university of groningen

The health economics of orphan drugs: Testing a MCDA framework to enhance patient access

9th European Patients' Rights Day, Brussels, May 2015

Carina Schey, Unit of Pharmacoepidemiology and Pharmacoeconomics

PhD Researcher

Supervisor: Prof. Dr. Maarten Postma

Health Economist at Global Market Access Solutions

GMAS Global Market Access Solutions

Background

- Increased demand on healthcare results in cost containment initiatives
- Current health technology appraisals might disregard several key disease attributes
- Orphan drugs are unlikely to achieve “accepted” cost/ QALY
- **Results in poor access to orphan drugs in many countries**
- New methods may facilitate reimbursement decision making which will enhance the access by patients to life-sustaining drugs

Aims

- To develop a multi-criteria decision analysis model based on the criteria and framework suggested by Hughes-Wilson *et al*, 2012 (1)
- To assess the:
  - Proposed criteria versus criteria highlighted in a literature review
  - Outcomes of the MCDA

1. Hughes-Wilson W, et al. Paying for the Orphan Drug System: break or bend? Is it time for a new evaluation system for payers in Europe to take account of new rare disease treatments? Orphanet Journal of Rare Diseases. 2012, 7: 74.

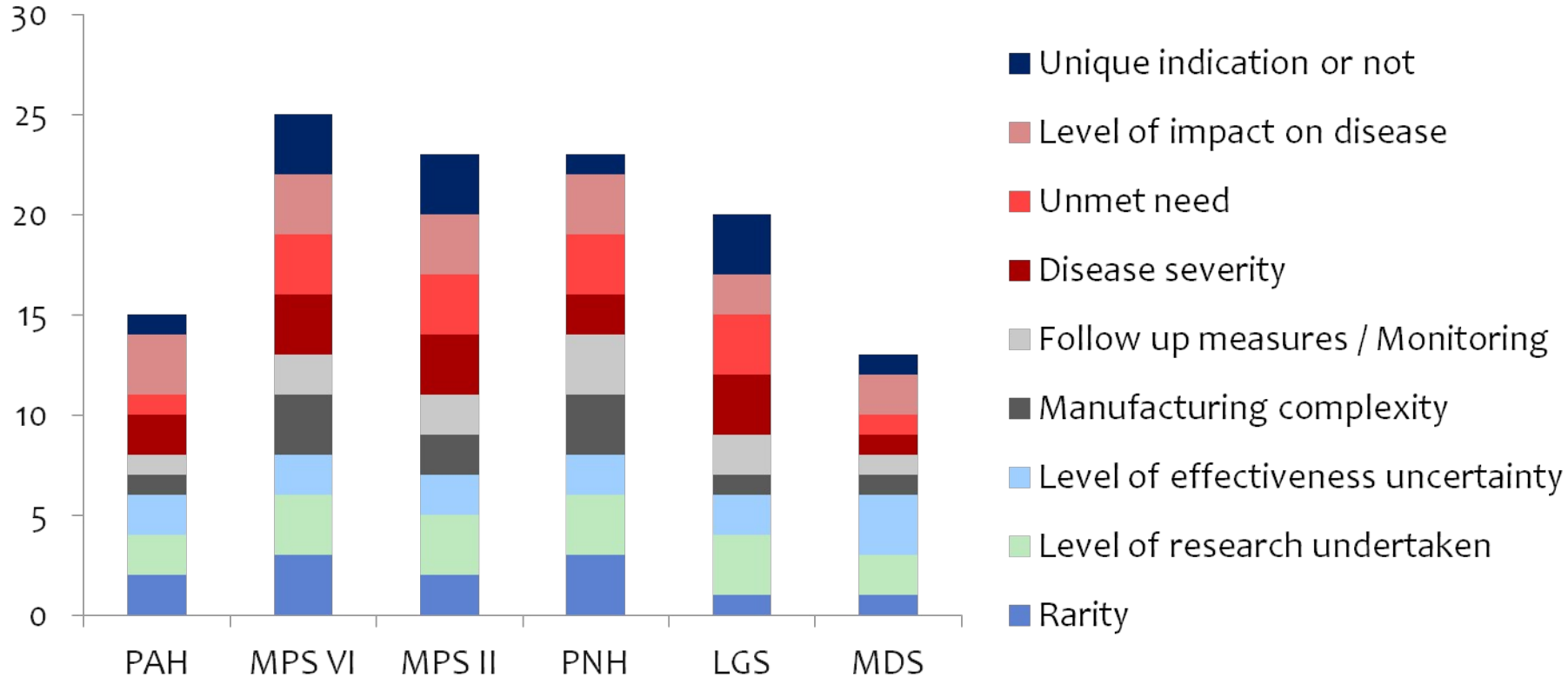
Methods

- Conducted a literature review
- Developed the MCDA framework
- Selection of study drugs
- Cost calculations
- Developed “scoring” system for different attributes
- Collected data for each criterion for each drug

The proposed criteria

- Disease severity
- Level of impact on disease
- Level of research undertaken
- Unmet need
- Manufacturing complexity
- Follow up measures
- Level of effectiveness uncertainty
- Disease rarity
- Uniqueness of the indication

Results: Individual criteria in relation to the overall drug score



PAH – Pulmonary arterial hypertension

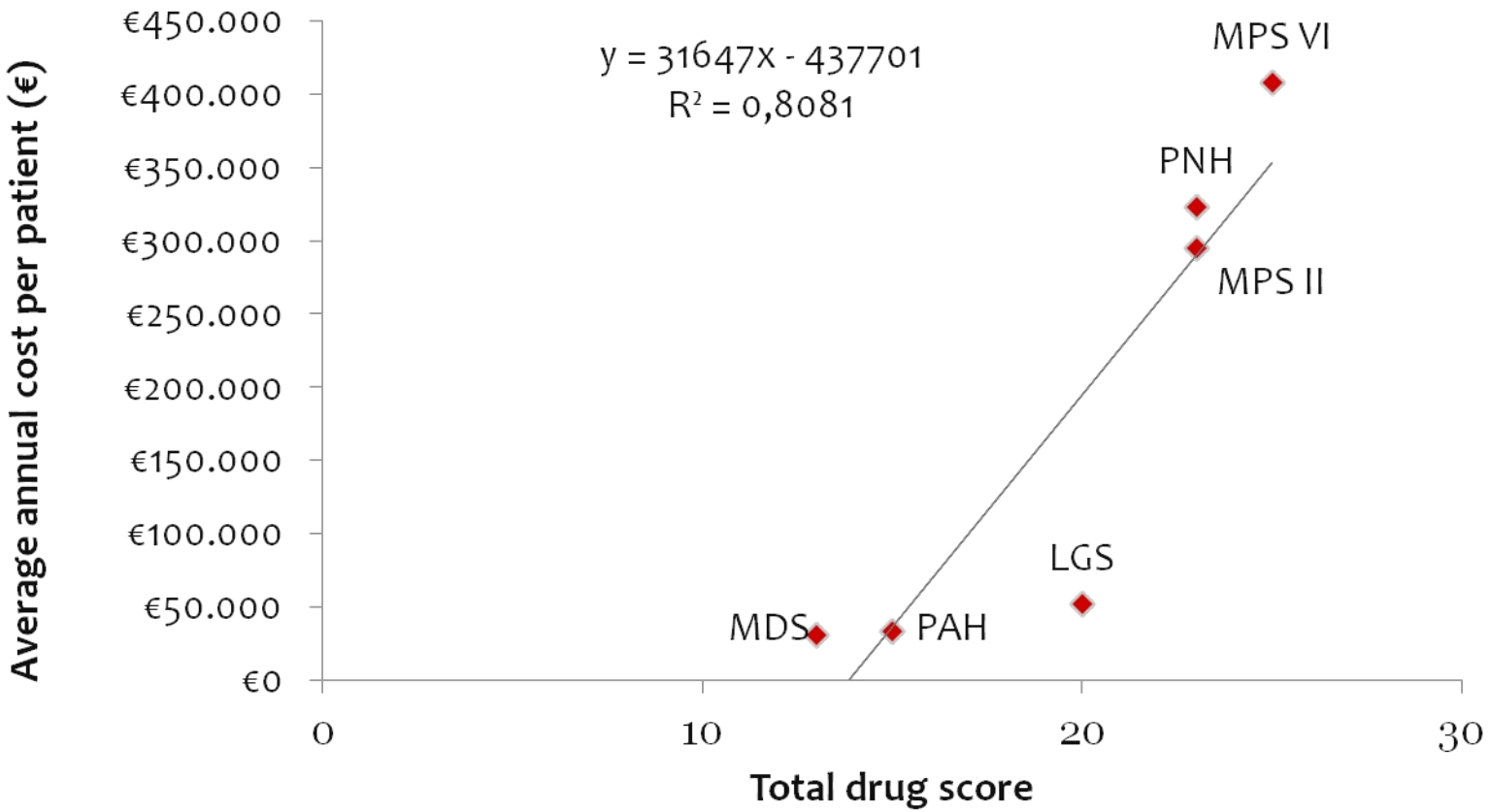
MPS II & VI – Mucopolysaccharidosis II & VI

PNH – Paroxysmal nocturnal haemoglobinuria Syndrome

LGS – Lennox-Gastaut

MDS – Myelodysplastic syndromes

Results: Average annual cost in relation to the total drug score



PAH – Pulmonary arterial hypertension

MPS II & VI – Mucopolysaccharidosis II & VI

PNH – Paroxysmal nocturnal haemoglobinuria Syndrome

LGS – Lennox-Gastaut

MDS – Myelodysplastic syndromes

Value of MCDA

- Rational and transparent
- Includes a wide range of attributes
- Robust assessment
- Facilitates within-therapy and across-therapy comparisons
- Supports decision making
- **Demonstrates true drug benefit and enhances patient access where there is huge unmet need**

Acknowledgements

- University of Groningen