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sent by email:

Mr John Chamberlayne
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14 June 2018

Dear Mr Chamberlayne

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

Thank you for your letter of 6 June, lodging the BPA'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

Ground 2 point 1

A valid point of appeal.

Ground 2 point 2

It is clear that the committee would agree with you that the ERG model has limitations (see FED 4.16, 4.19 and 4.23). However so far as modelling is concerned, it seems to me that the committee had three choices: to use the ERG model to inform its decision making, to use the company model, or to use no model. While I cannot rule it out, I am not aware of an appraisal or evaluation where a committee has concluded that all of the modelling it has been presented with is so flawed that none of it can be used to inform decision making. It strikes me that a committee that did not have some form of economic modelling to inform its decision making would have a very difficult task indeed. I cannot see in this case that the limitations in the ERG model would be so severe as to make it unreasonable to use it, which I think is your point. Nor can I see that the company model is so superior that the committee were arguably unreasonable in preferring the ERG model.

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

Ground 2 point 3

I do not think that reasonableness requires an ERG to engage with patients in developing an economic model. Patient input comes by way of patient experts and submissions, and comments in consultation, but it would not be usual to involve patients in developing an economic model (indeed again I may be wrong but I do not think it is ever done).

I think your point is also that the model does not capture patient testimony well, or at all. At least for present purposes I would accept that you are right. However the model is a decision making tool rather than the decision itself, and what is important is that the committee considers patient evidence and any other evidence that cannot be included in the model alongside the model, and comes to a decision that synthesises all of that evidence reasonably. The FED seems to me to make clear that the committee did take account of the voice of EPP patients (see for example FED 4.2, 4.9, and 4.11), and the consultation responses seem to have been taken into account.

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

Ground 2 point 4

As I am sure you will appreciate, NICE appraisals and evaluations often involve confidential information which NICE cannot disclose in consultation. The content of any possible MAA would typically be confidential, and I do not think it can be argued to be unreasonable that NICE has not provided them.

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