Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)

8th Industry stakeholder platform on research and development support

11 July 2022

Presented by Kristina Larsson
Head of Orphan Medicines Office
# EMA Survey on Orphan Maintenance Assessment Reports (OMARs)

<table>
<thead>
<tr>
<th>Launch</th>
<th>26 October 2021</th>
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<tbody>
<tr>
<td>Conclusion</td>
<td>10 December 2021</td>
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</tbody>
</table>
| Target group    | EU/EEA National Competent Authorities (NCA)  
Patients’ Organisations  
Healthcare professionals  
Academia  
Health Technology Assessment (HTA) Bodies  
Payer Community  
Pharmaceutical Industry |
| Contributions   | 79 responses received |
| Scope           | Gather information from various stakeholders on their current experience with the publicly available OMARs |
| Aim             | Continuous improvement of the OMAR publication process |

1 Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
The survey was made up of nine questions:

1. What is your affiliation?
2. Have you ever accessed an OMAR and/or an Orphan Designation Withdrawal Assessment Report published on EMA website?
3. How did you first learn about the OMAR?
4. Do you know where on EMA website you can find the OMAR for a particular orphan medicinal product?
5. Have you ever used/reviewed an OMAR?
6. Have you ever used the OMAR as reference for ... ?
7. Did you use the information as guidance for ... ?
8. Do you agree or disagree with the following statement: "The overall information contained in the OMAR is informative".
9. Would you like to share any observations on the OMAR and/or give any suggestions on how the OMAR could be improved?

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
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Have you ever accessed an OMAR and/or an Orphan Designation Withdrawal Assessment Reports published on EMA website?

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)

Q2 - "yes" answers

- Industry: 47.37%
- NCA: 13.16%
- Other: 10.53%
- HTA: 7.89%
- Payer: 7.89%
- Patients' organisation: 5.26%
- Healthcare professional: 2.63%
- Healthcare professional: 2.63%
- Healthcare professional: 2.63%
- NCA: 2.63%

48% Yes
52% No
How did you first learn about the OMAR?

<table>
<thead>
<tr>
<th>Source of information</th>
<th>Percentage</th>
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<tbody>
<tr>
<td>EMA communications</td>
<td>56.96%</td>
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<tr>
<td>Referral from a colleague or reference in a document</td>
<td>21.52%</td>
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<tr>
<td>Other*</td>
<td>12.66%</td>
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<tr>
<td>Discussions at stakeholder meeting (e.g. R&amp;D platform, EMA meetings with HTAs or payers)</td>
<td>7.59%</td>
</tr>
<tr>
<td>No Answer</td>
<td>1.27%</td>
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</tbody>
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*other sources include: BBC, COMP, stakeholder meetings, trade associations, reference in a document, company experience.

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
Do you know where on EMA website you can find the OMAR for a particular orphan medicinal product?*

61% of responders knew where on EMA website they could find the OMAR for a particular orphan medicinal product.

<table>
<thead>
<tr>
<th>Group</th>
<th>Percentage</th>
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<tbody>
<tr>
<td>Patients' organisation</td>
<td>18.75%</td>
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<tr>
<td>Industry</td>
<td>47.92%</td>
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<tr>
<td>NCA; Other</td>
<td>2.08%</td>
</tr>
<tr>
<td>Healthcare professional</td>
<td>4.17%</td>
</tr>
<tr>
<td>Payer</td>
<td>4.17%</td>
</tr>
<tr>
<td>HTA</td>
<td>4.17%</td>
</tr>
<tr>
<td>Other</td>
<td>6.25%</td>
</tr>
<tr>
<td>NCA</td>
<td>10.42%</td>
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Only positive responses to Q4
Have you ever used/reviewed an OMAR?

If “yes” to Q2:
Yes = 35
No = 3

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
Have you ever used the OMAR as reference for:

- An orphan designation maintenance procedure: 18 responses
- An orphan designation submission: 17 responses
- An orphan relative effectiveness assessment: 8 responses
- Other*: 8 responses
- No answer: 5 responses

Multiple responses were possible.

*other answers included: understand the grounds/arguments/points that the orphan mainenances, SB, prevalence, and/or both prevalence and SB were based on.

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
Did you use the information as guidance for:

- Methodology for the justification of significant benefit
- Prevalence calculation
- Other
- No answer

9 Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
Do you agree or disagree with the following statement: "The overall information contained in the OMAR is informative"

![Bar chart showing percentages of responses to the statement.]

Outcomes of the EMA Survey on Orphan Maintenance Assessment Reports (OMARs)
Would you like to share any observations on the OMAR and/or give any suggestions on how the OMAR could be improved?

Among others:

- improve on visibility and search function of OMARs on EMA website;
- advertise the possibility of pre-assessment meeting on EMA website;
- administrative suggestions (e.g., include Approval Commission Decision date, share full list of literature reference, maintenance of orphan criteria presented in tabular form);
- improve dissemination to some stakeholder groups (i.e., patients’ organisations);
- ensure same level of details across OMARs;
Cont.

• provide more detailed information
  o on the discussions around significant benefit (methodology used, category of evidence, what existing treatments will be considered, full reasoning of COMP’s conclusions, etc.);
  o on prevalence (how data submitted by the sponsor is validated);
  o on the chronically debilitating and/or life-threatening nature of the condition (to be supported by references to scientific literature);
  o on how deviations from advice received impact on the relevance of the data submitted
Thank you for your attention
Any questions?