Public consultation feedback on the Principles for value assessment and funding processes in rare diseases by European Working Group for Value Assessment and Funding Processes in Rare Diseases

Thank you for taking part in this public consultation.

Background to the European Working Group initiative

The experts of the European Working Group for Value Assessment and Funding Processes in Rare Diseases have drafted eight Principles which aim to provide a qualitative toolkit for European decision-makers involved in the value assessment and funding processes of OMPs. Their ambition is to facilitate patient access to these products by fostering greater uniformity and consistency in the methods used to make reimbursement and funding decisions for OMPs.

The European Working Group is composed of 10 individuals drawn from different disciplines – physicians, patient advocates, academic health economists, regulators and politicians – and from different European countries. The unifying characteristic is an interest or expertise in issues relating to rare diseases, OMPs and value assessment. The group is led by Professor Lieven Annemans from Belgium.

Objective of the Orphanet Public Consultation

The objective of the European Working Group members is to ensure the Principles are relevant and applicable at country level systems. The group is therefore seeking wider feedback on their draft document (see next page).

Anyone with an interest or expertise in OMPs Value assessment is invited to comment on the document. The closing date for receiving comments and feedback will be the 22nd of April 2016.

Complete the survey

Before answering the survey, please read the “Principles for value assessment and funding processes in rare diseases” (next page). Then click on the following link to provide your feedback on the document: Survey

Sponsor involvement

The Working Group meetings were initially sponsored by Celgene. Moving forward support for this initiative will be from a cross-industry consortium.

Organisation support

The Working Group was provided with organisational support by Dolon, a healthcare consultancy company with an expertise in rare diseases and therapies. Dolon is the facilitator of this survey.
Principles for value assessment and funding processes in rare diseases.

From the European Working Group for Value Assessment and Funding Processes in Rare Diseases.

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European Working Group members:

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1 Introduction

The principles outlined in this document aim to provide a qualitative toolkit for European decision-makers involved in the value assessment and funding processes of orphan medicinal products (OMPs). The ambition is to facilitate patient access to these products by fostering greater uniformity and consistency in the methods used to make reimbursement and funding decisions for OMPs. The principles are structured to provide a high-level roadmap in respect to:

- Value assessment of OMPs
- Pricing and reimbursement decision process for OMPs
- Sustainability of funding for OMPs
- European consolidation and cooperation

These principles are an output of a collaboration between rare disease experts, including Health Technology Assessment (HTA) representatives, physicians, patient representatives, academics, politicians and industry representatives.

2 Principles of OMP value assessment

The principles outlined in this section relate to the value assessment of OMPs, whereby value is defined as a combination of treatment and disease related characteristics that affect patients, health care systems and wider society. Value is to be distinguished from the concept of 'value for money'. The latter refers to whether, given its value, a new treatment is worth its cost.

PRINCIPLE 1: All regulatory and health technology assessments of OMPs undertaken at a European level should be acknowledged by national health authorities.

- National pricing and reimbursement agencies should build on and use the decisions and recommendations at a European level, including:
  - The Committee for Orphan Medicinal Products (COMP)'s assessment of significant benefit and prevalence
  - The EMA's European Public Assessment Report
  - Relative effectiveness assessments undertaken by the European permanent network on HTA.

PRINCIPLE 2: The assessment and appraisal of OMPs in Europe should incorporate rare disease expertise from both the healthcare professional (HCP) and patient perspectives.

- Due to the scarcity of disease-specific knowledge among policy makers in orphan diseases, HCPs and patients should be involved in the value assessment in the following ways:
Disease-specific expert physicians (and other relevant academic specialists) being consulted by the bodies that assess and appraise OMPs

Systematic representation in the bodies that assess and appraise OMPs of an overarching patient association

Disease-specific patient representatives being consulted about the specific issues related to the disease under evaluation.

PRINCIPLE 3: OMP assessment should consider all relevant elements of value for OMPs in an appropriate multi-dimensional framework

- Decision-makers should consider OMP value from the perspective of patients, the healthcare system and wider society (see table in annex - page 8).

- While the elements that are used to assess OMP value and the other considerations must reflect national societal preferences, the Working Group has proposed a draft set of core elements that it believes should be incorporated into all systems (see table in annex - page 8).

- Assessment and appraisal bodies should make explicit which elements of value they prioritise and based on which rationale.

- They should also make explicit how societal preferences affect the assessment of value of OMPs and how the rarity of a disease influences their assessment and appraisal.

PRINCIPLE 4: To accommodate evidential uncertainty, value assessment should be adaptive when relevant

- At the time of launch, it should be recognised that due to the size of the patient population and limited natural history data, there will often be incomplete information to provide certainty around the clinical benefit and overall value of a new OMP. Therefore there will often be a need for a continuum of evidence generation. Further data collection will often be required post-launch to enhance the understanding of the clinical benefit and the value overall, and to reduce uncertainty.

- Value assessment processes therefore need to be adaptive (i.e. contingent), where necessary, and continuous rather than binary at the point of launch. Evidential uncertainty should be considered in light of the specificities of the disease under assessment.

- Where adaptive processes are required, all parties (payers, assessment bodies, involved health care professionals, patients and industry) need to agree on this iterative process and be clear about:
  - the evidence required at each step of the assessment
  - the implications of not meeting the requirements and expectations initially agreed.

- Where possible, the collection and analysis of real-world data should be undertaken at a European level and should be integrated in disease level registries and databases to obtain more European consistency in the continuous assessment and appraisal of OMPs.
3 Pricing and reimbursement decisions

PRINCIPLE 5: Pricing and reimbursement decisions should be founded on the assessment of OMP product value and adjusted to reflect other considerations beyond product value.

- Price and reimbursement decisions should aim at contributing to the right balance between allowing sufficient revenue generation to fund new research investment in rare diseases and attract private funders, while maximising value for money for healthcare systems.
- Reimbursement decisions should be based on the value delivered by an OMP as described in Principle 3.
- Beyond OMP value, other considerations that are not directly related to the benefit of the product may be relevant when making pricing and reimbursement decisions (see table in annex – page 8).
- Among these, one may be cost-effectiveness. In those countries where cost-effectiveness is applied to assess value for money, the incremental cost-effectiveness ratio (ICER) thresholds should be modulated to reflect:
  - all the specificities of rare diseases (including rarity, unmet therapeutic need, societal preferences)
  - the value that the EU attributes to OMPs through the incentives put in place to develop them.
- All eligible patients within the authorised label of an OMP should be considered in the reimbursement decision although different decisions on access may apply to different sub-populations.
- Pricing and reimbursement decisions should be adaptive where relevant and should allow movement both up and down with newly generated evidence on value.
- If the price and reimbursement status of other OMPs is considered in the decision process, then it is important to ensure that different types of OMPs are categorised (e.g. based on disease characteristics, number of patients, characteristics of the product, etc.) to compare like with like.
4 Sustainable funding systems

PRINCIPLE 6: Adequate funding should be provided at the national level to ensure patient access to OMPs.

- Funding for OMPs should be coordinated at a national level in order to avoid disparities in access between regions and to pool the financial risk of irregular distribution of patients between regions.
- Regional and local funding bodies should liaise and cooperate with national authorities to avoid inconsistencies and inequalities in regional access.
- It is preferable that funding for OMPs should come out of normal healthcare budgets rather than from ear-marked rare disease funds that do not allow for a long-term perspective.
- Funding for OMPs should be managed in such a way as to avoid financial disincentives to provide OMPs.

PRINCIPLE 7: Rational and evidence-based funding mechanisms should be developed to guarantee long-term sustainability.

- Manufacturers, payers and assessment bodies should collaborate nationally to improve forecasting and cooperate at the European level for horizon scanning with the aim of helping budget holders predict and plan for expenditure and ensure adequate funding of OMPs.
- Manufacturers, payers, assessment bodies, centres of expertise and European reference networks should collaborate well in advance of a product launch:
  a. to collect data on the true prevalence of a given rare disease in order to minimise financial uncertainty for payers
  b. to develop adequate capabilities for large scale post-launch data collection.

5 European Consolidation

PRINCIPLE 8: In the future there should be greater consolidation of OMP value assessment processes at a European level.

- While recognising that the reality today is one of national level competence for price and reimbursement negotiations, there is potential for a greater role for European level value assessment in the future.
- Advantages from collaboration between European member states on value assessment would include:
  o Guarantee more consistency between member states in the definition and assessment of value
  o Greater concentration of clinical expertise
  o Pooling of data on epidemiology
- Opportunities for more systematic collection and assessment of data
- Reduced duplication of effort at the national level in the re-assessment of value and as such, faster access to medicines for patients.

- In order to coordinate efforts, member states should increasingly collaborate and share their knowledge in preparation for local evidence appraisals.

- A coordinated mechanism should be put in place at the European level to help reduce evidential uncertainties around OMPs and enable rapid and continuous data collection post launch.

- Reimbursement decisions should be maintained at the member state level and should be made at a national level. In those countries with regional or local decision power regarding price and reimbursement, a coordinated approach within the country should be applied in order to avoid variable geographic access to OMPs.

### 6 Conclusion

The principles above aim to provide a high-level framework for European decision makers to help them better capture the specificities of orphan drugs and rare diseases. Decision-makers, such as regulatory bodies, HTA bodies, payers, and manufacturers have a shared responsibility to ensure patient access to OMPs by engaging in discussions in a transparent and fair way. All parties should strive to apply these principles within their national context to provide optimal care for patients with rare diseases, whilst enabling the maintenance of a strong and continued research and innovation level.

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**Organisation support**

The Working Group was provided with organisational support by Dolon, a healthcare consultancy company with specialist expertise in rare diseases.
## Annex: Proposed elements for consideration in P&R frameworks for rare disease treatments

<table>
<thead>
<tr>
<th>At the Patient level</th>
<th>Value of an OMP</th>
<th>Impact of Disease</th>
<th>Impact of Treatment</th>
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<tbody>
<tr>
<td></td>
<td>• Unmet therapeutic need</td>
<td>- Unmet therapeutic need</td>
<td>- Added therapeutic benefit</td>
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<tr>
<td></td>
<td>• Existing treatment options</td>
<td>- Existing treatment options</td>
<td>- Improved survival</td>
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<tr>
<td></td>
<td>• Premature death</td>
<td>- Premature death</td>
<td>- Reduced morbidity</td>
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<tr>
<td></td>
<td>• Morbidity</td>
<td>- Morbidity</td>
<td>- Improvement in quality of life</td>
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<tr>
<td></td>
<td>• Quality of life impairment</td>
<td>- Quality of life impairment</td>
<td>• Side effects</td>
</tr>
<tr>
<td></td>
<td>• Age of onset</td>
<td>- Age of onset</td>
<td>• Treatment convenience</td>
</tr>
<tr>
<td></td>
<td>• Patient economic burden</td>
<td>- Patient economic burden</td>
<td>• Reduction in patient economic burden</td>
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<thead>
<tr>
<th>At the Healthcare system Level</th>
<th>Burden of disease on healthcare system</th>
<th>Impact of treatment on healthcare system</th>
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<tr>
<td></td>
<td>• Burden of disease on healthcare system</td>
<td>• Impact of treatment on healthcare system</td>
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<tr>
<td></td>
<td>• Healthcare system resources and budget</td>
<td>• Burden impact</td>
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<td></td>
<td>• Healthcare system organisation</td>
<td>• Impact on healthcare system organisation</td>
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<tr>
<th>At the Societal level</th>
<th>Burden of disease on carers, family and society</th>
<th>Impact of treatment on carer/family/societal burden</th>
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<td></td>
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<td>• Impact of treatment on carer/family/societal burden</td>
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<tr>
<td></td>
<td>• Quality of life</td>
<td>• Improvement in carer/family quality of life</td>
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<td></td>
<td>• Economic</td>
<td>• Economic benefit</td>
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### Considerations beyond product value

- Rarity
  - Sustainability of rare disease innovation
  - Evidence quality
- Societal preferences
- Priority of disease
- Innovation and research incentives