Incorporating patient perspective in funding decisions for rare disease treatments: a review of international payer systems

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Background and Objectives

- Healthcare payers are increasingly aware of the need to engage patient advocacy groups in the reimbursement process of rare disease treatments.
- The limited published information on the natural history of the disease, the symptomatology and the quality of life of patients and their families are common data gaps. In addition, the lack of understanding of the endpoints used in clinical trials and the uncertainty around the clinical evidence make their role crucial.
- Their real-life experiences help decision-makers to fully understand the disease burden, the costs that the disease imposes on wider society and the benefit of a treatment.
- The aim of this study was to investigate the extent to which pricing and reimbursement systems allow patient advocacy groups to get involved in current assessment processes for orphan treatments internationally and provide recommendations.

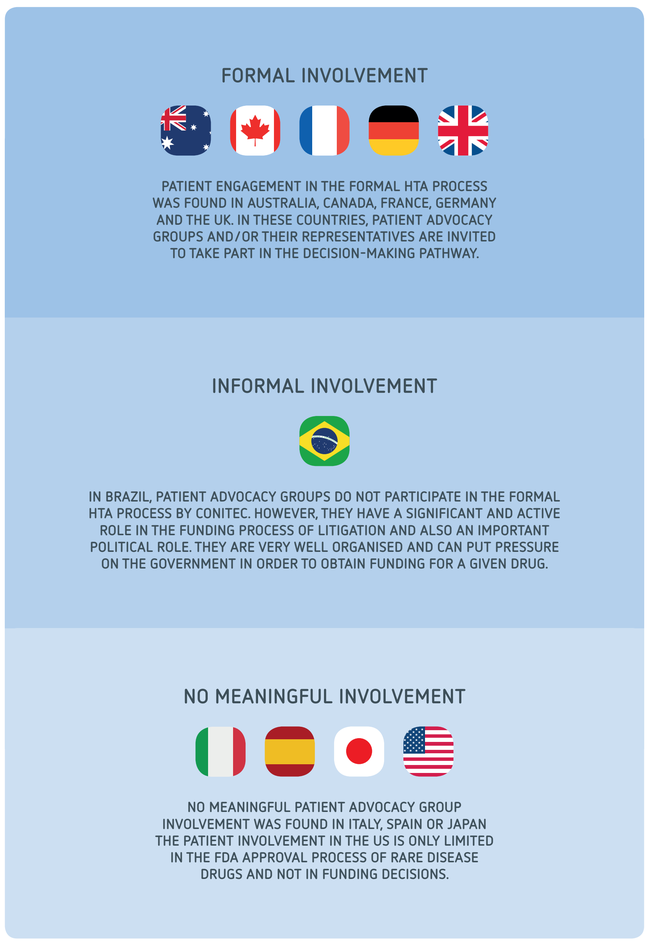
Methods

- Insight was obtained through a review of international pricing and reimbursement systems for orphan medicines and interviews with patient associations, payers and pharmaceutical industry representatives. Ten countries were selected to capture patient involvement in funding pathways in an international spectrum.

Results

In our analysis, 5 EU countries (France, Germany, Italy, Spain and the UK – England and Scotland), Australia, Brazil, Canada, Japan and the US were included. It was found that the extent of patient engagement in funding processes is highly variable internationally (Figure 1).

Figure 1.
Patient involvement in orphan drugs funding decisions across countries



Formal patient involvement in payer processes most frequently comprises of written submissions or patient representation in decision committees (Figure 2). In the UK, both England and Scotland that have rare-disease-specific reimbursement pathways tend to have more systematic and comprehensive patient involvement. Specifically, the Highly Specialised Technologies Programme (HST) in England and the Patient and Clinician Engagement (PACE) in Scotland involve patient groups to a great extent as part of the assessment process for ultra-rare disease medicines In all other countries there is no specific reimbursement process for orphan drugs and the role of patient advocacy groups is restricted to specific tasks across the funding pathway.

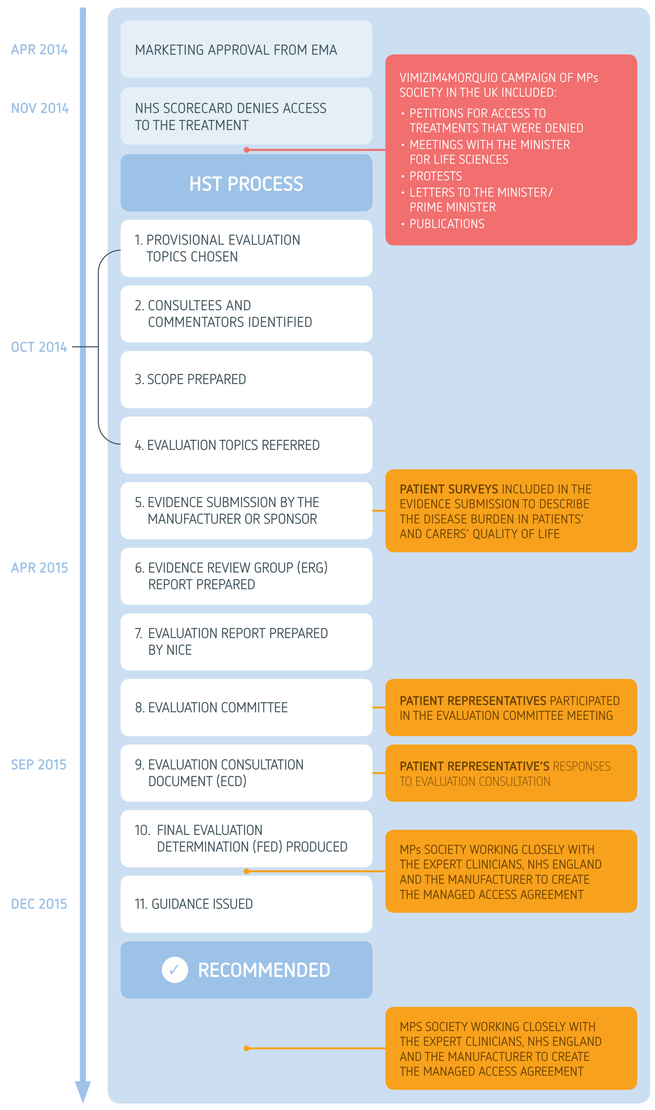
Figure 2. Patient involvement in different stages of the HTA process across countries

	DISEASE BURDEN KNOWLEDGE		
	PATIENTS' INPUT		
	PATIENT GROUP WRITTEN SUBMISSION	PATIENT HEARINGS PRIOR TO THE MEETING	ATTENDING THE DECISION MEETING
AUSTRALIA	✓	✓	✗
CANADA	✓	✗	✗
FRANCE	✗	✗	✗
GERMANY	✓	✗	✓
ENGLAND (UK)	✓	✗	✓
SCOTLAND (UK)	✓	✗	✓

	DECISION MAKING			
	INVOLVEMENT IN THE DECISION COMMITTEE		INVOLVED IN NEGOTIATIONS (ONLY IN RESPECT OF AGREEING MANAGED ACCESS AGREEMENTS)	INVOLVED IN IMPLEMENTATION OF THE DECISION
	PATIENT/CONSUMER REPRESENTATIVE	RIGHT TO VOTE		
AUSTRALIA	✓	✗	✗	✗
CANADA	✗	✗	✗	✗
FRANCE	✓	✓	✗	✗
GERMANY	✓	✗	✗	✗
ENGLAND (UK)	✓	✓	✓	✓
SCOTLAND (UK)	✓	✗	✗	✗

The case of the reimbursement of elosulfase alfa for the MPS IVA under the HST in England proves that patient advocacy groups can contribute significantly in the different stages of the HTA process, potentially leading to a positive decision. The MPS society in the UK was strongly involved, not only in the reimbursement process, but also in the implementation of the decision by working with the manufacturer of the drug on the Managed Access Programme (Figure 3).

Figure 3. Elosulfase alfa timeline and patient engagement in the HST process[1]



Recommendations

Patient engagement has been seen to improve the quality of funding decision making, particularly in respect to improving prevalence estimates, interpreting the relevance of clinical trial endpoints and establishing the impact of the disease (and treatment) on the lives of patients and their families.

However, the effectiveness and the value of patient involvement can be improved further through:

- Education of patient advocacy groups on payer systems
- Experience-sharing with other patient associations
- The use of patient surveys to provide insight into the disease impact
- Systematic representation in the HTA bodies that assess orphan drugs of an overarching patient association

Conclusions

There is a clear need for more systematic patient involvement in funding decisions for rare disease treatments. Although the extent to which the patient input influences these decisions may vary between countries, patient representatives (and individual patients) have a pivotal role to play in the understanding of their diseases and the interpretation of the benefit of new treatments. Payer decision bodies should incorporate both general rare disease patient representation and disease-specific patient input.

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References: *1) National Institute for Health and Care Excellence, 2016. Available at: https://www.nice.org.uk/guidance/hst2/history 2) Society for Mucopolysaccharide Diseases (MPS Society), UK, 2016. Available at: http://www.mpssociety.org.uk/en/support/current-campaigns/current-campaigns-our-fight-for-vimizim/whats-going-on-with-vimizim/*