EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Publication of the Orphan Maintenance Assessment Report

Industry stakeholder platform on research and development support, 15.11.2017

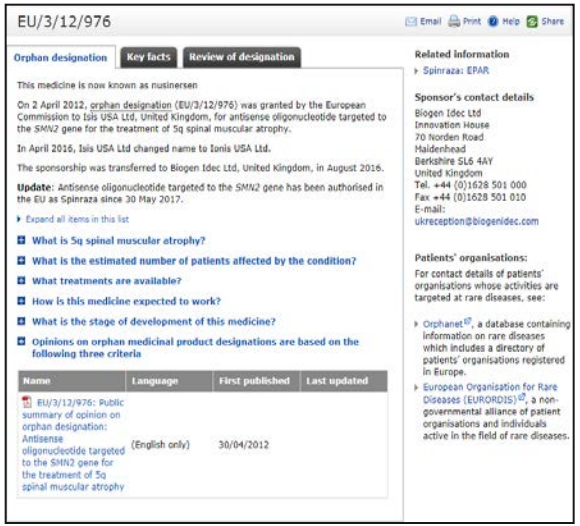
Presented by Kristina Larsson, Head of Orphan Medicines Office

Currently published information on orphan medicines

Public Summary of Opinion (PSO)

Recommendation for maintenance of OD at the time of MA

COMP minutes



EU/3/12/976

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Orphan designation | Key facts | Review of designation

This medicine is now known as nusinersen

On 2 April 2012, orphan designation (EU/3/12/976) was granted by the European Commission to Isis USA Ltd, United Kingdom, for antisense oligonucleotide targeted to the SMN2 gene for the treatment of 5q spinal muscular atrophy.

In April 2016, Isis USA Ltd changed name to Ionis USA Ltd.

The sponsorship was transferred to Biogen Idec Ltd, United Kingdom, in August 2016.

Update: Antisense oligonucleotide targeted to the SMN2 gene has been authorised in the EU as Spinraza since 30 May 2017.

Expand all items in this list

- What is 5q spinal muscular atrophy?
- What is the estimated number of patients affected by the condition?
- What treatments are available?
- How is this medicine expected to work?
- What is the stage of development of this medicine?
- Opinions on orphan medicinal product designations are based on the following three criteria

Name	Language	First published	Last updated
EU/3/12/976: Public summary of opinion on orphan designation: Antisense oligonucleotide targeted to the SMN2 gene for the treatment of 5q spinal muscular atrophy	(English only)	30/04/2012	

Related information

Spinraza: EPAR

Sponsor's contact details

Biogen Idec Ltd
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70 Norden Road
Maidenhead
Berkshire SL6 4AY
United Kingdom
Tel. +44 (0)1628 501 000
Fax +44 (0)1628 501 010
E-mail:
ukreception@biogenidec.com

Patients' organisations:

For contact details of patients' organisations whose activities are targeted at rare diseases, see:

Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.

European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

EU/3/12/976

Orphan designation | Key facts | Review of designation

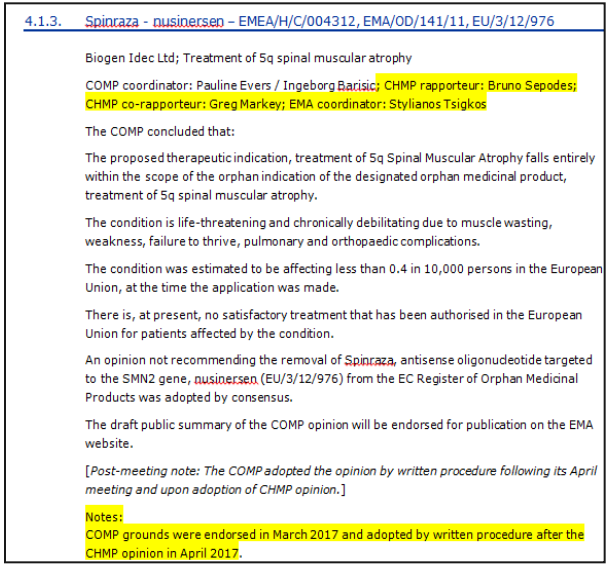
On 25 April 2017, the Committee for Orphan Medicinal Products (COMP) concluded its review of the designation EU/3/12/976 for Spinraza (nusinersen, previously known as antisense oligonucleotide targeted to the SMN2 gene) as an orphan medicinal product for the treatment of 5q spinal muscular atrophy. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. The COMP recommended that the orphan designation of the medicine be maintained[1].

[1]The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with the same therapeutic indication cannot be placed on the market.

Expand all items in this list

- Life-threatening or long-term debilitating nature of the condition
- Prevalence of the condition
- Existence of other methods of treatment
- Conclusions

Name	Language	First published	Last updated
Recommendation for maintenance of orphan designation at the time of marketing authorisation: Spinraza (nusinersen) for the treatment of 5q spinal muscular atrophy	(English only)	21/06/2017	



4.1.3. Spinraza - nusinersen – EMEA/H/C/004312, EMA/OD/141/11, EU/3/12/976

Biogen Idec Ltd; Treatment of 5q spinal muscular atrophy

COMP coordinator: Pauline Evers / Ingeborg Barisic; CHMP rapporteur: Bruno Sepodes; CHMP co-rapporteur: Greg Markey; EMA coordinator: Stylianos Tsigkos

The COMP concluded that:

The proposed therapeutic indication, treatment of 5q Spinal Muscular Atrophy falls entirely within the scope of the orphan indication of the designated orphan medicinal product, treatment of 5q spinal muscular atrophy.

The condition is life-threatening and chronically debilitating due to muscle wasting, weakness, failure to thrive, pulmonary and orthopaedic complications.

The condition was estimated to be affecting less than 0.4 in 10,000 persons in the European Union, at the time the application was made.

There is, at present, no satisfactory treatment that has been authorised in the European Union for patients affected by the condition.

An opinion not recommending the removal of Spinraza, antisense oligonucleotide targeted to the SMN2 gene, nusinersen (EU/3/12/976) from the EC Register of Orphan Medicinal Products was adopted by consensus.

The draft public summary of the COMP opinion will be endorsed for publication on the EMA website.

[*Post-meeting note: The COMP adopted the opinion by written procedure following its April meeting and upon adoption of CHMP opinion.*]

Notes:
COMP grounds were endorsed in March 2017 and adopted by written procedure after the CHMP opinion in April 2017.

Background for the new publication of the OMAR*

- There is an expected benefit in providing more detailed information of the review of the orphan status at time of marketing authorisation:
  - Stakeholders have expressed interest that this information be made available. This includes down-stream decision makers, patients' organisations as well as regulators themselves.
  - Industry at the R&D stakeholder platform in April 2017 equally asked for more transparency on these assessments.
- Publication will increase transparency and ensure that we apply a consistent approach when publishing key opinion documents.

* Orphan Maintenance Assessment Report

What is going to be published?

- Positive and negative reports
- Assessment with List of Questions in case of withdrawal (in line with CHMP outputs)
- Use very similar process as for EPAR in terms of identification of commercially confidential information
- Shorter timelines than EPAR as the OMAR is expected to be not longer than 20 pages
- Aim to publish the OMAR at the same time as the EPAR
- This more extensive document will replace the currently published summary
- For all opinions as of Oct CHMP 2017.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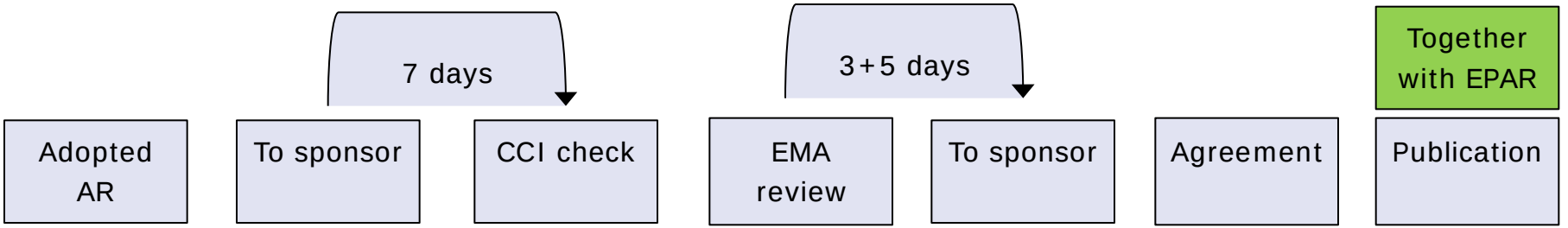
Orphan Maintenance Assessment Report

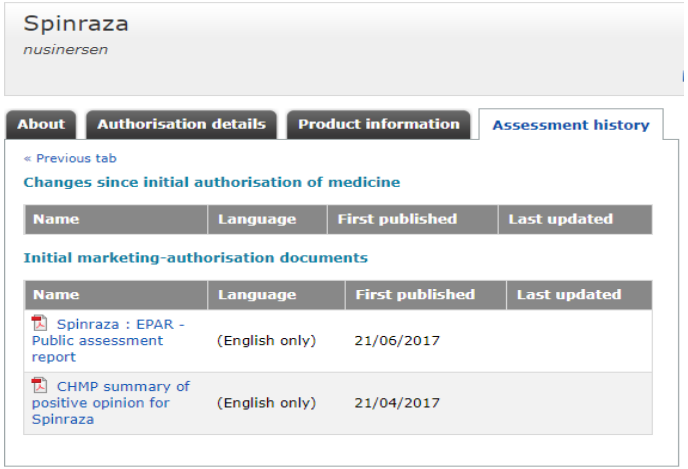
Spinraza

Note

Process for interaction with the applicant post-adoption



Where will it be published?



Spinraza: OMAR – Orphan Maintenance Assessment report

\+ what is the orphan status?

Structure and content of OMAR

1. Condition
2. Intention to treat
3. Seriousness
4. **Prevalence**
5. Existing methods
6. **Significant Benefit**

Same as in maintenance template!

Duplication of information already contained within the EPAR to be avoided.

Condition / Intention to treat / Seriousness

- Generally kept short, with reference to the EPAR where possible.
- Some aspects to be addressed:
  - Discussions of any changes to the condition or classifications of the condition.
  - A statement on whether the therapeutic indication falls within the scope of the orphan condition.
  - Confirmation of the seriousness of the condition is confirmed based on review at time of designation.
  - Survival and main complications of the condition will be discussed.

Prevalence

- This section will be expanded for purpose of the OMAR to include information on:
  - how the sponsor came to the final prevalence conclusion
  - what methodology the sponsor used in their calculation
  - details of the type of sources used
  - the final concluded prevalence
- Specific published references will be mentioned if this information is important for the assessment.

Existing methods

- List of all authorised products for the condition.
- Treatment algorithm.
- Reference to guidelines used (e.g. ESMO).
- Any new treatments since designation to be specifically mentioned.
- If hospital preparations exist and are considered satisfactory methods, these will be included here.

Significant Benefit

- Relevant products for the population covered by the therapeutic indication identified. A discussion vs each of those relevant products will be captured.
- Information from the sponsor's submission will be used here to show what the COMP based their assessment on.
- Trials relevant to the Significant Benefit assessment will be mentioned (reference to the EPAR where possible).
- If specific analysis are done only for the Significant Benefit assessment these will be covered here (e.g. indirect comparisons).
- Detailed description of the reasons why the COMP considers the Significant Benefit positive (or negative for negative opinions).

Questions and Discussion!