Document 2

From: s22
To:
Subject: RE: Orphan drug designation application outcome letter - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]
Date: Thursday, 23 January 2025 1:07:49 PM
Attachments: image002.jpg
image003.png
image004.png
image005.png
image006.gif

Dear s22

As requested in the ODD letter I confirm that a submission to register sodium oxybate will be made in February 2025.

Kind Regards
s22

s22

Reach Pharmaceuticals Pty Ltd
M: s22
E: s22 @reach-pharma.com
W: www.reach-pharma.com
A: Ground Floor, Corporate One, 84 Hotham Street, Preston VIC 3072, Australia

---

From: s22 @Health.gov.au>
Sent: Wednesday, 20 November 2024 6:04 PM
To: s22 @reach-pharma.com>
Subject: Orphan drug designation application outcome letter - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]

Dear s22

Please see attached the decision letter for your orphan drug designation application for sodium oxybate with the following indication:-
Treatment of narcolepsy.

Kind regards
s22

s22
s22 |Application Entry, Support and Export Section
Prescription Medicines Authorisation Branch | Medicines Regulation Division

T: s22 @health.gov.au

Therapeutic Goods Administration
Australian Government, Department of Health and Aged Care
PO Box 100
Woden ACT 2606
www.tga.gov.au

From: s22 [redacted]
To:
Subject: Orphan drug designation application outcome letter - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]
Date: Wednesday, 20 November 2024 6:04:26 PM
Attachments: sodium oxybate (SODIUM OXYBATE - REACH) - Reach Pharmaceuticals Pty Ltd - PM-2024-05368-1-1 - Orphan drug Designation outcome letter ~ final.pdf

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Dear s22 [redacted]

Please see attached the decision letter for your orphan drug designation application for sodium oxybate with the following indication:-
Treatment of narcolepsy.

Kind regards

s22 [redacted]

s22 [redacted]

s22 [redacted] |Application Entry, Support and Export Section
Prescription Medicines Authorisation Branch | Medicines Regulation Division

T: s22 [redacted] @health.gov.au

Therapeutic Goods Administration
Australian Government, Department of Health and Aged Care
PO Box 100
Woden ACT 2606
www.tga.gov.au

Australian Government
Department of Health and Aged Care
Therapeutic Goods Administration

s22 [redacted]
Reach Pharmaceuticals Pty Ltd
Ground Floor Corporate One
84 Hotham Street
Preston VIC 3072

Our Reference: E24-219885

Email: s22 [redacted]@reach-pharma.com

Your application under regulation 16H(1) of the Therapeutic Goods Regulations 1990 for orphan drug designation in relation to sodium oxybate (SODIUM OXYBATE-REACH) – PM-2024-05368-1-1.

Dear Sir/Madam,

I refer to the above application which was accepted by TGA on 13th November 2024.

Decision maker
I am a delegate of the Secretary of the Department of Health and Aged Care under regulation 16J(1) of the Therapeutic Goods Regulations 1990 (the Regulations) for the purposes of your application to designate the above medicine as an orphan drug.

Decision
Consideration of your application PM-2024-05368-1-1 has been completed.

I am writing to inform you, as required by regulation 16J(6) Regulations, of my decision to designate sodium oxybate (SODIUM OXYBATE-REACH) as an orphan drug under regulation 16J(1)(b)(i) of the Regulations. The indication is for the "treatment of narcolepsy".

The designation comes into force when it is made and remains in force for 6 months. Under regulations 16L and 16 M of the Regulations, the designation can be extended or revoked in certain circumstances.

Publication of decision
Under regulation 16J(5), as I have decided to designate sodium oxybate (SODIUM OXYBATE-REACH) as an orphan drug, I am required to publish a notice on the Department's website stating:
(a) Reach Pharmaceuticals Pty Ltd is the sponsor of the medicine;
(b) the indication of the medicine is for "treatment of narcolepsy";
(c) the dosage form of the medicine is oral solution; and
(d) the medicine is a designated orphan drug.

This information is published on the TGA website at the following address:
https://www.tga.gov.au/designation-notices

Application for registration
The TGA would appreciate advice on when you plan to submit an application to register the designated product for this indication. If the indication for sodium oxybate (SODIUM OXYBATE-REACH) in your registration application differs from that in your application for orphan designation, additional information may be required to demonstrate that orphan designation still applies.

Yours sincerely,

Signed and authorised by

s22
Delegate of the Secretary

20th November 2024

From: s22
To:
Subject: Response to Request for information - Orphan drug designation - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]
Date: Friday, 15 November 2024 2:08:05 PM
Attachments: image002.png
image003.png
image004.png
image005.png
Roth 2024.pdf
Dauvilliers 2020.pdf
Dauvilliers 2017.pdf
Roth 2017.pdf

H s22
Please see the response below and attachments.

I did stop the clock when I sent out the question so please let me know if you would like me to re-activate the clock once you are satisfied with the information provided.

Kind regards
s22

From: s22 @reach-pharma.com>
Sent: Friday, 15 November 2024 1:57 PM
To: s22 @Health.gov.au>
Subject: RE: Request for information - Orphan drug designation - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]

REMINDER: Think before you click! This email originated from outside our organisation. Only click links or open attachments if you recognise the sender and know the content is safe.

Dear s22

Apologies for the oversight, I thought the narcolepsy export that had prepared the section included the papers.

Please refer to my comments:

Dauvilliers, Y., et al. (2017). "Effect of sodium oxybate, modafinil, and their combination on disrupted nighttime sleep in narcolepsy." Sleep medicine **40**: 53-57
s22 *: Please find attached*

Dauvilliers, Y., et al. (2020). "Narcolepsy Severity Scale: a reliable tool assessing symptom severity and consequences." Sleep **43**(6). [sic]
s22 *Please find attached*

Roth, T., et al. (2023). "Effects of oxybate dose and regimen on disrupted nighttime sleep and sleep architecture." Sleep medicine. [sic]
s22 *Submitted as Roth 2024, I have re-attached it*

Roth et al, 2017 [sic]
s22 *Please find attached*

Kind Regards
s22

s22

Reach Pharmaceuticals Pty Ltd
M s22
E: s22 @reach-pharma.com
W: www.reach-pharma.com
A: Ground Floor, Corporate One, 84 Hotham Street, Preston VIC 3072, Australia

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From: s22 @Health.gov.au>
Sent: Friday, 15 November 2024 1:43 PM
To: s22 @reach-pharma.com>
Subject: Request for information - Orphan drug designation - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]

Dear s22

The clinical evaluator reviewing your orphan drug designation application for sodium oxybate has noted that you have made reference to additional literature, but they are unable to find the papers among the attachments.

Could you please provide PDF copies of the following articles:

Dauvilliers, Y., et al. (2017). "Effect of sodium oxybate, modafinil, and their combination on disrupted nighttime sleep in narcolepsy." Sleep medicine **40**: 53-57

Dauvilliers, Y., et al. (2020). "Narcolepsy Severity Scale: a reliable tool assessing symptom severity and consequences." Sleep **43**(6). [sic]

Roth, T., et al. (2023). "Effects of oxybate dose and regimen on disrupted nighttime sleep and sleep architecture." Sleep medicine. [sic]

Roth et al, 2017 [sic]

Kind regards
s22

s22
s22 |Application Entry, Support and Export Section
Prescription Medicines Authorisation Branch | Medicines Regulation Division

T: s22 @health.gov.au

Therapeutic Goods Administration
Australian Government, Department of Health and Aged Care
PO Box 100
Woden ACT 2606
www.tga.gov.au

From: s22 [redacted]
To:
Subject: Request for information - Orphan drug designation - PM-2024-05368-1-1 - sodium oxybate [SEC=OFFICIAL]
Date: Friday, 15 November 2024 1:42:37 PM

Dear s22 [redacted]

The clinical evaluator reviewing your orphan drug designation application for sodium oxybate has noted that you have made reference to additional literature, but they are unable to find the papers among the attachments.

Could you please provide PDF copies of the following articles:

Dauvilliers, Y., et al. (2017). "Effect of sodium oxybate, modafinil, and their combination on disrupted nighttime sleep in narcolepsy." Sleep medicine **40**: 53-57

Dauvilliers, Y., et al. (2020). "Narcolepsy Severity Scale: a reliable tool assessing symptom severity and consequences." Sleep **43**(6). [sic]

Roth, T., et al. (2023). "Effects of oxybate dose and regimen on disrupted nighttime sleep and sleep architecture." Sleep medicine. [sic]

Roth et al, 2017 [sic]

Kind regards
s22 [redacted]

s22 [redacted]
s22 [redacted] |Application Entry, Support and Export Section
Prescription Medicines Authorisation Branch | Medicines Regulation Division

T: s22 [redacted]@health.gov.au

Therapeutic Goods Administration
Australian Government, Department of Health and Aged Care
PO Box 100
Woden ACT 2606
www.tga.gov.au

Australian Government
Department of Health
Therapeutic Goods Administration

TGA use only

This form, when completed, will be classified as **'For official use only'**.
For guidance on how your information will be treated by the TGA see: Treatment of information provided to the TGA at <https://www.tga.gov.au/treatment-information-provided-tga>.

Designation checklist: for sponsor seeking Orphan Drug designation

Please note: The check lists are intended to assist applicants to determine if they have provided all of the necessary information to allow the TGA to make an informed decision on the designation application.

It is your responsibility to download, complete and attach the checklist as part of the supporting documentation provided for a designation application. See **TGA Business Services** for more information.

1. **Is your intended application for only one medicine and one indication?** ☒ Yes ☐ No

   You need to submit separate applications for each medicine and indication

2. **Have you prepared a cover letter for your application?** ☒ Yes ☐ No

3. **Have you included a description of the condition for which the medicine, including vaccines or in vivo diagnostic agents is intended?**

   – Details of the condition ☒ Yes ☐ No

   – Proposed indication ☒ Yes ☐ No

   – Medical plausibility ☒ Yes ☐ No

     - the rationale for use of the medicine in the proposed orphan indication (the medicine will be effective in the treatment, prevention or diagnosis of the condition).
     - if the proposed orphan condition is a subset of a condition affecting a larger population, then the medicine would not be effective for the larger population.

4. **Have you included a justification of the life threatening or seriously debilitating* nature of the condition?** ☒ Yes ☐ No

   – * A prominent feature of the orphan condition (i.e. affecting an important portion of the target population) is morbidity with a well-established, major impact on the functioning of the person based on objective and quantifiable medical or epidemiological information. Short lived and /or self-limiting morbidity is not considered seriously debilitating.

5. **Have you included the prevalence of the condition?**
   - Prevalence of the proposed orphan condition in Australia ☒ Yes ☐ N/A
   - Prevalence and incidence of the overall condition in Australia ☒ Yes ☐ N/A

6. **Have you included a justification for the lack of financial viability?**
   - Grants and tax incentives ☐ Yes ☒ N/A
   - Past and future Australian development costs ☐ Yes ☒ N/A
   - Australian production and/or marketing costs ☐ Yes ☒ N/A
   - Expected Australian revenues ☐ Yes ☒ N/A
   - Certification (qualified accountant) ☐ Yes ☒ N/A

7. **Have you included a comparison against registered therapeutic goods* for the prevention diagnosis or treatment of the proposed condition?**
   - Details of registered therapeutic goods ☒ Yes ☐ N/A
   - Declaration that there are no registered therapeutic goods ☐ Yes ☒ N/A
   - Justification of significant benefit ☒ Yes ☐ N/A

   **Note: for the purposes of this comparison, registered therapeutic goods do not include therapeutic goods in the part of the Register for provisionally registered goods.*

8. **Have you included a description of the product development?** ☒ Yes ☐ No

9. **Have you included the current regulatory status in Australia and overseas?** ☒ Yes ☐ No

10. **Have you included a Bibliography containing all published references referred to in your application?** ☒ Yes ☐ No

EUROPEAN
COMMISSION

Brussels, 21.6.2022
C(2022) 4421 final

COMMISSION IMPLEMENTING DECISION

of 21.6.2022

relating to the designation of "Sebetralstat" as an orphan medicinal product under Regulation (EC) No 141/2000 of the European Parliament and of the Council

(Text with EEA relevance)

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

COMMISSION IMPLEMENTING DECISION

of 21.6.2022

relating to the designation of "Sebetralstat" as an orphan medicinal product under Regulation (EC) No 141/2000 of the European Parliament and of the Council

(Text with EEA relevance)

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

THE EUROPEAN COMMISSION,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products[1], and in particular the first sentence of Article 5(8) thereof,

Having regard to the application submitted by Kalvista Pharmaceuticals (Ireland) Limited on 22 February 2022 under Article 5(1) of Regulation (EC) No 141/2000,

Having regard to the favourable opinion of the European Medicines Agency, drawn up on 12 May 2022 by the Committee for Orphan Medicinal Products and received by the Commission on 20 May 2022,

Whereas:

(1) The application submitted by Kalvista Pharmaceuticals (Ireland) Limited concerning the medicinal product “Sebetralstat” was validated on 25 March 2022 under Article 5(4) of Regulation (EC) No 141/2000.

(2) "Sebetralstat" meets the designation criteria referred to in Article 3(1) of Regulation (EC) No 141/2000.

(3) The application should therefore be accepted,

HAS ADOPTED THIS DECISION:

Article 1

The medicinal product “Sebetralstat” is designated as an orphan medicinal product for the indication: Treatment of hereditary angioedema. It shall be entered in the Community Register of Orphan Medicinal Products under number EU/3/22/2625.

Article 2

The European Medicines Agency shall make available to all interested parties the opinion of the Committee on Orphan Medicinal Products referred to in this Decision.

[1] OJ L 18, 22.1.2000, p.1.

Article 3

This Decision is addressed to Kalvista Pharmaceuticals (Ireland) Limited, 10 Earlsfort Terrace, Dublin 2, D02 T380, Co. Dublin, Ireland.

Done at Brussels, 21.6.2022

For the Commission

s22 [redacted]

CERTIFIED COPY
For the Secretary-General

s22 [redacted]

Decision-making & Collegiality
EUROPEAN COMMISSION

From: s22
To: s22
Cc: s22
Subject: PM-2024-03443-1-2 - sebetralstat (EKTERLY) - Orphan Outcome letter [SEC=OFFICIAL]
Date: Friday, 2 August 2024 11:03:25 AM
Attachments: PM-2024-03443-1-2 - sebetralstat (EKTERLY) - JACE Pharma Pty Ltd - Orphan outcome letter.pdf
image003.png
image004.gif
image005.png
image006.png

Dear s22,

Please find attached the Orphan outcome letter for your sebetralstat (EKTERLY) application PM-2024-03443-1-2.

Regards,

s22
s22 | **Application Entry Team**
Applications, Exports and Engagement Section
Prescription Medicines Authorisation Branch
Medicines Regulation Division | Health Products Regulation Group
Australian Government, Department of Health and Aged Care
s22 @health.gov.au
Location: 27 Scherger Drive, Fairbairn Business Park, Level 1 North
PO Box 100, Woden ACT 2606, Australia

The Department of Health and Aged Care acknowledges First Nations peoples as the Traditional Owners of Country throughout Australia, and their continuing connection to land, sea and community. We pay our respects to them and their cultures, and to all Elders both past and present.

This response is general information given to you without prejudice; it is not binding on the TGA and you should get your own independent legal advice to ensure that all of the legislative requirements are met.

Australian Government
Department of Health and Aged Care
Therapeutic Goods Administration

s22
JACE Pharma Pty Ltd
s22

Our Reference: E24-335811

Email: s22 @s47G

Your application under regulation 16H(1) of the Therapeutic Goods Regulations 1990 for orphan drug designation in relation to sebetralstat (EKTERLY) PM-2024-03443-1-2

Dear s22 ,

I refer to the above application which was accepted by TGA on 31 July 2024.

Decision maker
I am a delegate of the Secretary of the Department of Health and Aged Care under regulation 16J(1) of the Therapeutic Goods Regulations 1990 (the Regulations) for the purposes of your application to designate the above medicine as an orphan drug.

Decision
Consideration of your application PM-2024-03443-1-2 has been completed.
I am writing to inform you, as required by regulation 16J(6) Regulations, of my decision to designate sebetralstat (EKTERLY) as an orphan drug under regulation 16J(1)(b)(i) of the Regulations. The indication is for the treatment of hereditary angioedema.

The designation comes into force when it is made and remains in force for 6 months. Under regulations 16L and 16 M of the Regulations, the designation can be extended or revoked in certain circumstances.

Publication of decision

Under regulation 16J(5), as I have decided to designate sebetralstat (EKTERLY) as an orphan drug, I am required to publish a notice on the Department's website stating:
(a) JACE Pharma Pty is the sponsor of the medicine, ;
(b) the indication of the medicine is for the treatment of hereditary angioedema;
(c) the dosage form of the medicine is tablet; and
(d) the medicine is a designated orphan drug.
This information is published on the TGA website at the following address:
https://www.tga.gov.au/designation-notices

Application for registration
The TGA would appreciate advice on when you plan to submit an application to register the designated product for this indication. If the indication for sebetralstat (EKTERLY) in your registration application differs from that in your application for orphan designation, additional information may be required to demonstrate that orphan designation still applies.

Yours sincerely,

Signed and authorised by

s22
Delegate of the Secretary

02 August 2024