primary endpoints. The latter implies only in 67 percent of the assessments a primary endpoint to be relevant for the benefit-harm-balancing. Moreover, explorative mortality endpoints reached the highest agreement and explorative endpoints capturing health-related quality of life no agreement, referring to the mutual relevance of endpoints for the risk-benefit-ratio and the benefit-harm-balancing.

CONCLUSIONS:

The missing information transparency of the assessment reports compared to the information offered within the early benefit assessment makes an assignment of endpoints with respect to the mutually relevant clinical trial sometimes troublesome. To warrant, in the long run, a broader confirmatory basis for decisions in health care supported by HTA, a closer inter-institutional cooperation of approval authorities and German HTA jurisdictions seems favorable.

OP136 Clinical Benefit Of Oncological Therapies At The Time Of Approval

AUTHORS:

Nicole Grössmann (Nicole.Groessmann@hta.lbg.ac.at), Claudia Wild

INTRODUCTION:

In the last decade an increasing number of high-priced, new cancer treatments received marketing authorization in Europe. What is actually known about the clinical benefit of those therapies at the time of approval needs to be elucidated in order to inform decisions about the use and reimbursement of these novel treatment options. Thus, the aim of the current analysis was to systematically investigate oncological therapies approved between January 2009 and April 2016. We extracted, as well as quantified the level of knowledge of the clinical benefit at the time of marketing authorization.

METHODS:

To assess the benefit of new interventions as well as expanded indications, we extracted the median gain of the two study endpoints: progression-free survival (PFS) and overall survival (OS). Information is based on approval documents provided by the European Medicines Agency (EMA) and assessments from the Austrian Horizon Scanning programme (HSO). We included all cancer therapies approved in Europe between 1 January 2009 and 15 April 2016.

RESULTS:

Cancer drugs for 134 new indications approved since 2009 were identified. In the case of thirty-seven indications (27 percent), no data was available for PFS or for OS. A positive difference in median overall survival was reached by seventy-six licensed indications (55.5 percent); twenty-two (16 percent) of them showed a difference of more than three months. Regarding the study endpoint progression-free survival, an improvement was shown in ninety indications (65.2 percent).

CONCLUSIONS:

Scarce knowledge regarding the clinical benefit of anti-cancer therapies is available at the time of approval. In addition, the survival benefit of the approved indications is less than three months in the majority of approved therapies.

OP138 Access To Orphan Drugs In The United Kingdom And Other European Countries

AUTHORS:

Bernarda Zamora (bzamora@ohe.org), Martina Garau, François Maignen, Phill O'Neill, Jorge Mestre-Ferrandiz

INTRODUCTION:

Under the Orphan Regulation, the European Medicines Agency (EMA) intended to incentivize the research and development of new treatments for rare and

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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 Eropean 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

AUTHORS:

Michela Tinelli (m.tinelli@lse.ac.uk), Olina Efthymiadou, Jean Mossman, Panos Kanavos

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 cognitionon (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:

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

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