

Recommendations from the European Working Group for Value Assessment and Funding Processes in Rare Diseases (ORPH-VAL)

In press: Orphanet J of Rare Diseases

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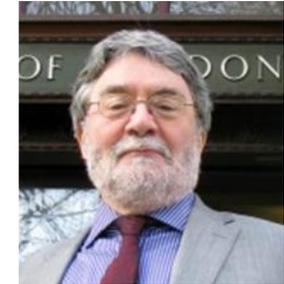
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Challenges in the value assessment and funding processes of OMPs

1. P&R decision criteria

- **Variability of elements** considered within P&R decisions across countries
- **Lack of consideration of value elements** that are particularly important in rare diseases
- **Uncertainty or lack of transparency** about the relative importance of different elements
- **Lack of flexibility** of cost-effectiveness based frameworks

2. P&R decision processes

- **Duplication (and sometimes contradiction)** of assessments made at European level (e.g EMA)
- **Difficulties in interpreting evidence** due to characteristics of rare diseases
- Inconsistent and non-standardised involvement **of rare disease stakeholders**

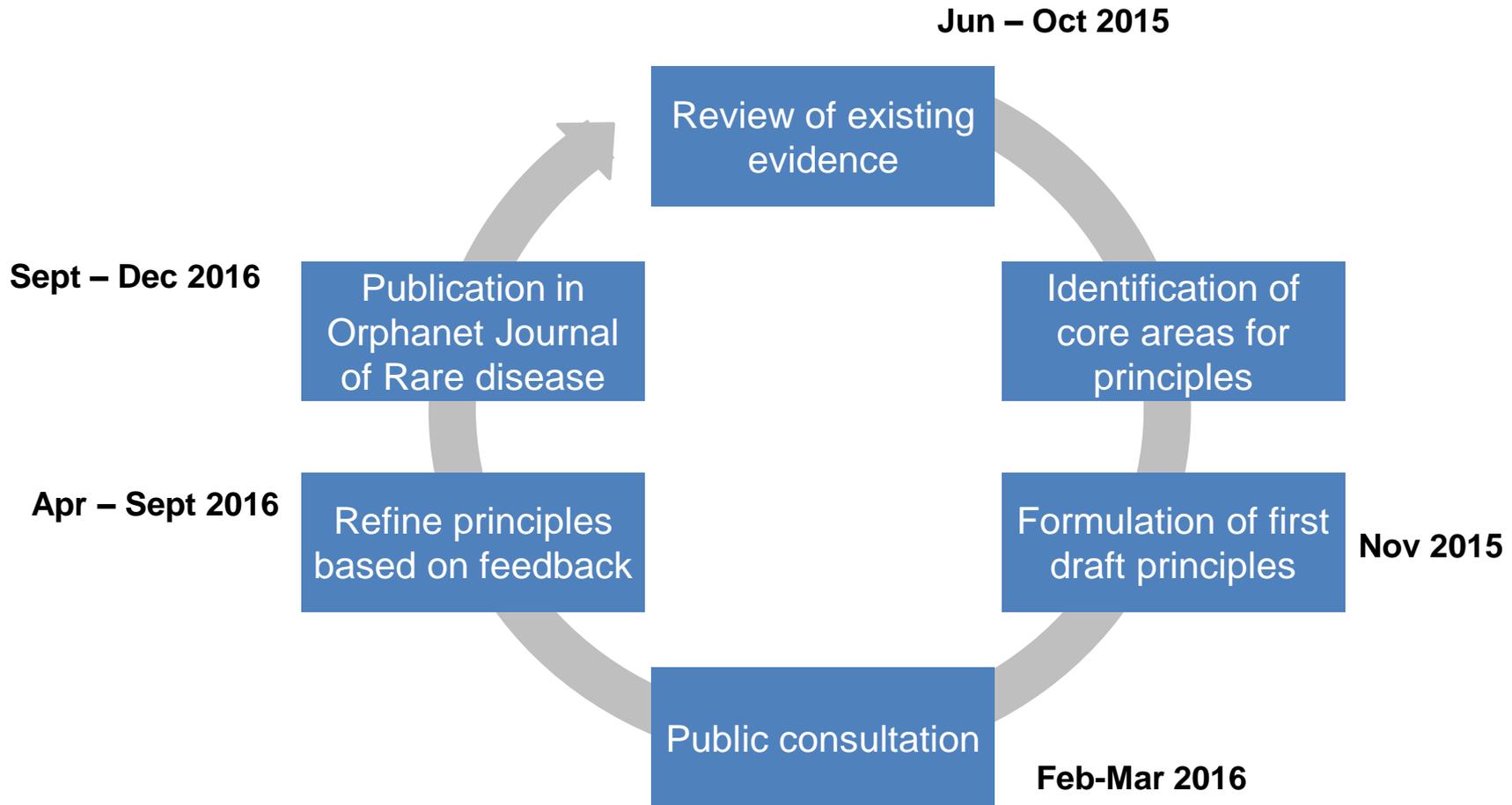
3. Sustainable funding systems

- **Disparities** in access between regions
- Concern about **long-term sustainability** of OMPs on healthcare budgets

4. European collaboration

- **Duplication and inconsistency in evidence generation** at national level
- **Lack of disease-specific knowledge** in every country

Methods for development of principles



What is value?



Value = how much
are we willing to
pay for it?

Value for Money =
is it worth its
price?

definitions ▾ value 

OXFORD DICTIONARY

[**val**-yoo]

Spell Syllables

[Synonyms](#) [Examples](#) [Word Origin](#)

[See more synonyms on Thesaurus.com](#)

noun

1. relative worth, merit, or importance:
the value of a college education; the value of a queen in chess.
2. monetary or material worth, as in commerce or trade:
This piece of land has greatly increased in value.
3. the worth of something in terms of the amount of other things for which it can be exchanged or in terms of some medium of exchange.
4. equivalent worth or return in money, material, services, etc.:
to give value for value received.
5. estimated or assigned worth; valuation:
a painting with a current value of \$500,000.
6. denomination, as of a monetary issue or a postage stamp.

Guide to core elements of value

OMP value		
	DISEASE	TREATMENT
Patient level	Survival/life expectancy; Morbidity	
	Patient experience and quality of life	
	Patient economic burden	
Healthcare system level	Existing treatment options	Side effects
		Convenience
	Healthcare system resources and budget	
Societal level	Healthcare system organisation	
	Family/Carer Quality of life	
	Family/carer economic burden	
	Societal economic burden	

Considerations beyond OMP value

Rarity

- Sustainability of innovation in rare diseases
- Small budget impact

Societal preferences

Uncertainty of OMP value

Quality of evidence

Uncertainty around value parameters

Principle 1 “value”

OMP assessment should consider all relevant elements of product value in an appropriate multi-dimensional framework

- Decision-makers should consider OMP value from the perspective of patients, the healthcare system and wider society
- Set of core elements should be common to all health systems
- HTA agencies and payers should make explicit which elements of value they prioritise, how the rarity of a disease influences their assessment, and how societal preferences are incorporated into their decisions

Principle 2 “value for money”

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

- P&R decisions should reflect the value that the EU attributes to OMPs through the incentives put in place to develop them
- Price should, among other elements, be informed by size of product value in light of price-value precedents (*benchmark*)
- Beyond OMP value, P&R decisions should reflect other considerations, such as societal preferences, rarity, affordability and sustainability of innovation in rare diseases
 - Modulate cost-effectiveness thresholds when applied
 - Balance between incentivising new research investment in rare diseases while maximising value for money for healthcare systems

Principles 3 “no duplication”

Those making P&R decisions about OMPs at a national level should take account of all official regulatory and health technology assessments of OMPs undertaken at the European level

- National P&R agencies should build on 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 and Summary of Product Characteristics
 - Relative effectiveness assessments undertaken by the European network for HTA

Principle 4 “involve expertise”

The assessment and appraisal of OMPs to inform national P&R decisions should incorporate rare disease expertise including both the healthcare professionals’ (HCP) and patients’ perspectives

- HCPs and patients and their carers should be involved in the value assessment in the following ways:
 - Disease-specific expert physicians to be involved in bodies that assess and appraise OMP
 - Systematic representation of patient associations in meetings that assess and appraise OMPs
 - Disease-specific patient representatives should be involved throughout the process and given appropriate training and support to contribute fully

Principle 5 “adaptive processes”

To accommodate uncertainty, value assessment and pricing and reimbursement decisions should be adaptive subject to the need and availability of information over time.

- Given the nature of rare diseases, there is inherent uncertainty around all elements of product value. When assessing value, payers should consider this uncertainty
- To account for clinical and economic uncertainty, value assessment processes need to be adaptive (i.e. contingent), where necessary, and continuous rather than binary

Principle 5 (continued)

- Where adaptive processes are required, all parties (payers, HTA agencies, involved HCPs, patients and industry) need to agree on this iterative process and clearly document :
 - the evidence required and milestones for each step of the assessment
 - the implications of not meeting the requirements and expectations initially agreed
 - each stakeholder's shared responsibility to collect and evaluate the data
- Where possible, the collection and analysis of real-world data should be co-ordinated at a European or international level and should be integrated in disease level registries and databases:
 - obtain more European consistency in the continuous assessment and appraisal of OMPs
 - to collect data on the true prevalence of a given rare

Principle 6 “eligible patients”

All eligible patients within the authorised label of an OMP should be considered in the national P&R decision although different decisions on access may apply to different sub-populations

- Wherever possible, reimbursement decisions should seek to ensure that all patients specified in the product marketing authorisation should receive access to treatment
- Reimbursement may be reflective of situations where there is a broad spectrum of disease and clearly defined patient subgroups in which OMP value substantially differs

Principle 7 “national level funding”

Funding should be provided at the national level to ensure patient access to OMPs

- Funding for OMPs should be co-ordinated at a national level in order to avoid disparities in access between regions and to pool the financial risk of irregular distribution of patients
- 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

Principle 8 “long term funding”

Evidence-based funding mechanisms should be developed to guarantee long-term sustainability

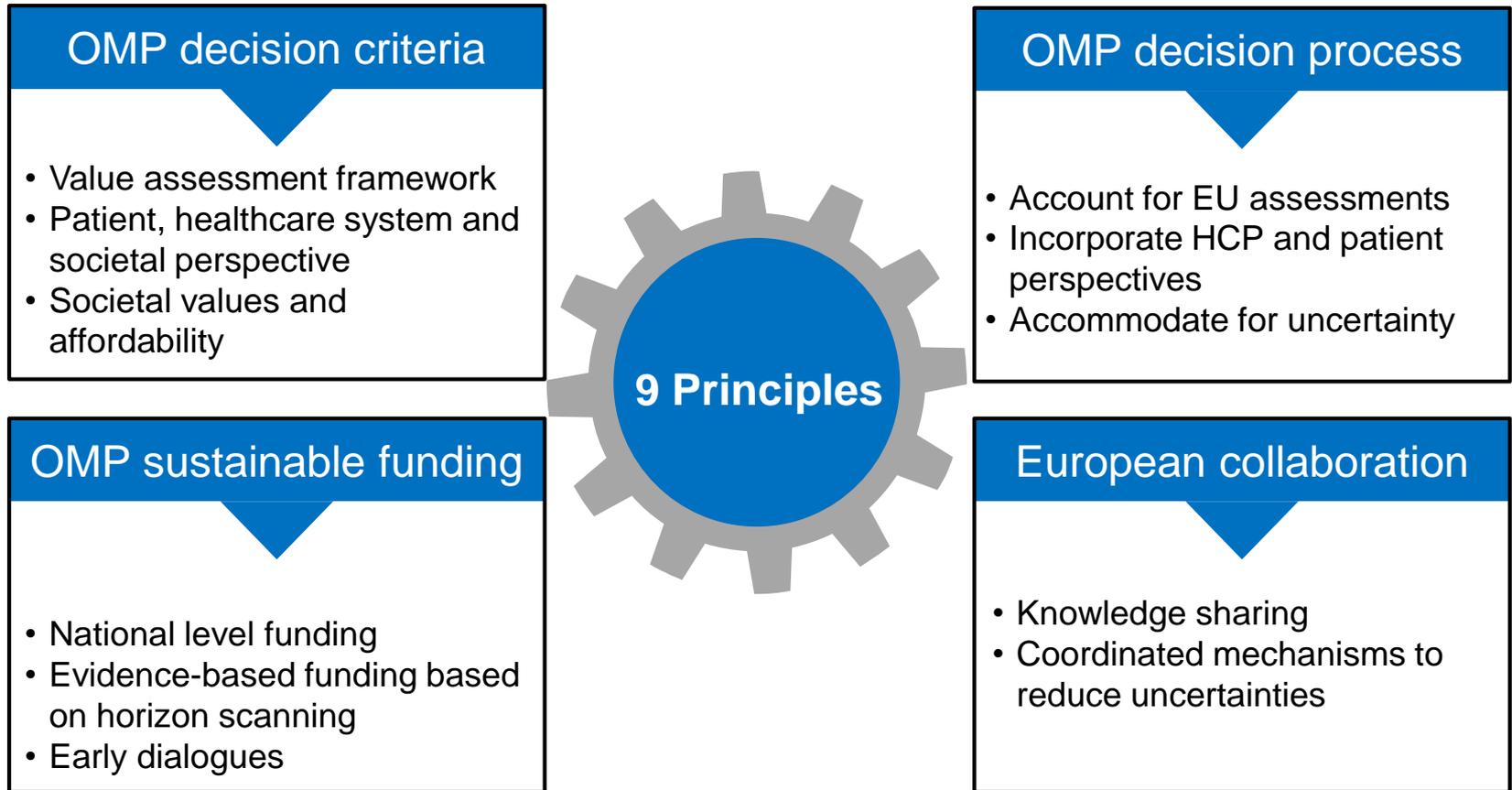
- Manufacturers, payers and HTA agencies should collaborate nationally to improve forecasting and cooperate at the European level for horizon scanning with the aim of helping budget holders predict and plan for expenditure and ensure adequate funding of OMPs
- Early stage dialogue should occur between all stakeholders to ensure long term sustainability of outcomes

Principle 9 “co-ordination”

In the future there should be greater co-ordination of OMP value assessment processes at a European level

- Greater role for co-ordination of certain elements of value assessment in the future at EU level. Rationale:
 - Guarantee more consistency between Member States in the definition and assessment of clinical value
 - Greater concentration of clinical expertise
 - 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
- Member States should increasingly collaborate and share their knowledge in preparation for local evidence appraisals
- A co-ordinated 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

Overview of Principles



The Principles are a set of recommendations that seek to improve the consistency of value assessment and funding decisions for OMPs across Europe

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