These analyses suggest that OXY access restrictions such as PA and TC did not result in cost savings.

PSY70
ESTIMATED ERROR IN USING NATIONAL INCIDENCE FIGURES VERSUS STATE ESTIMATES FOR RARE DISEASE CALCULATIONS OF ESTIMATED CASES AND COST PER CASE DETECTED IN NEWBORN SCREENING (NBS)CONGENITAL ADRENAL HYPERPLASIA (CAH) AND CONGENITAL HYPOTHYROIDISM (CH)

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OBJECTIVES: In 2006, the American College of Medical Genetics (ACMG) recommended a significantly expanded group of rare conditions for state-based US NBS programs. Initial efforts to explore the implications of this expansion used national incidence data applied to the states due to an inability to gather current state-specific numbers. Older state-specific numbers were later identified. This research assesses the error associated with estimating consequences of these programs by using national vs. state-specific incidence estimates. **METHODS:** We collected data on national disease incidence and state-specific numbers of births (2011), state-specific disease cases (2003 and 2006) and number of required NBS tests/birth and reported fees. We developed two estimates of cases: 1) expected cases by applying national incidence to current state births and 2) cases identified using state-specific actual observations (averaged over the 2 years and applied to current number of births). We also calculated the cost per expected identified case using both methods of estimating cases. **RESULTS:** The differences in numbers based on state actuals vs. expected cases calculated from national incidence estimates was expressed as a percentage of the actuals. For CH this ranged from -71% to + 200% (mean=-33%; 46 were negative; 2 were positive; 3 unchanged) and in CAH from -67% to + 500% (mean=+70%; 9 were negative; 22 were positive; 7 unchanged and 13 missing or undefined due to no cases being in the denominator of the calculation). Similar differences were observed in calculations of cost per identified cases. **CONCLUSIONS:** Sampling variation and the association with ethnicity and other differences by state demographics implied added variation in state actuals compared to national incidence used in predictions. The one-sided error observed in the CH calculations leads us to question the accuracy of the national incidence figure. Application of national incidence to state-specific situations should proceed with caution.

PSY71
INTER-TEMPORAL CHANGE OF BODY MASS INDEX IN BRAZILWHAT IS THE ROLE OF FOOD PRICES?

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BACKGROUND:: Obesity can be understood as a problem resulting from the imbalance between the intake and the individual caloric expenditure, which has sparked concern among researchers and policymakers in public health. The alert comes up with the fast growth in the prevalence of obesity in adults and children in Brazil. **OBJECTIVES:** In this context, the challenge of this essay is to analyze the major changes in BMI between 2002 and 2009 and to estimate the relationship between food prices and obesity, in order to check the effect of food prices on BMI in Brazil. **METHODS:** To assess the changes in BMI over time, first of all, we analyze its distribution using the relative distribution method proposed by Handcock and Morris (1998). Then, recentered influence function (RIF) regressions were applied to decompose the changes in the BMI distribution into composition and structure effects and identify the specific contribution of food prices. The empirical estimates needed are based on micro-data from household budget survey in two points of time: 2002-2003 and 2008-2009 accomplished by the Brazilian Institute of geography (IBGE). Were considered only the information regarding the adult population aged less than 20 years. **RESULTS:** The main changes in BMI distribution showed that the density has moved to the right over the time and there were increases in the right hand tail. Despite the small effect, the RIF results show that the prices variations over the period contributed to change BMI. If prices had not changed, the median BMI would be 0.22 higher. The increases in the prices of meat, ssb and fruits and vegetables showed the greatest mean effects on BMI, especially to upper quantiles of the distribution. There are also evidences that income and age contributed to rise BMI, while education is associated to a lowering effect.

PSY72
AN EUROPEAN OVERVIEW OF THE FUTURE CHANGES IN EVIDENCE REQUIREMENTS FOR THE REIMBURSEMENT OF ORPHAN DRUGS-A STAKEHOLDER ANALYSIS

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OBJECTIVES: In 2000 the Orphan Drug Act was designed to supported drug development for rare disease in Europe. Now thirteen years after the Act was passed, orphan drug development has immensely increased and health care systems are challenged to find more appropriate assessment mechanism for the reimbursement of orphan drugs. Public awareness has lately been raised by major discussions about the discontinuation of reimbursement for several orphan drugs. By conducting qualitative interviews this article aims to identify the view of different stakeholders about recent and future changes within the reimbursement assessment and its evidence requirements for orphan drugs. **METHODS:** Twenty semi-structured interviews were conducted with relevant stakeholders from the orphan drug community. Interviewees were scientific experts, reimbursement agencies, industry and patient organizations from five European countries. The interviews were analyzed with the framework analysis technique. **RESULTS:** All twenty stakeholders have reported about recent or future changes in their national reimbursement practice for orphan drugs. The most emerging theme focused around more scrutiny in the reimbursement assessment of orphan drugs. **CONCLUSIONS:** All twenty stakeholders gave recommendations about a stronger European cooperation for the value assessment of orphan drugs, with eleven stakeholders suggesting a European reimbursement system for orphan drugs.

PSY73
CONSIDERATION FOR RARE DISEASES IN DRUG REIMBURSEMENT DECISION-MAKING

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OBJECTIVES: Reimbursement processes have been implemented to inform which therapies should be funded in light of scarce health care resources. However, the applicability of standard processes to drugs for rare diseases is heavily debated. As a result of the small patient populations affected by rare diseases, coupled with a limited understanding of the natural history of these conditions and the high cost of these treatments, it is argued that drugs for rare diseases may not meet the evidentiary standards routinely applied when making resource allocation decisions. This study identified current reimbursement processes for prescription pharmaceuticals both within Canada and internationally, with the objective of assessing how drugs for rare diseases are considered within existing processes. **METHODS:** Using the G20 countries as a sampling frame, a review of published and grey literature was conducted to identify the reimbursement processes used in 28 countries, and in Canadian provinces/territories. A search for peer-reviewed publications was conducted using Medline, Scopus, CINHAL, EconLit and PsychInfo. The grey literature search included websites of health technology assessment agencies and government agencies. **RESULTS:** Drugs for rare diseases are considering uniquely for reimbursement within three Canadian provinces and seven countries. Reimbursement frameworks focused specifically on the reimbursement of drugs for rare diseases are limited. In some jurisdictions, drugs for rare diseases are considered uniquely within the established decision-making process for drugs. Varying decision criteria are applied within the identified processes for the reimbursement of drugs for rare diseases. **CONCLUSIONS:** This review identifies approaches for making resource allocation decisions for drugs; explicitly considering funding decisions related to drugs for rare diseases. An understanding of these frameworks and the decision criteria applied when making resource allocation decisions may help inform the development of more standardized approaches for the reimbursement of drugs for rare diseases.

PSY74
THE ECONOMIC AND HUMANISTIC BURDEN OF RELAPSED/REFRACTORY (R/R) INDOLENT NON-INDOLENT NON-HODGKIN'S LYMPHOMA (INHL): AN EVIDENCE GAP ANALYSIS

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OBJECTIVES: To identify research studies that examine the economic and/or humanistic burden of R/R iNHL, and identify evidentiary gaps which could be informed by future research. **METHODS:** iNHL refers to a group of largely incurable slow-growing lymphomas that run a relapsing course after therapy, and can lead ultimately to life-threatening complications. Although many therapies are available, patients eventually relapse and become refractory to existing therapies. As such, additional treatment options with improved response rate, durability of response and more manageable toxicity are needed to treat patients with R/R iNHL. A structured literature search was performed to assess the economic and patient burden of iNHL. English-language articles published since 2009 were systematically reviewed in PubMed, EMBASE and Cochrane databases. Additionally, searches from global HTA organizations and conference abstracts were performed. Research was considered relevant to the economic and humanistic burden of iNHL based on reported outcomes such as resource utilization, costs, or relevant patient-reported outcomes associated with relapsed/refractory iNHL. **RESULTS:** Few cost-of-illness studies or HTAs address iNHL. Evidence was limited to hospital-based direct treatment costs, omitting societal and indirect costs of the disease. Multiple cost-effectiveness analyses were identified focusing on Rituximab; however, few studies evaluated the cost-effectiveness of alternative 2nd or 3rd line therapies in the case of R/R iNHL. The majority of identified patient reported outcomes (PRO) research exists as conference abstracts. No studies were identified that examine PRO in a R/R iNHL population. **CONCLUSIONS:** The economic and humanistic burden of R/R iNHL has not been widely reported in the literature. Areas of future research may include evaluating both direct and indirect costs in R/R iNHL. PROs are not well understood in iNHL, and future research should focus on QoL and related factors that may help evaluate any trade-off between progression-free survival and the severity/duration of adverse events.

PSY75
HTA ASSESSMENT COMPARISON OF ORPHAN DRUGS IN FRANCE AND GERMANY

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OBJECTIVES: In context of Health Technology Assessment (HTA) decision framework, some countries (e.g. Germany) have a special regulation for orphan drugs (OD), while others (e.g. France) don't. The aim of the study was to compare the HTA decisions, prices and reimbursements for the OD that have been evaluated in France and Germany. **METHODS:** We selected OD assessed under AMNOG law in Germany and reviewed HTA assessments from the Transparency Committee (TC) for France and from IGWiG/G-BA for Germany, and extracted prices and reimburse-

ment levels of these drugs in both countries. **RESULTS:** Eight OD were identified. In Germany, G-BA decisions related to additional benefit were non-quantifiable for 3 drugs, minor for 4 drugs, and minor in one subgroup and considerable in other subgroup for one drug. In France, one product was not assessed by the TC, and improvement in actual benefit (IAB) was rated as weak for 4 products, moderate for 2 products and important for one product. In Germany all these drugs were 100% reimbursed, while in France, depending on actual benefit (AB) ratings, reimbursement levels varied from non-reimbursement to 100%. At time of analysis, reimbursement status was granted in France for only 4 products (15% for one drug, 65% for one drug and 100% for 2 drugs). In Germany, OD prices were about 20% higher than in France before rebates, and about 20% lower after rebates. **CONCLUSIONS:** Substantial improvement for OD were less frequently acknowledged by German HTA than by French HTA and prices appeared to be lower in Germany than in France. However, price volume agreements in France might have contributed to hidden discounts. Access level of OD appeared higher in Germany.

PSY76
HEALTH ECONOMICS AND OUTCOMES DATA REQUIREMENTS IN INDOLENT NON HODGKINS LYMPHOMA (INHL) AND CHRONIC LYMPHOCYTIC LEUKEMIA (CLL) FROM UNITED STATES PAYER PERSPECTIVE

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OBJECTIVES: To understand current considerations and management of the iNHL and CLL disease states and the extent to which health economic outcomes data informs payer decision making process. **METHODS:** A national level in-depth telephone survey of 45 payers was done in the month of November, 2013. The payer mix included both Commercial (n=20) and Medicare (n=25) payers that were either medical or pharmacy directors to determine clinical, economic and humanistic outcomes data critical for formulary and medical policy deicisons for iNHL and CLL. **RESULTS:** US Payers are most interested in the endpoints that directly tie to their major cost drivers: hospitalization, re-hospitalization and emergency services in both the disease state. Payers were split on preferring the comparative data of the cost-effectiveness analysis and the simple presentation of a budget impact model. There was no difference in payer types when it came to preference for any particular health economics model. Amongst the various adverse events related to treating iNHL and CLL, neutropenia is the most concerning adverse event from payer perspective, given the associated high cost of related treatments and hospitalizations. Payers consistently reported that the greatest unmet need in the iNHL and CLL today is more treatment options for relapsed and refractory patients, with greater response rates, increased durability of response and less toxicity leading to increased overall survival and progression. **CONCLUSIONS:** While in iNHL/CLL space payers admit decisions are based more on clinical evaluations rather than cost drivers, payers appreciate the value of demonstrating a reduction in resource utilization costs as they can directly evaluate the impact of this data on their health plan.

PSY77
PATIENTS WITH RELAPSED OR REFRACTORY CHRONIC LYMPHOCYTIC LEUKAEMIA (R/R CLL) INELIGIBLE FOR CYTOTOXIC THERAPYWHO ARE THEY AND WHAT IS THEIR UNMET NEED?

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OBJECTIVES: To identify criteria used to categorize patients with R/R CLL who are ineligible for cytotoxic therapy and the unmet need in these patients. **METHODS:** Structured searches related to R/R CLL; performance status; comorbidities; organ function; and treatment patterns were performed on PubMed, Google Scholar, and the Cochrane databases and supplemented with hand searches of published literature, clinical guidelines, and online sources. **RESULTS:** 440 publications were identified. 123 were considered relevant to the study. Patients ineligible for cytotoxic therapy were identified by a combination of assessments for performance status (using ECOG), comorbidities (CIRS), and organ function (creatinine clearance (CrCl)). Unfit patients are ">65 years with a comorbidity" (ECOG 3–4 or CIRS >6 or CrCl <70 mL/min). NCCN clinical guidelines do not refer to assessment scores or define unfit: the NCCN categorizes R/R CLL patients as "≥ 70 years, or younger with comorbidities" or "frail with significant comorbidity". These criteria are not aligned with others such as slow-go (ECOG 2–4 or CIRS >6 or CrCl <70 mL/min) and no-go (fatal comorbidities with very short life-expectancy). Treatment regimens recommended by the NCCN contain drugs contraindicated for common comorbidities. The RCTs for the recommended regimens did not include patients with contraindicated comorbidities. There is an unmet need in these patients for appropriate treatment. **CONCLUSIONS:** There exists unmet need in patients identified as unfit using current criteria: they can be restricted from recommended drugs due to contraindications for common comorbidities. Most of these patients also have not been included in RCTs. Criteria used to identify patients with R/R CLL ineligible for cytotoxic therapy are not yet standardized; standardized assessments are needed for uniform identification and RCT enrolment.

PSY78
EMERGENCY PHYSICIANS' INTENTION TO USE THE TEXAS PRESCRIPTION MONITORING PROGRAMA PILOT STUDY

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OBJECTIVES: Inappropriate use of prescription opioids costs insurers over $72 billion annually in direct health care expenses. Prescription monitoring programs (PMP) are state-operated databases which allow authorized clinicians (e.g., prescribers, pharmacists) to query a patient's opioid dispensing history via a secured online connection, during patient care. Since the emergency department (ED) is a primary target for drug-seeking patients, this pilot study was conducted to validate a survey instrument designed to assess emergency physicians (EPs) intention to use the recently available Texas PMP. **METHODS:** A cross-sectional survey of EPs was conducted at a statewide emergency medicine conference. A 34-item questionnaire, based on the Technology Acceptance Model (TAM), was developed to assess EPs intention to use the Texas PMP. Items related to technology acceptance (perceived ease of use, perceived usefulness, attitude, and intention) were assessed using 5-point Likert scale responses (1=strongly disagree to 5=strongly agree). The survey expanded on a previous exploratory survey of EPs. Correlation analyses were used to validate the survey instrument scales. **RESULTS:** Of the 45 respondents, most were male (68.9%), attending EPs (57.8%), with 10.8±11.1 years in emergency medicine, from a community hospital setting (55.6%), and were users of the Texas PMP (51.2%). Among those who were not registered, 39.2% reported lack of awareness as the primary reason for not being registered. Standardized Cronbach's alphas for the constructs of perceived ease of use, perceived usefulness, attitude, and intention for PMP users were 0.88, 0.90, 0.74, and 0.84, respectively; and 0.77, 0.87, 0.84, and 0.74, respectively for PMP non-users. **CONCLUSIONS:** Considering the ED as a source of diversion, it is important to understand EPs utilization of PMPs. EPs use of PMPs may help to mitigate the economic burden associated with the non-medical use of prescription opioids, while improving patient outcomes. Future studies using this survey instrument are needed to further assess the predictive utility.

PSY79
AN EVALUATION OF OPIOID OVERUTILIZATION QUALITY METRICS USING RECEIVER OPERATING CHARACTERISTIC CURVES AND PROXIES FOR OVERUTILIZATION

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OBJECTIVES: To evaluate the sensitivity and specificity of opioid overutilization quality measures based on the Centers for Medicare and Medicaid Services (CMS) Controlled Substance Overutilization Monitoring System (OMS) using proxy indicators of potential overutilization. **METHODS:** Based on the CMS measure (proportion of patients with opioid prescriptions from ≥4 prescribers and ≥4 pharmacies among patients with ≥2 opioid prescriptions), claims from the 2012 IMS LRx database were used to evaluate the metric at different prescriber and pharmacy thresholds. Cash payment of ≥1 opioid prescription was used as a proxy for potential overutilization and set as the dependent variable in logistic regression models to generate separate receiver operating characteristic (ROC) curves for thresholds of 2-8 prescribers and 2-8 pharmacies. Optimal cutpoints for number of physicians and pharmacies were selected as the value on the curve with the shortest distance to perfect prediction. Sensitivity, specificity and positive predictive value (PPV) for every combination were calculated. As a sensitivity analysis, the process was repeated using ≥1 opioid-abuse related diagnosis as a proxy for potential overutilization. **RESULTS:** Of the 1,213,909 qualified patients, 5.9% met the CMS criteria for overutilization, while 13.8% met the alternative ROC optimal criteria (≥4 prescribers and ≥3 pharmacies). Defining cash payment patients (12.3% of total) as overutilizers, the CMS definition had sensitivity (15.0%), specificity (95.3%) and PPV (31.0%) The ROC alternative had sensitivity (28.0%), specificity (88.2%) and PPV (24.9%). For patients with opioid abuse-related claims defined as overutilizers (2.6% of total), the CMS criteria had sensitivity (16.1%), specificity (94.3%) and PPV (7.0%). The ROC alternative had sensitivity (28.5%), specificity (86.6%) and PPV (5.3%). **CONCLUSIONS:** Using two proxies for opioid overutilization, analysis of ROC curves suggested optimal criteria similar to the CMS criteria. Quality organizations can use the range of results and their preferences for sensitivity and specificity tradeoffs to develop needed quality measures.

PSY80
THE IMPORTANCE OF FACT OVER OPINION IN CHOICE OF CONDITIONS TO BE RECOMMENDED FOR NEWBORN SCREENING (NBS)

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OBJECTIVES: In 2006, the American College of Medical Genetics (ACMG) recommended expanding NBS. Recommendations relied largely on a stakeholder survey on 19 attributes of different rare conditions under consideration. The percentage of respondents agreeing to an attribute's presence determined its score. This research examines one particular attribute and asks how the recommendation of included conditions changes with the substitution of scoring based on the actual facts for that based on surveyed opinion. **METHODS:** The original report indicated each condition's scores for survey questions. Unlike some questions, that of whether multiplex technologies (allowing multiple conditions to be screened with a single test) were available, had a correct answer. Original scores for the question totaled between 0-200, depending on the respondents' percentage indicating yes. Answers were re-scored 200 or 0 as factually appropriate - existence/non-existence of multiplex screening for each condition. **RESULTS:** After eliminating conditions with missing data, 78 out of the original 84 conditions remained. 42 conditions (54%) increased their scores; 30 (38%) decreased. Of conditions with increasing scores, the mean increase was 85. Of conditions decreasing their scores, the mean decrease was 43. We estimate the potential change in recommendations as 4 conditions moving from the Core to secondary or Not Recommended and 10 moving from Secondary to Core status. **CONCLUSIONS:** As the only conditions capable of having recommendations altered by this correction were those roughly 200 points +/- category cutoffs and some ACMG rules further limited reclassification, this single correction was limited in its ability to alter recommendations (67% of the conditions could not change). Nonetheless the changes were significant (of those that could change, 50% did). As other questions in the survey were also questions of fact, doing a similar analysis for all such questions could further significantly alter the conditions recommended for the panel.