



Response Initial scrutiny: International Porphyria Patient Network (IPPN)

5 April 2023

Dr Mark Chakravarty

Lead Non-executive Director NICE Appeals – Technology Appraisals and Highly Specialised Technologies

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Dear Marc,

Re: Final Evaluation Determination – Afamelanotide for treating erythropoietic protoporphyria [ID927]

Thank you very much for considering our appeal points and for giving us the chance to elaborate relevant points at the planned hearing with the Appeal Panel.

We also would like to thank you for providing us with detailed justifications regarding the appeal points you are not minded referring on to the Appeal Panel. The IPPN acknowledges the rationale for not including 1(a).2; 1(a).3; 1(b)1 and 1(b)2 as appeal points or independent appeal points, respectively.

However, we would like to reinforce our position regarding point 2.1 and kindly ask you to reconsider it as suitable for referral to the Appeal Panel based on the following: The QALY gain is calculated as a product of the increase in HRQoL benefits and the time over which these benefits are accrued. As this measure is used by NICE to compare health interventions within and between diseases, the QALY gain in our understanding needs to be calculated in the most consistent manner possible. The IPPN agrees that a treatment time of 60 years would better reflect the approximate average life expectancy of patients with EPP and therefore treatment time-horizon, if a treatment starting age of 22 years is assumed. However, in the evaluation of eliglustat (HST5), a treatment starting age of 32 to 38 years was assumed, and the HST committee accepted a time-horizon of 70 years. In the other example provided in the appeal letter (HST1), a time-horizon of 125 years was accepted which is clearly longer than the usual life expectancy and therefore treatment time. It does not add up that patients who are assumed to no longer be alive can accrue benefits for a QALY calculation while the benefits experienced by patients with EPP over the age of 70, who can be treated with afamelanotide, should not contribute to the QALY gain of afamelanotide. As analysed by the IPPN, the median time horizon in the appraisals of highly specialised technologies is 100 years. (Barman-Aksözen et al. 2023) In our assessment, the committee should have either requested time-horizons based on realistic life expectancies in all evaluations or needs to consistently apply previously accepted assumptions for the QALY calculation for their evaluations. The same can be said for applying age-adjustments to utility values. Based on the above detailed considerations, the IPPN is under the impression that it was unfair to use a shorter than usual time-horizon and age-adjusted utilities for the calculation of the QALY gain of afamelanotide.

We can confirm that the IPPN will attend the appeal hearing with two to three participants and that the material submitted by the IPPN does not contain any confidential material that would need redaction.

The main IPPN contact for the appeals procedure is [REDACTED].

Yours sincerely

[REDACTED]

[REDACTED]

Vice-President IPPN

www.porphyria.network

Barman-Aksözen, J.; Minder, A.-E.; Granata, F.; Pettersson, M.; Dechant, C.; Aksözen, M.H.; Falchetto, R. Quality-Adjusted Life Years in Erythropoietic Protoporphyrin and Other Rare Diseases: A Patient-Initiated EQ-5D Feasibility Study. *Int. J. Environ. Res. Public Health* 2023, 20, 5296.

<https://doi.org/10.3390/ijerph20075296>