Sent by e-mail only: XXXXXXXXXXXXXXXXX ; XXXXXXXXXXXXXXXXXXXXX

FAO XX X XXXXX & XX X XXXXXXXX

British Paediatric Neurology Association

2 St Andrews

Regent's Park

London

NW1 4LB

3 June 2024

Dear XXXXXXXX and XXXXXXXXXXX

**Re: Final Draft Guidance – Fenfluramine for treating seizures associated with Lennox-Gastaut Syndrome in people 2 years and over (ID1651)**

Thank you for your letter of 24 May 2024 responding to my initial scrutiny views. This is my final decision on initial scrutiny.

I assess each of your points in turn.

***Ground 1(a): In making the assessment that preceded the recommendation, NICE has failed to act fairly***

**Appeal point 1(a).1: Large unmet need in patients with Lennox-Gastaut syndrome**

I explained in my letter of 10 May 2024 why I was not minded to refer this appeal point to the Appeal Panel. I note you have provided no further comment or argument regarding unmet need and I therefore confirm this point will not be referred.

Separately, I understand that you consider the BPNA did not have the opportunity to participate in this evaluation. I explained in my letter of 10 May 2024 that the BPNA participated in this evaluation as a consultee and invited you to explain whether and how in your view NICE departed from the requirements of the Manual.

In response you stated that your nominated expert was not selected or asked to give written advice or evidence before the first committee meeting. You have not explained whether or why you consider this procedurally unfair. I have nonetheless considered this as a potential appeal point.

I have considered the process in the Manual regarding expert nomination and selection (see paragraphs 1.3.10 to 1.3.20 in particular).[[1]](#footnote-1) I have identified no departure from this in this case: you were properly invited to nominate an expert and did so; NICE was required to select experts from the nominees; and you were informed that your nominated expert had not been selected.

You have not made an argument that NICE's rejection of your nominated expert was in some way unfair, and I have identified no obvious unfairness here. It appears from the papers that the committee had the benefit of paediatric expertise from the selected experts.

Likewise you have not made an argument – and I have identified no requirement - for nominated experts that are not selected by NICE to be invited to provide written submissions or otherwise be treated preferentially to other stakeholders as a matter of procedural fairness. I note you were informed that your nominated expert could observe the committee meeting and of the opportunity to comment on the draft guidance during the consultation period.

As I have identified no arguable unfairness, I will not refer this point to the Appeal Panel.

I note that you seek clarity from NICE on how BPNA can participate in technology evaluations. Such guidance is not within the remit of the appeals process but I will ensure your letter is passed to NICE to respond as it considers appropriate.

***Ground 2: the recommendation is unreasonable in the light of the evidence submitted to NIC***

**Appeal point 2.1: The Appraisal Committee’s refusal to recommend the use of fenfluramine for treatment of seizures in patients with Lennox-Gastaut syndrome cannot reasonably be justified in the light of the evidence submitted**

I explained in my letter of 20 May 2024 why I am not minded to refer this point to the Panel. I note you have provided no further arguments and I therefore confirm this point will not be referred.

For the avoidance of doubt, I understand you consider the reduction in frequency of generalised tonic-clonic seizures justifies a positive recommendation for fenfluramine; however, you have not persuaded me that the committee's consideration of this issue was unreasonable. It appears to me the committee understood the impact on seizures [(see e.g. section B3.3.3 of the committee papers for the 31 January 2024 meeting. I can see no arguable case that, in light of the evidence of reduction in seizures, anything but a positive recommendation is unreasonable in the sense that it is obviously and unarguably wrong, illogical, or 'does not add up' (see NICE's appeals process guide, as set out in my letter to you of 10 May 2024).[[2]](#footnote-2)

Conclusion

For the reasons set out above, I will not refer your appeal for consideration at an appeal hearing. This letter therefore brings your appeal to an end.

Thank you for your comments and engagement in the appeals process.

As noted above, I will share your request for clarity as to participation in evaluations with NICE to respond as they see appropriate.

Yours sincerely

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Dr Mark Chakravarty

Lead Non-Executive Director for Appeals & Vice Chairman

National Institute for Health and Care Excellence

1. <https://www.nice.org.uk/process/pmg36/resources/nice-health-technology-evaluations-the-manual-pdf-72286779244741> [↑](#footnote-ref-1)
2. <https://www.nice.org.uk/process/pmg41/chapter/making-an-appeal#the-grounds-of-appeal> [↑](#footnote-ref-2)