Towards a common European Value & Funding System for Orphan Drugs: implementing the ORPH-VAL Principles

EURORDIS 2nd Multi-stakeholder Symposium

23rd February 2017

Meeting Agenda

Time	Agenda	Lead
9.00 - 9.30	**Introduction to session and principles**	**LA**
9.30 - 10.00	Review of principles vs current P&R systems in three countries: France, Germany, UK	AH
10.00 - 10.30	Panel discussion	LA / Panellists
10.30 - 11.00	Discussion with the audience	All

Workshop Objective and Participants

Objectives

- Introduce ORPH-VAL principles on value assessment and funding for OMPs (In Press)
- Explore where existing European P&R systems are aligned/misaligned with the principles
- Discuss ways to improve alignment and how to utilise the principles to enact change

Participants

- Lieven Annemans, Professor of health economics, Ghent University, Belgium (Chair)
- Ruediger Gatermann, Director Healthcare Policy and External Affairs, CSL Behring (Rapporteur)
- Michael Schlander, Chairman and Scientific Director, Institute for Innovation and Value in Healthcare, Germany
- Chris Sotirelis, Trustee Advisor, UK Thalassemia Society
- Adam Hutchings, Director, Dolon Ltd
- **YOU!**

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

In press: Orphanet Journal of Rare Diseases

Members of the Working Group

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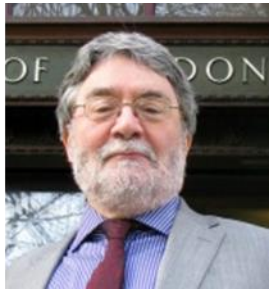

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Challenges in the value assessment and funding processes of OMPs

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**

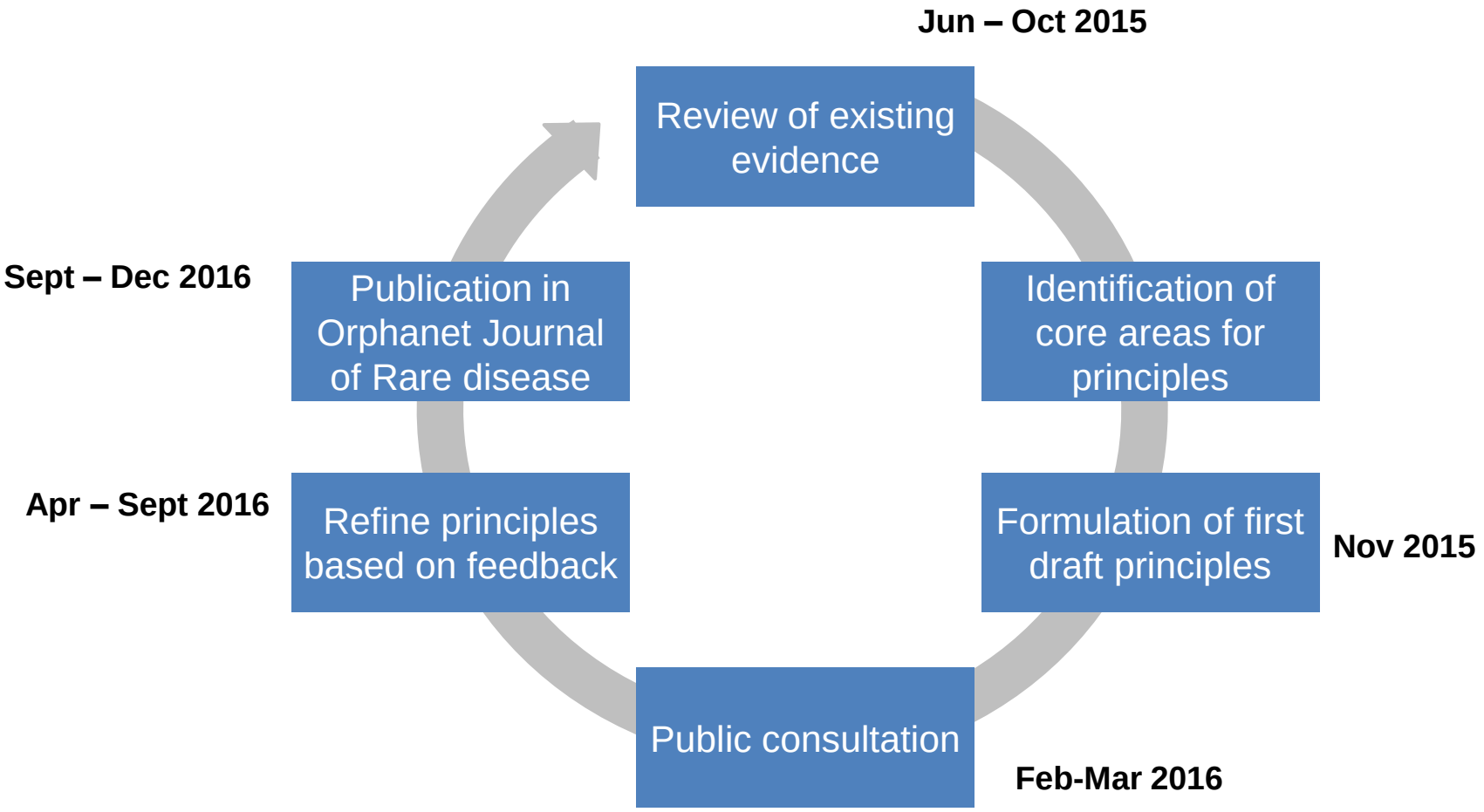
3. Sustainable funding systems

- **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?

OXFORD DICTIONARY

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

Value for Money = is it worth its price?

[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	
	Existing treatment options	Side effects Convenience
Healthcare system level	Healthcare system resources and budget Healthcare system organisation	
Societal level	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

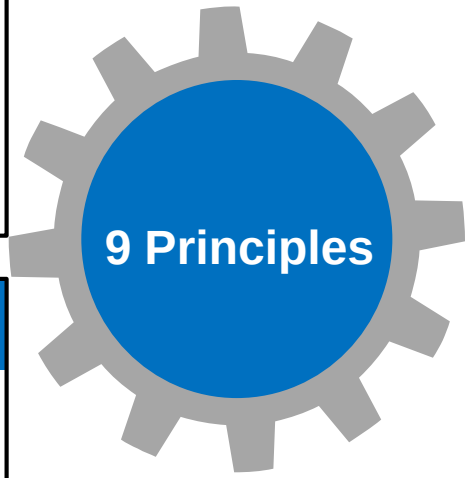
Overview of Principles

OMP decision criteria

- Value assessment framework
- Patient, healthcare system and societal perspective
- Societal values and affordability

OMP decision process

- Account for EU assessments
- Incorporate HCP and patient perspectives
- Accommodate for uncertainty



OMP sustainable funding

- National level funding
- Evidence-based funding based on horizon scanning
- Early dialogues

European collaboration

- Knowledge sharing
- Coordinated mechanisms to reduce uncertainties

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

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

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9.30 - 10.00	**Review of principles vs current P&R systems in three countries: France, Germany, UK**	**AH**
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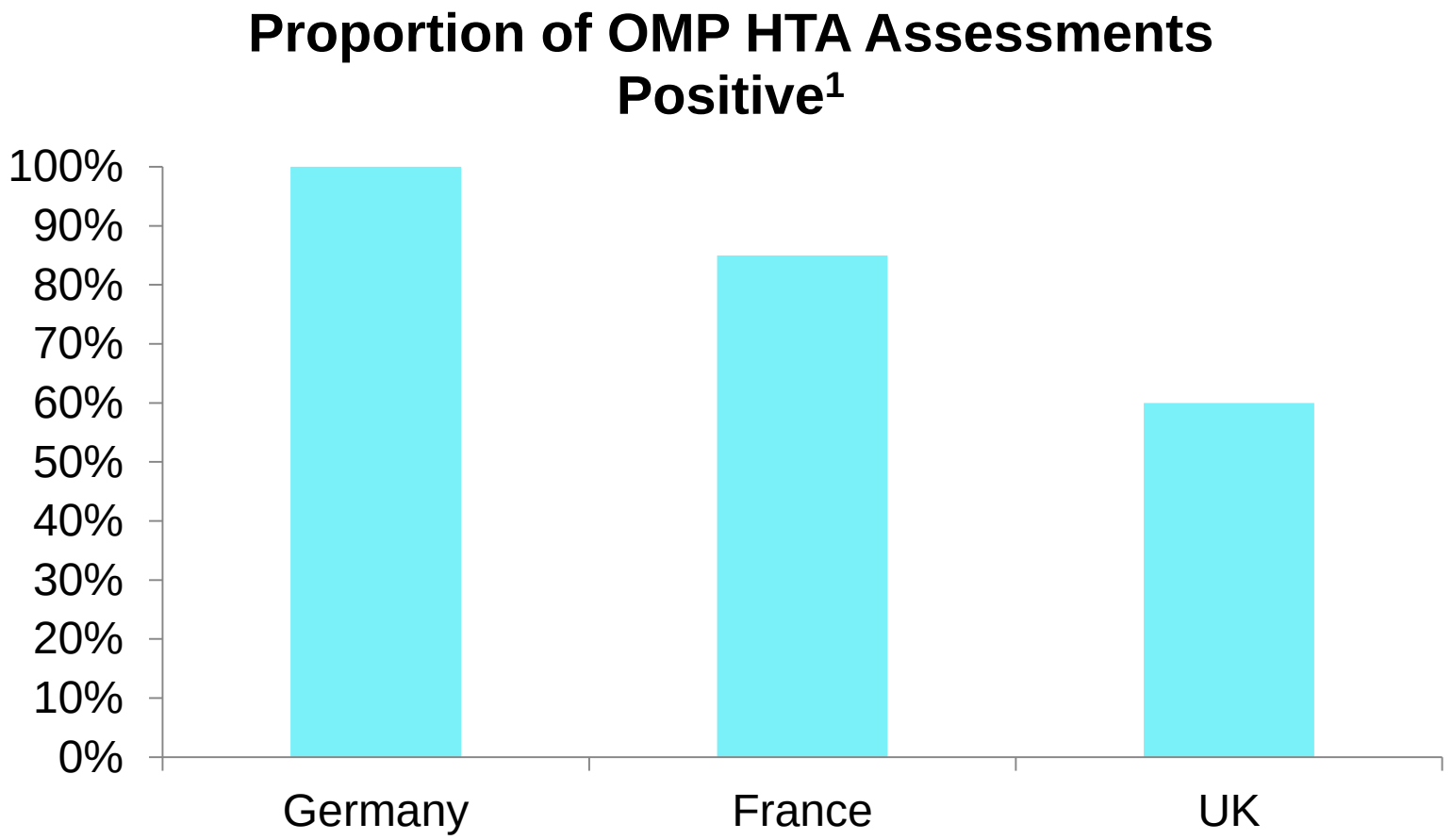
What is patient access to OMPs like in each country?



1. Kawalec 2016.
Note: Estimates of OMP access are complex and vary between sources

Comparing the principles against existing payer systems*

Principles	Sub-principles	FR	DE	UK
Principle 1: OMP assessment should consider all relevant elements of product value for OMPs in an appropriate multi-dimensional framework	OMP value from the perspective of patients, the healthcare system and wider society?			
	Core elements of value considered?			
	Societal values underpinning value assessment are explicit?			
	Use of multi-criteria decision analytic (MCDA) approach?			
Principle 2: Pricing and reimbursement decisions should be founded on the assessment of OMP value for money and adjusted to reflect other considerations beyond product value	Reimbursement decisions based on product value?			
	Price informed by price-value precedents for other specialist medicines?			
	P&R status modulated to reflect other rare disease considerations (e.g budget impact)?			
	ICER thresholds should be modulated to reflect specificities of rare diseases?		N/A	
	Balances incentives for new research investment in rare diseases while maximising value for money for healthcare systems			

*Subjective assessment of the author – for discussion only

Caption	Mostly aligns with principle	Somewhat aligns	Not closely aligned

Comparing the principles against existing payer systems

Principles	Sub-principles	FR	DE	UK
Principle 3: All official regulatory and health technology assessments of OMPs undertaken at the European level should be acknowledged by national health authorities	Assessment builds on the decisions and recommendations at a European level			
Principle 4: The assessment and appraisal of OMPs in Europe should incorporate rare disease expertise including both the healthcare professionals' and patients' perspectives	HCPs and patients and their carers should be involved in the value assessment in the following ways: - Disease-specific expert physicians provide direct input - 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			

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Comparing the principles against existing payer systems

Principles	Sub-principles	FR	DE	UK
Principle 5: To accommodate uncertainty, value assessment and pricing and reimbursement decisions should be adaptive subject to the need and availability of information over time	Consider uncertainty in light of disease prevalence, disease severity and unmet need, prior research conducted in the disease,			
	Value assessment processes should be adaptive and continuous			
	P&R decisions should allow movement both up and down with newly generated evidence on value			
	Where adaptive processes are required, all parties (payers, HTA agencies, involved HCP, patients and industry) involved			
	Collection and analysis of real-world data should be co-ordinated at a European or international level			
Principle 6: All eligible patients within the authorised label of an OMP should be considered in the reimbursement appraisal although different decisions on access may apply to different sub-populations	Reimbursement decisions should seek to ensure that all patients specified in the product license 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			

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Comparing the principles against existing payer systems

Principles	Sub-principles	FR	DE	UK
Principle 7: 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			
	Funding for OMPs should come out of normal healthcare budgets rather than from ear-marked rare disease funds			
Principle 8: Evidence-based funding mechanisms should be developed to guarantee long-term sustainability	Manufacturers, payers and HTA agencies should collaborate nationally to improve forecasting of OMP expenditure			
	Early stage dialog between all stakeholders should be put in place to ensure long term sustainability of outcomes			
Principle 9: In the future there should be greater co-ordination of OMP value assessment processes at a European level	Collaborate with other European payers in regard to value assessment and data generation			

*Subjective assessment of the author – for discussion only

Caption	Mostly aligns with principle	Somewhat aligns	Not closely aligned

Increasing Alignment – Germany*

1. *'Broader definition of value'*

Greater emphasis on patient quality of life, carer/family burden, wider societal value

2. *'Recognise implications of rarity for evidence generation'*

Recognise the need for consideration of evidence generation challenges when assessing treatments for rare diseases – currently many products get 'unquantifiable' benefit due to trial design, endpoint or comparator

3. *'Greater use of adaptive processes'*

Create processes to allow for more adaptive P&R decisions with evidence generation requirements

*These proposals are for the purpose of creating discussion

Increasing Alignment – UK*

1. *'One process for ALL OMPs'*

More alignment between NICE HST and STA processes. Currently OMPs are assessed through both processes . HST is reflective of the specificities of rare diseases and OMPs, STA is not.

2. *'Flexible ICER thresholds'*

Have a flexible ICER threshold that could be modulated to reflect the specificities of diseases under consideration, including those aspects that are important in rare diseases

3. *'Strike the balance'*

Balance incentives for new research investment in rare diseases while maximising value for money for healthcare system

*These proposals are for the purpose of creating discussion

Increasing Alignment – France*

1. *'Greater patient voice'*

Strengthen/increase patient representatives voice and input during the decision making process, for example by formally including a patient/carer perspective form as part of submissions

2. *'Broader definition of value'*

Greater emphasis on patient quality of life, carer/family burden, wider societal value

3. *'Transparent societal preferences'*

Increase transparency of societal preferences behind reimbursement and pricing decision criteria

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Panel discussion

- Do you agree with the assessment of FR, DE, UK vs principles?
- Any additional/alternative proposed changes to improve alignment?
- Do you think these proposed changes are feasible?
- What are the pathways to implementation and potential hurdles?

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Audience questions

- What is the most important change for each market to further alignment?

	Germany	UK	France
1	‘Broader definition of value’	‘One process for ALL OMPs’	‘Greater patient voice’
2	‘Recognise implications of rarity for evidence generation’	‘Flexible ICER thresholds’	‘Broader definition of value’
3	‘Greater use of adaptive processes’	‘Strike the balance’	‘Transparent societal preferences’