

## Response ID ANON-EUBY-Y6HE-H

Submitted to **Specialised Services clinical commissioning policies and service specification - 8th Wave**

Submitted on **2016-03-25 08:57:40**

### About you

#### 1 What is your name?

**Name:**

Matt O'Neil

#### 2 Who are you responding on behalf of?

**Who are you responding on behalf of?:**

Narcolepsy UK

#### 3 What is your job title?

**Job title:**

Chair of Trustees

#### 4 What is your email address?

**Email:**

matt.oneill@narcolepsy.org.uk

### Specialised Services clinical commissioning policies and service specifications

#### 5 Would you like to comment on a service specification, or clinical commissioning policies?

Clinical commissioning policies

### Clinical Commissioning Policies

#### 12 Please indicate which clinical commissioning policy you would like to comment on:

Sodium oxybate for symptom control of narcolepsy with cataplexy (children) (E09X03):

### Clinical Commissioning Policies (continued)

#### 13 Has all the relevant evidence been taken into account?

No

**If you selected 'No', please give details:**

Only 5 responses were received to the initial consultation in December 2015, Narcolepsy UK was one of these respondents. We consider that the lack of responses indicates that it may be possible that not all evidence has been taken into account.

This proposition identifies a small cohort of paediatric patients who have not responded to first and second line medications. We believe this is a mechanism to utilise cheaper, tricyclic antidepressants or other SSRI off-label drugs ahead of Sodium-Oxybate, the single drug approved for cataplexy by the EMEA and the FDA.

Narcolepsy UK believe Sodium-Oxybate should be considered a first line medication for the treatment of cataplexy.

#### 14 Does the impact assessment fairly reflect the likely activity, budget and service impact?

No

**If you selected 'No', please tell us what is accurate?:**

We were unable to view an "Impact Assessment" but have raised questions concerning the size of the potential annual cohort, cost-effectiveness measurement of sodium-oxybate and also the allocation / ring-fencing of budget by the NHS England Specialised Commissioning Team.

We do have a concern that implementation of this policy could be detrimental to some children already prescribed sodium oxybate, particularly if an annual cohort was implemented retrospectively of failed to take into account the recent uplift in serious cataplexy cases attributed to vaccination with Pandemrix.

#### 15 Does the policy proposition accurately describe the current patient pathway that patients experience?

No

**If you selected 'No', what is different?:**

There is no formal patient pathway for children with narcolepsy treated by NHS England.  
However, many children are successfully treated with sodium-oxybate for their cataplexy by NHS England currently.

**16 Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?**

**Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?:**

Narcolepsy UK welcome the overall view that clinical consensus can be applied to this policy and that the application of adult evidence to post-pubescent children will mean that the policy can proceed as a routine commissioning policy.

The evidence of harms in the evidence base requires more balance. Untreated, sleep-deprivation itself or combined with other symptoms of narcolepsy are equally capable of creating harmful situations as are some of the alternative treatments referred to as first or second line. See 17. 8. Proposed patient pathway comments.

The major impact on equality and health inequalities is that, despite Sodium-Oxybate being licensed under the brand name Xyrem for a decade, there is still no routine clinical commissioning policy for adults. If there is strong evidence for the drug's effectiveness in the adult population, why is there no adult policy?

Without an approved adult clinical commissioning policy, any children <18 years will run the risk of losing access to their medication on reaching adulthood. Narcolepsy UK do not consider that to be an acceptable outcome.

**17 Are there any changes or additions you think need to made to this document, and why?**

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Plain Language Summary (Page 5)

Para 1 - Should read "Narcolepsy is a chronic neurological disorder".

Para 3 - Puberty should be re-labelled as "pre-pubescent puberty", a well recognised symptom of narcolepsy in children. By stating puberty, Narcolepsy UK believe that there is a chance that some children will be designated as failing to meet the policy criteria of reaching puberty and weighing >40kgs. ee 17 "Precocious puberty".

1. Introduction

Para 1 - Narcolepsy UK believe Sodium-Oxybate should be considered a first line medication for the treatment of cataplexy.

3. Definitions

Para 1 - Narcolepsy is a chronic neurological condition.....

Other symptoms need to be articulated - Excessive Daytime Sleepiness, hypnagogic & hypnopompic hallucinations, sleep-paralysis.

5. Epidemiology and needs assessment

There needs to be acceptance of the impact of narcolepsy induced by the Pandemrix vaccination in the size of the potential cohort "10 paediatric patients per year". Narcolepsy UK would like to see the evidence behind the calculation of this number. It may well prove accurate for a normal year but it will not stand the test if it was universally applied today or in the very short term.

6. Evidence Base

Narcolepsy UK believe that clinical consensus indicates that sodium-oxybate should be prescribed.

2. Whether or not sodium-oxybate is cost-effective is a contentious issue. The drug itself is an extremely basic compound, the cost is dictated by the manufacturer & patent holder.

7. Proposed criteria for commissioning

In some cases, 3 months may be insufficient to gauge the success of the medication. This may depend on progression through dosage, weight / BMI variances etc.

8. Proposed patient pathway

It is interesting that much is made of the evidence of harms in the medical base but the is also true of the alternative first & second line medications proposed.

In fact the British National Formulary states under "Suicidal behaviour and antidepressant therapy" that "The use of antidepressants has been linked with suicidal thoughts and behaviour; children, young adults, and patients with a history of suicidal behaviour are particularly at risk. Where necessary patients should be monitored for suicidal behaviour, self-harm, or hostility, particularly at the beginning of treatment or if the dose is changed."

Great care should be taken with psychological assessments of children as it is very easy to misinterpret their articulation of their symptoms, particularly dreams, nightmares, sleep-paralysis & hallucinations with other possible factors.

Narcolepsy UK also believe that these children should be assisted with a full care package to also assist them in their educational studies.

9. Proposed governance arrangements

Narcolepsy UK would like to offer their experience and that of other adult patients to inform any psychologists or CAMHS staff working with children with narcolepsy.

This is important with such a rare & debilitating condition and any psychiatric help / cognitive behavioural therapy should take into account the experiences of adults with narcolepsy.

#### 10. Proposed mechanism for funding

Narcolepsy UK welcome the recommendation that this policy be approved as a routine commissioning policy but are only too aware of how the issue of "cost-effectiveness" may impact the practical application of this policy.

We would be interested if there is any intention of "ring-fencing" budget based on the potential annual cohort of patients to ensure the practical application of this policy?

#### 11. Audit Requirements

Narcolepsy UK welcome participation in the EU - Narcolepsy Network database to enhance the scientific understanding of this condition. As members of the EU-NN, Narcolepsy UK recognise the current paucity and accuracy of data available in the UK.

#### 12. Documents which have informed this policy proposition

We note the inclusion of the Scottish Medical consortium paper (246/06). March 10, 2009. The date of publication was actually March 10, 2007.

It is a well established fact that whilst this paper states that "Sodium oxybate (Xyrem) is not recommended for use within NHS Scotland for the treatment of cataplexy in adult patients with narcolepsy", it is routinely prescribed for both adults and children by NHS Scotland.

#### **18 For E03X16 only : Prescribing of Cross-sex Hormones as part of the Gender Identity Development Service for Children and Adolescents.**

**What are the ethical considerations, including the level of competency required to understand the implications, for a young person under the age of 16, to access cross sex hormone treatment which may result in potential harms?:**

N/A

#### **19 For B01X09 only: Proton Beam Therapy for Cancer of the Prostate.**

**Has the evidence review captured all the relevant and eligible papers?:**

N/A

#### **Final question**

**20 Before completing the survey you must declare any financial or other interests in any specialised services.**

**c:**

Narcolepsy UK receive no funding, including sponsorship or grants, from companies that manufacture drugs or treatments used in the treatment of specialised services.