OBJECTIVES: To explore opinions among professionals in health economics and related fields on global trends in the use of HE economic analysis in various decision-making areas and specifically in applying HE analysis to individualized medicine and orphan medications. The survey captured professionals who work with HE data. The survey captured 45 responses from respondents working with HE data in various decision-making areas and specifically in applying HE analysis to individualized medicine and orphan medications.

RESULTS: Seventy-three professionals completed the survey; 53% from Europe, 30% United States, and 16% from other countries. 25% were from the pharmaceutical/medical technology industry, while 75% were from academia/government and other institution types. The survey respondents expected the role of HE analysis in reimbursement decisions (89%), followed by manufacturers’ internal pricing (78%), clinical guideline development (70%), and clinical practice (59%). Opinions on whether cost-effectiveness analysis of individualized medicine will become a dominant approach in the next three years varied widely with 49% of respondents in agreement and 19% disagreeing. Equally, 37% of the respondents agree and 37% disagree that orphan drugs should be subjected to the same value-based assessment schemes mainly to arrange broad market access, and were successful in their approach to many orphan treatments, patients who must show evidence of the clinical need (e.g. pathology report to confirm the diagnosis) and whose condition is inadequately controlled by existing, less expensive therapies. For many orphan patients, must also demonstrate adequate clinical improvement; clinical outcomes are evaluated according to predetermined quantifiable criteria. Because of the availability of multiple effective agents for a single clinical indication (e.g. rheumatoid arthritis), Australia was the first country to establish an ‘interchangeability rule’ under a publicly funded, national drug coverage system. Australia has created an innovative funding approach to balance the benefits, risks, and costs of specialty pharmaceuticals.

OBJECTIVES: To provide an overview of past and current models and practices in innovative contracting in Germany, the UK, Sweden, and France. Another aim was to explore the experiences of different stakeholders in innovative contracting in Germany and the UK. METHODS: A comprehensive literature search on innovative contracting from 2008 onwards was performed. Information on the country involved, drug type, characteristic of the therapeutic area, timeframe, terms of the agreement and stakeholders involved was extracted. Interviews with 22 stakeholders from Germany (n=14) and the UK (n=8) were conducted. Stakeholders included pharmaceutical company staff, payers, medical practitioners, governmental bodies and academics. RESULTS: The countries showing the highest activity in the use of innovative contracting were the UK (23 from 60 contracts), followed by Sweden (15/60) and Italy (10/60). Most schemes were applied to oncology drugs (29/60). The most frequently mentioned innovative contract model in the literature was the Coverage with Evidence Development (CED) scheme (23/60). From the interviews, it was observed that most stakeholders applied these schemes mainly to arrange broad market access, and were successful in their implementation (32%). Where stakeholders avoided innovative contracting schemes, this was due to their complexity, high administrative burden, and uncertainty for payers. The high-admistration burden was regarded as the greatest pitfall of innovative contracting schemes, being mentioned by 45% of the stakeholders. For the future, they prefer the use of simple rebate schemes. CONCLUSIONS: Innovative contracting provides a valuable tool to increase innovative pharmaceutical market access whilst limiting the impact on payers’ budgets under control. These schemes have not yet gained widespread acceptance, and stakeholders in the UK and in Germany are suspicious as to their benefit and their future relevance. Systematic research is needed to allow for the evaluation of these schemes.
ever, many governments are forsaking such tactics to focus on shorter-term quick fixes. Whilst recognising that risks-sharing agreements represent an important market access strategy, the objective of this research was to examine the market expansion in number of risk-sharing agreements through 2007-2010 is still continuing, or if there is a gradual levelling off across the world. METHODS: Secondary research was conducted examining reimbursement decisions around the world, with a focus on Australia, Belgium, Brazil, Canada, China, France, Germany, Hungary, Italy, The Netherlands, New Zealand, Poland, Russia, Spain, Turkey, UK and United States. This was supplemented by primary research with payers and organisations through interviews in native languages to identify potential risk-sharing guidelines/policies and their perceptions is as general opinion. RESULTS: Thirty-two new risk-sharing agreements were found in the period of review (May 2011 - May 2012), which is roughly in-line with the rate found in previous years. The number of new drugs with risk-sharing agreements attached to them actually declined, and most new agreements are being negotiated for drugs which are new to the market. The resulting payers tend to be heavily risk-based, although new performance-based agreements continue to emerge, including in emerging markets. The majority continue to focus on the oncology area. CONCLUSIONS: As part of the process of updating the National List of Health Services (NLLHS) in Israel, both health-plans (“payers”) and manufacturers provide estimates on the expected number of patients that will utilize the drug. Currently, payers face major financial consequences when actual drug utilization is significantly higher than the allocated budget. We suggest a risk-sharing model that imposes a potential penalty on the two stakeholders; if the actual number of patients exceeds the manufacturers’ prediction, the manufacturer will reimburse the payers by a rebate rate α from the deficit. In case of under-utilization, payers will refund the government at a rate of γ from the surplus budget. Our study objective was to identify the optimal early estimations of both ‘players’ prior to and after implementation of the risk-sharing model. Using a structured approach, we found that both players’ statements are considered simultaneously, we examined the impact of risk-sharing within a given range of rebate proportions (α, γ), on players’ early budget estimations. RESULTS: With no risk-sharing, manufacturers and health-plans will choose to announce the smallest and highest number of patients, from the cumulative distribution function of patients, respectively. When increasing "α" to be over 50%, manufacturers will announce a larger number and health-plans will announce a lower number of patients than they would without risk-sharing, thus, substantially decreasing the gap between their estimates. On the other hand, in case of increasing "γ", there appears to be a notable “leveling off” of the rapid expansion of this strategy in previous years. This is relatively unsurprising as it reaches a natural plateau, but still notable against the background of ongoing global austerity.

PHP191 IMPACT OF A FINANCIAL RISK-SHARING SCHEME ON BUDGET-IMPACT ESTIMATIONS: A GAME-THEORETIC APPROACH Hammesmeyer A, Segall A, Greenberg D, Ben Gurion University of the Negev, Beer-Sheva, Israel OBJECTIVES: Without the two stakeholders, if the actual number of patients exceeds the manufacturer’s prediction, the manufacturer will reimburse the payers by a rebate rate α from the deficit. In case of under-utilization, payers will refund the government at a rate of γ from the surplus budget. Our study objective was to identify the optimal early estimations of both ‘players’ prior to and after implementation of the risk-sharing model. Using a structured approach, we found that both players’ statements are considered simultaneously, we examined the impact of risk-sharing within a given range of rebate proportions (α, γ), on players’ early budget estimations. RESULTS: With no risk-sharing, manufacturers and health-plans will choose to announce the smallest and highest number of patients, from the cumulative distribution function of patients, respectively. When increasing "α" to be over 50%, manufacturers will announce a larger number and health-plans will announce a lower number of patients than they would without risk-sharing, thus, substantially decreasing the gap between their estimates. On the other hand, in case of increasing "γ", there appears to be a notable “leveling off” of the rapid expansion of this strategy in previous years. This is relatively unsurprising as it reaches a natural plateau, but still notable against the background of ongoing global austerity.

PHP192 RECENT GLOBAL INSIGHTS INTO RISK SHARING AGREEMENTS: A COMPARATIVE ANALYSIS Bruning A, Morawski J, Nijhuis T, Quantities Consulting, Noordwijk, The Netherlands OBJECTIVES: To evaluate whether risk-sharing agreements (RSA) are utilised by health technology assessment (HTA) agencies around the world. Similarities and differences between appraisals where an RSA is applied will be assessed across the different agencies. METHODS: Nine select HTA agencies across the globe (MOHTEL, NICE, FRAC, SM/C, TL/9N, INESS/D, CADTH, NCPE, and AWMSG) were scanned to determine what type of RSAs were adopted for drug appraisals. Only single technology appraisals published between 2010 and April 2012 were included in the search. Comparisons were made between the agencies to determine whether any common trends were present, particularly for appraisals on the same drug. RESULTS: In total 100 HTAs (74 treatments) were identified that included an RSA across the 9 agencies. The number of RSAs identified per agency was as follows: MOHTEL (24 HTAs), NICE (23), FRAC (15), SM/C (14), TL/9N (10), INESS/D, CADTH (6), NCPE (6), and AWMSG (2). Overall there was very little consistency between agencies as to which treatments included an RSA. For the very few treat-ments with an RSA fwith more than one agency, the type of agreement applied between these agencies varied. RSAs identified in NICE submissions were often elaborate whilst the remaining agencies usually applied service reductions or cost agreements. Interestingly, all recently submitted oncology thera-pies to INESS/D were required to have a shared financial risk agreement for recommendation. CONCLUSIONS: RSAs are applied by several HTA agencies from around the world. There does not seem to be consistency in RSAs amongst the different agencies. If an RSA is made for a particular treatment for one agency, this does not mean an RSA will be applied by another agency for the same treatment.

HEALTH CARE USE & POLICY STUDIES - Conceptual Papers

PHP193 PAEDIATRIC USE MARKETING AUTHORISATION (PUMA): THE CHALLENGES OF COST-EFFECTIVENESS MODELLING WHEN LIMITED CLINICAL TRIAL INFORMATION IS AVAILABLE Gladwell D1, Lee D1, Tate F, Barry A1, Birreret N1

TrenMed, Sheffield, UK; 2Hayward Medical Communications, Neumarket, Suffolk, UK Paediatric use marketing authorisation (PUMA) was developed by the European Medicines Agency to promote the development of paediatric formulations of products that are already authorised but are no longer covered by intellectual property rights (patent, supplementary protection certificate). There are a number of aims of which 2 of which are of interest here: ensure that medicines used to treat children are subject to a quality, efficacy and are approved. The aim of this article is to achieve these objectives without subjecting the paediatric population to unnecessary clinical trials and without delaying the authorisation for other patients. In September 2011 BUCCOLAM® was the first product to receive a PUMA for the treatment of prolonged, acute, convulsive seizures. Products approved in this way are likely to have less comparative data which makes both Pharmacoeconomic value demonstration and assessment more challenging. In order to undertake cost-effectiveness analyses for BUCCOLAM to inform HTAs, de novoprivate data gathering was required. This included: gathering expert views on treatment pathways, downstream consequences of seizures and utilities (utilising a Delphi process); gathering information on treatment pathways and the frequency and locale of seizures (patient/carer surveys); and a cost-gathering exercise with hospitals. The SIVACHTA system allowed the data gathered about inpatient treatment to be supplemented with extensive sensitivity analysis which addressed the economic uncertainties resulting from the limited clinical trial data. In other countries, where reimbursement is linked to the strength of efficacy evidence it can be very difficult for a PUMA to demonstrate value and therefore may be necessary for HTA bodies to review their requirements for interventions licensed via this regulatory process and prepare an alternative pathway to assess their value. In many cases this approach has been taken for orphan and ultra-orphan diseases where the same data challenges many may apply.

PHP194 WHY DO PATIENTS ENGAGE IN MEDICAL TOURISM? Carrera P

University of Twente, Enschede, The Netherlands Medical tourism is commonly perceived and popularly depicted as an economic issue, both in the system and at the levels of national care. Why do patients engage in medical tourism, however, is more complex, driven by patients’ unmet need(s), the nature of services sought and the manner by which treatment is accessed. In order to harness and promote the opportunities medical tourism offers, as well as address and contain attendant threats, an informed decision is crucial. This paper aims to enhance the current knowledge on medical tourism by isolating the types of decisions that patients make – and based on the existing literature, proposing a theoretical sequence in opting for or against medical care abroad. It proposes a sequential decision-making process to engage in medical tourism, which includes considerations of the required treatments, location of treatment, and the quality and safety issues that are attendant to seeking care. Where patient involvement is regarded as crucial in achieving the desired health outcomes and promoting the effective use of resources, the active role of the patient under medical tourism should prove to be valuable. In consideration of the challenges and opportunities that medical tourism offers, bringing forward scholarship on the globalization of health care in general and of medical tourism in particular, calls for developing empirical evidence on this increasingly popular and complex form of accessing and provision of medical care.

PHP195 ADOPTION OF NEW TECHNOLOGIES IN TWENTY ESTABLISHED AND EMERGING MARKETS Herz D, Marcarelli AD, Patel L, Garfield S, GfK Bridgehead, Wayland, MA, USA OBJECTIVES: The purpose of this analysis was to evaluate drivers affecting market access for new technologies in twenty established and emerging markets (Australia, Brazil, Canada, China, Denmark, Finland, France, Germany, Italy, India, Israel, Japan, Norway, Poland, Russia, Singapore, Spain, Turkey, the UK, Sweden, and Switzerland). METHODS: Health care spend, government debt ratio, financing structure and regulatory policy were examined relative to their impact on market access. Comprehensive reviews of publicly available literature, data sources, policies and regulations were performed for each country of interest. RESULTS: En- trants to markets without an official regulatory body face stiff competition from non-standard or counterfeit comparator products. At the other extreme, some countries require substantial country-specific clinical evidence for approval, making the approval of new technologies longer and more expensive. High government debt ratios were found to be predictive of increased austerity measures, which broadly have a negative impact on market access for new technologies, placing pressure on downward pricing in countries that use national fee schedules. DGB-based systems were found to allow for far greater receptivity to new technologies than fixed budget systems that require patients to access health care through annual global payments. The likelihood of a medical device receiving an HTA in any country is dependent on (1) regulatory requirements for market entry and (2) the existence of device-focused HTAs, which are not as pervasive as HTAs for pharmaceuticals. In countries where inpatient procedures are funded by global payments, hospital level reviews are more likely than a national assessment for medical device technologies. In countries where fee-for-service dominates device