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 TECHNIQUE TO ESTIMATE CASES AND COST PER CASE DETECTED IN NEWBORN SCREENING (NBS) CONGENITAL ADRENAL HYPERPLASIA (CAH) AND CONGENITAL HYPOPHYTOSIDISM (CH) Iatrogenia A, Biro S
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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 evaluates outcomes associated with two reimbursement 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. In some states, it was not possible to detect 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 -72% to 200%. In 9%, 66% 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 within the established decision-making process for 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.

PSY73
CONSIDERATION FOR RARE DISEASES IN DRUG REIMBURSEMENT DECISION-MAKING Gómez D1, Coyle D2, Clifford T3, Jones B1
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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, stakeholders have been affected by varying processes for reimbursement within the established decision-making process for 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 BASED ANALYSIS Leinwand B1, Brown J2, Ral K3, Innocenzi T1, Agevat B3
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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 hematologic malignancies that have a relapsing course, and can lead ultimately to life-threatening complications. Although many therapies are available, patients eventually relapse and become refractory to existing therapeutic regimens. 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 Cochran databases. Additionally, searches included grey literature, conference abstracts, book chapters, letters, and case reports. Results: From the literature search, 23 studies were identified that examine PRO in a R/R INHL population. 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 Rémuzat C1, Maougi O2, Rodrigues J1, Korchagina D1, Toumi M1
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OBJECTIVES: In context of Health Technology Assessment (HTA) decision framework work, some countries (e.g. Germany) have a special regulation for orphan drugs (OD). Others (e.g. France) do not have a specific reimbursement 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 interviews, this article aims to identify the various stakeholders perceptions 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 drugs. Interviewees were scientific experts, reimbursement agencies, industry and patient organizations from three European countries. The interviews were analyzed with the framework analysis technique. RESULTS: All twenty stakeholders have reported 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.