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28 June 2018

Dear Dr Benneyworth

RE: Final Evaluation Determination – Afamelanotide for treating erythropoietic protoporphyria

Thank you for your letter dated 14 June 2018, acknowledging our appeal against the above Final Evaluation Determination.

Ground 2, Point 1

We are pleased that you accept Ground 2, Point 1 to be a valid appeal point.

We disagree with the rejection of Ground 2, Point 3 and Point 2. Our further reasoning is below.

Ground 2: Point 3

Firstly, we would like to make it clear that our point is that the model does not capture patient testimony well. The ERG model was developed prior to patient testimony and has not been amended subsequently.

Our point stems from the fact that the ERG model fails to capture and assess the now recognised gulf between benefits reported by patients and the level of benefit that is indicated by the model. Furthermore, we do not believe that the ERG model fully assesses the real benefits of the treatment that have been communicated in patient and clinical expert testimony and communication throughout the consultation process. Our position is that:

- The ERG model is based on a model that uses measures of disadvantage that are irrelevant to EPP.
- The measure taken from the trial data uses something that is at best a feature of the treatment (additional time in light) and does not assess the real benefit of that feature which has been communicated in testimony and elsewhere.

Whilst we recognise that some attempt has been made by the committee to apply expert testimony alongside the model, the fact is that the ERG model still lies at the root of the economic decision. Decision making models and procurement exercises from all walks of life that fail to engage key expert stakeholders at the start of the modelling process, frequently result in projects that fail and/or run over budget. Cases surrounding ultra-rare conditions such as EPP, where there has been no previous decision making process, or scrutiny and review of the process applied, should surely mean that stakeholder engagement in the decision model is vital.

The ERG model should not be considered any differently. Its now recognised weaknesses stem almost entirely from the failure to engage patient and clinical experts in its development. We therefore propose that by failing to engage the patient voice in its development, NICE has not taken all reasonable measures to ensure the ERG model is a decision-making tool that is truly fit for purpose.

Ground 2 Point 2

Once the reasons why the ERG proved to be such a contentious economic model on which to base the recommendation are understood, the flaws behind the entirely reasonable attempts the committee made to incorporate the expert testimony become only too apparent. Simple extrapolation from a model, especially of a linear nature, is not scientific or truly objective. We therefore contend that the tool used for economic decision making is inappropriate and that any attempt to base decisions on it is unreasonable.

A flawed model (see G2, P3 above) will always be a flawed model. To rely on extrapolation and guestimation from a flawed model would therefore not appear to be a sound and reasonable basis from which to make the decision.

We therefore ask that you carefully review your decision to reject both Ground 2 Points2 and Point 3 of our appeal.

Ground 2, Point 4

We accept your explanation for the rejection of Ground 2, Point 4.

Yours sincerely

John Chamberlayne

BPA Chairman