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 methodologies and evidences for pricing and reimbursement of orphan drugs and represent the results graphically. **METHODS:** Cerezyme, Myozyme and Elaprase are reimbursed for rare diseases 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 prevalence, 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 0.60 to 0.85 and median cost was distributed between 1.8 and 3.0 hundred million won per year approximately. The affordable threshold for 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 NATIONAL COVERAGE DECISION MAKING BY MEDICARE 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 an impetus for or considered in the decision, and 2) Make inferences on Medicare's future use of CER from past behavior and recent health reform developments. **METHODS:** We reviewed documentation to identify whether a comparative study or health technology assessment (HTA) was cited in Medicare's decision initiation rationale or referenced in the decision. Specifically, we determined the: 1) Number of NCDs and degree to which CER was used; 2) Types of products and services (e.g., device, procedure); 3) Therapeutic areas; 4) Organizations producing technical CER materials; 5) Inclusion of cost effectiveness; and 6) Frequency and content of a coverage with evidence development (CED) requirement. We characterized Medicare's historical coverage and payment behavior and prognosticated on how aspects of health reform may affect future CER use. **RESULTS:** More than 55% or 36 NCDs considered CER, with radiological procedures and diagnostic/screening tests comprising over half. Sources for the CER technical work were 5 US and 5 international organizations. Eleven of the decisions considered cost effectiveness; 4 reported a cost-effectiveness ratio. While a minority, CED judgments increased over time. Medicare has historically covered and set reimbursement levels that allow for the cost of care plus some profit, only recently and selectively considering evidence of comparative clinical or cost effectiveness. While provisions of the Affordable Care Act and regulatory changes promote the greater use of CER, there are official and practical impediments that serve as a counterbalance. **CONCLUSIONS:** Medicare increasingly will use CER in making NCDs but in ways less straightforward than predicted. While many methods are available to Medicare, perhaps the most promising in the current political environment are evidence threshold "creep", CED, and several novel applications of CER to coverage, coding, and pricing.

PHP121

THE ROLE OF MOLECULAR TESTS IN SHAPING COMPARATIVE EFFECTIVENESS REVIEW (CER)

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OBJECTIVES: 1) Perform a comprehensive review of the US molecular testing environment; 2) Infer from **RESULTS:** Diagnostic/treatment areas most likely to include comparative effectiveness reviews (CER) involving molecular testing, how CER will be shaped by molecular diagnostics, evidence used in coverage decisions; and 3) Determine global generalizability of US trends. **METHODS:** We reviewed: 1) All non-perinatal molecular tests with actual or potential for CER interface, all Medicare national (NCDs) and selected local coverage decisions (LCDs) involving molecular tests and CER, and US government and related agency high priority disease areas for CER, assessing actual or potential molecular testing inclusion, and 2) We inferred: Clinical areas most likely involving molecular diagnostics and CER,iImpact of molecular testing on CER, evidence required for Medicare/other payer coverage, and universality of US findings. **RESULTS:** 1) Inventory indicates 442 molecular tests/combinations of interest; 259 have potential degrees of CER interface; 2) Medicare database yielded: 2 NCDs (Screening DNA Stool Test for Colorectal Cancer, Pharmacogenetic Testing for Warfarin Response), 9 LCDs and articles: many denials of molecular test coverage, denials cite lack of evidence, including trials, clinical utility, and few cite cost-effectiveness data; 3) Five governmental or health technology assessment organizations point to 14 clinical areas as highest CER priorities: six known to have associated molecular tests; 5 already involved CER activity, and another 7 predicted to have future molecular testing and CER activity per identified priority areas for research. **CONCLUSIONS:** Expect molecular testing: to play an increasing future role in CER, particularly in 7 areas (cancer and hematopathology most prominently, inclusion will necessitate more methodologically sophisticated 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 basis of INAHTA definition of social issues 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 issues 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 analyzed period, were: changes in access to health care (48%), influence 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 subcultures (15%) and others (35% - 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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OBJECTIVES: 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 (STAs) published between January and December 2010. The appraisals for the same drugs were identified on 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. Explicit or implicit use of cost-effectiveness thresholds based on 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