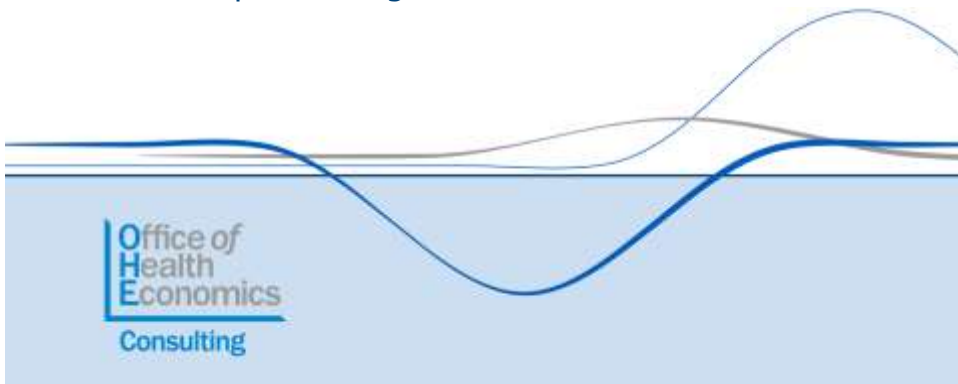


Sustainable funding and fair pricing for orphan drugs. What are the solutions?

ISPOR European Congress 2017



Introduction



- Ongoing debate on sustainable funding of orphan drugs
- The evidence in support of paying a premium for orphan drugs is mixed
 - However, many countries in Europe provide access
- The aim of this workshop is to discuss options
 - To make the funding of valuable orphan drugs sustainable for healthcare systems
 - To provide a 'fair' reward to manufacturers investing in areas of high unmet need

Structure of the workshop



- Martina Garau
Rate of HTA approval/reimbursement of orphan drugs in EU
- Saskia Knies
Insights and learnings from the Dutch approach
- Mike Drummond
New method to adjust the cost effectiveness threshold to reflect the different population sizes
- Olivier Ponet
New approach to improve access to orphan drugs

Definitions



- Orphan drugs prevalence: no more than 5 in 10,000 patients (EMA eligible criteria)
- Ultra-orphan drugs prevalence: less than 1 in 50,000 (NICE, 2004)

Voting



1. Do you agree that there should be a higher cost effectiveness threshold for orphan drugs compared to that used to appraise treatments for common conditions?
2. If you agree, on which basis the threshold should be adjusted?
3. If adjustments to the threshold are possible, should decision makers distinguish between orphans and ultra-orphans?
4. Do you agree that the evidence requirements for orphan drugs should be different (or less stringent) than those expected for common conditions?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Do you agree that there should be a higher cost effectiveness threshold for orphan drugs compared to that used to appraise treatments for common conditions?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: If you agree, on which basis the threshold should be adjusted?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: If adjustments to the threshold are possible, should decision makers distinguish between orphans and ultra-orphans?

Live Content Slide

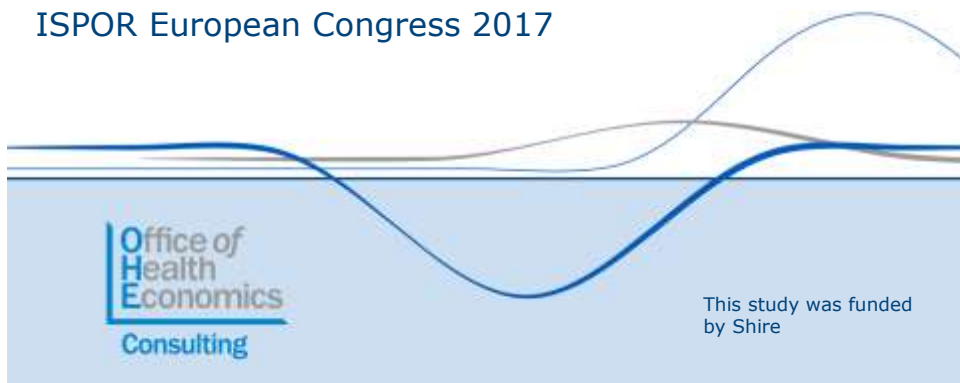
When playing as a slideshow, this slide will display live content

Poll: Do you agree that the evidence requirements for orphan drugs should be different (or less stringent) than those expected for common conditions?

Comparing HTA approval and reimbursement of orphan medicinal products (OMPs) in EU

Zamora, B., Maignen, F., O'Neill, P., Mestre-Ferrandiz, J. and **Garau, M.**

ISPOR European Congress 2017



Objectives



- To compare the *availability* of and *access* to OMPs in the UK (England, Scotland and Wales), France, Germany, Italy and Spain
- To compare the speed of access to OMPs in the selected countries
 - months between the marketing authorisation and the decision to *recommendation/reimbursement* in each countries.

Methods



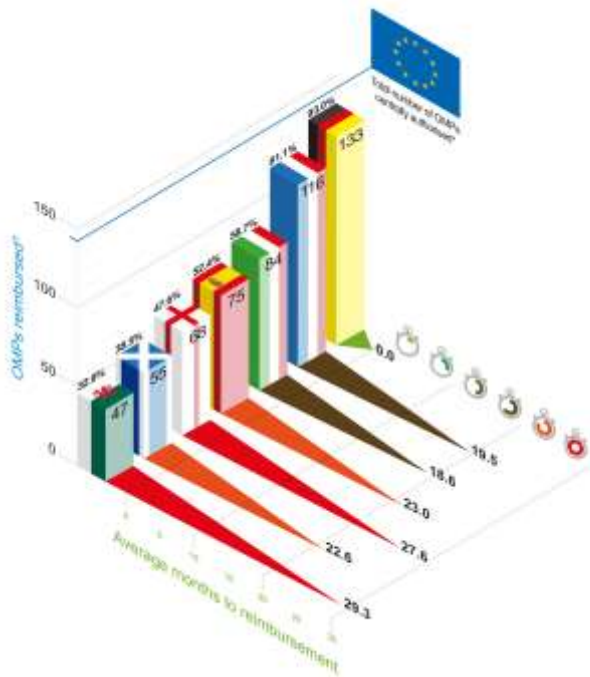
- Data from the European Medicines Agency's (EMA) and DG Health and Food Safety's websites on medicinal products with an orphan designation and marketing authorisation
- Time period covered from 2000 to June 2016, i.e. from inception of Regulation (EC) No 141/2000, to May 2016
- Data on these OMPs were collected concerning
 - Health Technology Assessment (HTA), funding or commissioning, and/or reimbursement decisions
 - Date of publication of these decisions
- Systematic and consistent approach to data extraction

Results - Orphan designations and marketing authorisations



	2001-2005	2006-2010	2011	2012	2013	2014	2015	2016 (Jan-May)	Total
Number of OMPs designations	173	355	86	118	124	182	185	137	1,360
Number of OMPs authorised	22	45	7	12	10	17	20	10	143
Average months from designation to authorisation	72.7	54.0	47.1	29.4	19.9	15.6	n/a	n/a	54.7

- EC has granted 1,360 orphan designations, of which 143 (10.5%) have obtained a marketing authorisation
- There has been an increase in both orphan designations and central marketing authorisations



Results - Access to OMPs across EU countries

Notes on the previous slide



* 143 OMPs obtained a marketing authorisation since the implementation of the EU Regulation on Orphan Medicines (Regulation (EC) No 141/2000)

† OMPs reimbursed refers to Health Technology Assessment (HTA) recommendations to use or inclusion in reimbursement lists in respective national health systems.

Limitations



- Country comparisons need to be interpreted with caution due to different national regulations and procedures, and the heterogeneous information publicly available
- There are mechanisms ensuring access to OMPs (e.g. compassionate programmes or individual patient funding requests)
- HTA recommendations might not necessarily lead to fast access/impede the possibility of prescribing OMPs in practice
- Time to access new OMPs is affected by numerous factors
- Only 5 countries of the 28 EU member states were included
- More research is required to estimate uptake of OMPs in clinical practice

Summary



- There is still considerable variation in funding and provision of OMPs across EU countries
- The 143 OMPs are most widely accessible in Germany and France
- In England, Italy, Scotland, Spain, and Wales between 30% and 60% of OMPs are reimbursed
 - In England, less than 50% of OMPs are routinely funded by the NHS, with one-third of these recommended by NICE
- In Germany reimbursement is automatically granted to OMPs
- The shortest time from authorisation to a reimbursement decision is in France and Italy (19 months on average), the longest is in England and Wales (28.5 months on average)

Thank you!



To enquire about additional information and analyses, please contact Martina Garau at mgarau@ohe.org

The slides were based on:

Zamora, B., Maignen, F., O'Neill, P., Mestre-Ferrandiz, J. and Garau, M. (2017). Comparing Access to Orphan Medicinal Products (OMPs) in the United Kingdom and other European countries. OHE Consulting. Available at:

<https://www.ohe.org/publications/comparing-access-orphan-medicinal-products-omps-united-kingdom-and-other-european>

To keep up with the latest news and research, subscribe to our blog, [OHE News](#)
Follow us on Twitter [@OHENews](#), [LinkedIn](#) and [SlideShare](#)

Key definitions



- **Availability**

Possibility that an OMP can be prescribed within the national health system and dispensed in pharmacies or hospitals

- **Access**

Health Technology Assessment (HTA) recommendations to use or inclusion in reimbursement lists in national health systems