and NHS England on cost capping might have impacted on cost effectiveness I feel it is the responsibility of the company, and not NICE, to ensure that those discussions take place so as to inform the value proposition it puts forward to NICE.

I would not be minded to refer this point to an appeal panel.

Please let me have any further observations you may have on the points that I am not minded to consider valid within the next ten working days, **no later than Thursday 28 June**, and I will then finalise my decision on initial scrutiny.

Yours sincerely

Dr Rosie Benneyworth
Vice Chair
National Institute for Health and Care Excellence