OBJECTIVES: More than 7000 rare diseases have been identified, and mostly have a genetic disorder. In 1983, the Orphan Drug Act was implemented in the United States to encourage the development of drugs for rare diseases. Since then, many orphan drugs have been developed but payers concern about their high prices due to a limited health care budget. In this article we tried to find a solution against lack of methodological science of orphan drugs, and pharmaceutic cost-effectiveness of rare diseases. We established orphans for the first time in Korea. We adopted 3 products to estimate the affordable threshold in cost-effectiveness plane along two properties: 1) reflection of the cost increase in the health care budget, and 2) index of effectiveness including the product, severity and efficacy for each product. Then we modeled a new product by changing its properties and showed results. RESULTS: We defined and analyzed the function of affordable threshold based on cost and index of effectiveness in two dimensions. The index of effectiveness was calculated from 0.05 to 0.25 million earned cost was distributed over average cost of 1.8 and 3.0 hundred million won per year approximately. The affordable threshold for a new drug highly depends on weights of prevalence, severity and efficacy. CONCLUSIONS: Evidences for rare diseases are often generated from the surrogate outcome, small population and no comparator. Therefore, it is difficult to assess cost-effectiveness of drugs for rare diseases with current approach. We showed that the affordable threshold can be calculated by the products’ properties and monitoring periodically. This method needs the social agreement for weights and we discuss further limitations.

PHP120
THE PAST AS PROLOGUE: USE OF COMPARATIVE EFFECTIVENESS REVIEWS (CER) IN DIOGARES TOOLS AND PREDICTIONS IN THE UNITED STATES AND PREDICTIONS ON FUTURE USE OF CER
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OBJECTIVES: 1) Review all Medicare national coverage determinations (NCDs) from 2007 through 2011 to identify how CER was explicitly used for or considered in the decision, and 2) Make inferences on Medicare’s future use of CER from past behavior. METHODS: We used current Medicare Health Reform developments (CED) requirement. We characterized Medicare’s historical coverage which CER was used; 2) Types of products and services (e.g., device, procedure); 3) Methodology to identify whether a comparative study or health technology assessment (HTA) was used. RESULTS: We reviewed 132 AHTAPol Recommendations and 13 NICE Technology Appraisals issued in 2010 were reviewed (in 2 cases, because of the lack of evidence, NICE was unable to make a recommendation). Social implications were found in respectively: 27% and 82% of recommendations. The impact of social implications on HTA recommendations was more common in the UK. In total 59 and 12 were reviewed for AHTAPol and NICE, social implications were found in respectively: 46% and 83% of recommendations. The impact of social implications on HTA recommendations was more common in the UK. Social implications, frequently raised by AHTAPol during the analysis, were: changes in access to health care; changes on patient’s functioning in society (15%), patient’s ability to work (14%) and others (21% - mainly, avoidable hospitalization). NICE paid more attention to: changes in access to health care (26%), influence on patient’s functioning in society (15%), influence on social results (15%) and others (18% - mainly, discrimination). CONCLUSIONS: During the analyzed period, NICE considered social implications more frequently than AHTAPol. NICE and AHTAPol paid attention to different types of social implications.

PHP123
TO WHAT EXTENT DOES ADVICE FROM THE SCOTTISH MEDICINES CONSORTIUM (SMC) AGREE WITH THAT PUBLISHED BY NICE?
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OBJECTIVE: In the UK, the National Institute for Health and Clinical Excellence (NICE) assesses the cost-effectiveness of therapies in England and Wales. In Scotland, the Scottish Medicines Consortium (SMC) is responsible for such decisions. There are recognised differences in how these agencies operate, with the SMC adopting an early, rapid approach to health technology appraisal and NICE favouring a more extensive, detailed review. Conflicting decisions between the two agencies can lead to differential drug availability, however, it is generally believed that the recommendations are broadly the same. The purpose of this review is to evaluate the level of agreement over the last year. METHODS: The NICE website was searched for single technology appraisals (STA) published between January and December 2010. The appraisal was compared with the recommendation of the SMC website and the recommendations of NICE and the SMC compared. RESULTS: Nineteen STAs were performed by NICE in 2010. These included 11 drugs for cancer indications and an assortment of 8 others. Of the 19 drugs evaluated, NICE recommended 12 and rejected 7. For the same drugs, the SMC recommended 8 and rejected 11. Decisions between the agencies were the same for 13 drugs, equating to agreement in 68.4% of cases. Of the 6 cases where the recommendation differed, 5 were recommended by NICE in all five cases the SMC found that the economic cases presented by the manufacturers were not sufficiently robust. In one instance weaknesses in the clinical data were also implicated. The one drug recommended by the SMC in contradiction of NICE was also rejected based on cost-effectiveness. CONCLUSIONS: In general, there is reasonable agreement between decisions made by NICE and the SMC. Poor evidence regarding cost-effectiveness is the most commonly cited reason for one agency not recommending a drug.

PHP124
PERSONALIZED DECISION MAKING IN CANCER MEDICINE? SYSTEMATIC OVERVIEW OF HTA PROCEDURES AND SPECIFIC APPROACHES IN TEN COUNTRIES ACROSS FOUR CONTINENTS
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OBJECTIVES: Capacity constraints jeopardize health care systems’ sustainability all over the world while the number of Health Technology Assessment (HTA) agencies continues to increase. This paper will analyze how CMS and other health technology assessment organizations manage to identify most cost-effectiveness thresholds held on basis of HTA/economic evaluations should indicate whether a technology is worth its costs. Personalized cancer medicine (PCM) promises to be different from established technologies raising the question whether decision making also differs for PCM. Our goal was to identify cost-effectiveness thresholds in general or specific to PCM to finally provide input for decision makers and expert panels. METHODS: A conceptual evaluation framework was developed comprising eight phrased CER, will make cost-effectiveness part of CER, will require strong clinical utility evidence for payer coverage, and trends will be universal and more pronounced ex-US.

PHP122
A COMPARISON OF HTA RECOMMENDATIONS ISSUED BY AGENCY FOR HEALTH TECHNOLOGY ASSESSMENT IN POLAND (AHTAPOL) AND NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE (NICE) IN THE UK - CONSIDERATION OF SOCIAL IMPLICATIONS IN HTA
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OBJECTIVES: Verification whether social implications were considered in HTA process in Poland and the UK. METHODS: The comparative analysis included following stages: 1) HTA recommendations issued in the period of January 2010 to May 2011 for AHTAPol and September 2010 to May 2011 for NICE. 2) HTA recommendations were labeled as positive, negative or other (when outcome was neither positive nor negative). 3) Check-list was composed on the base of INAHTA definition of social consequences in HTA and also of a definition which additionally introduced changes in equity and access as a social effect of implementation of a technology. Social implications were grouped in 6 categories; and 4) The impact of consideration of social implications in HTA recommendations was determined. RESULTS: Total of 132 AHTAPol Recommendations and 13 NICE Technology Appraisals issued in 2010 were reviewed (in 2 cases, because of the lack of evidence, NICE was unable to make a recommendation). Social implications were found in respectively: 27% and 82% of recommendations. The impact of social implications on HTA recommendations was more common in the UK. In total 59 and 12 were reviewed for AHTAPol and NICE, social implications were found in respectively: 46% and 83% of recommendations. The impact of social implications on HTA recommendations was more common in the UK. Social implications, frequently raised by AHTAPol during the analysis, were: changes in access to health care (26%), influence on patient’s functioning in society (15%), influence on social results (15%) and others (18% - mainly, discrimination). CONCLUSIONS: During the analyzed period, NICE considered social implications more frequently than AHTAPol. NICE and AHTAPol paid attention to different types of social implications.