

life-threatening conditions. Marketing authorisation of orphan medicinal products (OMPs) by the EMA is only the first step, as medicines are made available to patients when reimbursement or Health Technology Assessment (HTA) decisions are implemented by national health systems. We analyzed the availability and access to OMPs in the United Kingdom (UK), France, Germany, Italy and Spain. We compared the availability, which is the possibility to prescribe a given OMP, to the access, which refers to the full or partial reimbursement by the public health service.

METHODS:

We collected data on launches, HTA decisions, any centralized commissioning and/or reimbursement decision for all the OMPs authorised since 2000 in the UK countries (England, Scotland and Wales), France, Germany, Italy and Spain.

RESULTS:

Since the Orphan Regulation inception, the EMA granted marketing authorization to 143 OMPs. These OMPs are most widely accessible in Germany and France. Reimbursement in Germany is immediate after authorization. France and Italy present a delay of 19 months from authorization to reimbursement, which is shorter than in other countries. In England, less than 50 percent of centrally authorised OMPs are routinely funded by the National Health Service (NHS), including one-third of these recommended by the National Institute for Health and Care Excellence (NICE), and those reimbursed via commissioning policies and the Cancer Drugs Fund.

CONCLUSIONS:

The assessment of degree of access to OMPs across Europe is limited by differences in the national HTA and reimbursement systems and the heterogeneous information made publicly available on their decisions. Nonetheless, our study suggests that the primary purpose to grant equal availability to OMPs to the patients in the European Union via the implementation of the orphan regulation was partially achieved with

important variations of access observed across the countries included in our study.

OP141 Patient Relevant Outcome Measures As Predictors Of Healthcare Use In Multiple Sclerosis

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INTRODUCTION:

Multiple-sclerosis (MS) is a highly disabling chronic disorder affecting young adults with long term economic consequences on society that escalate as MS disability increases (1,2). In the long-term, progression of MS results in increased level of disability and most patients will eventually experience some degree of functional impairment of the nervous system that impacts on mobility as well as sensory and coordination issues, bladder and sexual functioning, and mood and cognition (2). This is usually accompanied by a deterioration of their quality of life. Patient relevant outcome measures (PROMS) are largely used to measure individual disability, and quality of life in MS (2). International evidence from the International Multiple Sclerosis Study (IMPrESS) (2) was used to quantify the relationship between healthcare resources utilisation and disability, quality of life in individuals with MS.

METHODS:

Multivariable logistic regression was performed in order to identify patient-related variables reporting disability (Barthel) and utility (EQ-5D) that predict use of healthcare services (visits to GP, specialists, nurses, hospitalisation and treatment) and work limitation within the participants of the IMPrESS.

RESULTS:

Responses were collected from 1,152 individuals across 21 countries of which 74.3 percent (856) were useful for

analysis. Preliminary findings indicated that for the pooled data sets both EQ-5D and Barthel scores were predictors of healthcare resource use, across different categories ($p < .05$), except for nurse visits (Barthel only; $p < .09$). Overall the association between PROM data and use of healthcare resources appeared to be stronger with EQ-5D compared to Barthel. EQ-5D appeared to also predict the impact of MS on loss of productivity (in terms of work limitation; $p < .05$).

CONCLUSIONS:

PROMs can be used to predict the economic consequences of MS on healthcare providers and society, but more research is needed to confirm the robustness of the evidence and its validity across individual healthcare system settings.

REFERENCES:

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