OBJECTIVES: Despite the clearly evident better clinical outcomes with prophylaxis treatment strategy in patients with severe hemophilia, prophylaxis currently remains the standard of care for patients with severe hemophilia A and B. There are several factors contributing to this, including the lower cost of prophylaxis therapy, compared to on-demand therapy, the lower risk for joint and soft tissue bleeds, and the higher level of patient and family satisfaction with prophylaxis. However, several limitations of prophylaxis therapy also exist. Further, the development of long-acting factor products offering a modest improvement in convenience is likely to drive-up treatment costs. This study aims to understand the preferences of patients and their willingness-to-pay for prophylaxis therapies (on-demand, standard prophylaxis, long-acting prophylaxis).

METHODS: U.S. adult patients and caregivers of children with hemophilia (n = 79) completed a discrete-choice survey that presented a series of trade-off questions, each including a pair of hypothetical treatment profiles, that had been assigned costs for attaining improvement in health states. The relative importance of treatment attributes such as out-of-pocket treatment costs, dose adjustment, treatment-related complications, and clinical efficacy was assessed through a series of ordered mixed logit models. Based on the attribute estimates, patients’ WTP was determined. RESULTS: Out-of-pocket treatment costs (P < .001), treatment complications (P < .001) and clinical efficacy & dosing regimen (P < .001) were perceived to be the most important treatment attributes. Patients were willing to pay on average $156 per/month for improvement in each of the prophylaxis dosing regimens (i.e. 3 times weekly vs 2 times weekly vs 1 time weekly vs 1 time in two weeks). CONCLUSIONS: The results suggest that patients are willing to pay more for improvements in treatment-related complications, clinical efficacy & dosing regimen. These estimates of patients’ willingness-to-pay can be used to provide guidelines for resource allocation. Literature also suggests that patient preferences are likely to directly translate in increased treatment adherence, leading to greater treatment effectiveness. Literature also suggests that patient preferences are likely to directly translate in increased treatment adherence, leading to greater treatment effectiveness.

PSY45

PSYCHOMETRIC PROPERTIES OF THE ADHOC RATING SCALE-IV (ADHD RS-IV) AND ADULT ADHD SELF-REPORT SCALE (ASRS) IN A PHASE 3B CLINICAL TRIAL OF PATIENTS WITH PHENYLKETONURIA

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OBJECTIVES: Phenylketonuria (PKU) is the most common in-born error of metabolism in children. Long-term complications of pain-related disability and delayed development are common in PKU. Parents and parents of children with PKU have established that inattentiveness is an important psychiatric symptom. Although the Adult ADHD Self-Rating Scale (ASRS; self-report) and ADHD Rating Scale (ADHD-RS-IV; parent-report) instruments have been validated for measuring symptoms of inattentiveness in persons with ADHD, their psychometric value for measuring PKU-related inattentiveness has not been established. This study investigated the psychometric properties of the ASRS and ADHD-RS in PKU in a Phase 3b clinical trial.

METHODS: A post-hoc analysis was conducted in participants ≥8 years of age in a Phase 3b double blind, placebo-controlled clinical trial in PKU over a 13-week period. The ASRS/ADHD-RS-IV reliability, construct validity, and responsiveness were measured using clinician- and adult-/parent-reported outcomes collected in this clinical trial. RESULTS: Data analysis revealed strong internal consistency reliability for both measures (α ≥ .87). In addition, the test-retest analyses resulted in ICCs ≥ .87 in the PKU population. In a sub-analysis of non-serious limitations. The Clinical Global Impression-Severity (CGI-S) scale. Correlations between the ASRS/ADHD-RS-IV and the CGI-S and age-appropriate Behavior Rating Inventory of Executive Function (BRIEF) Working Memory subscale were consistently moderate-to-strong (r ≥ .50). Similarly, results of the change score correlations were moderate (r ≥ .40) for both measures when compared changes over time in the BRIEF Working Memory subscale. CONCLUSIONS: These findings of reliability, validity, and responsiveness of both the ASRS and the ADHD-RS IV suggest that the inattentiveness subscales in PKU can further support their use for the assessment of inattentive symptoms among persons with PKU ≥8 years old in clinical trials.

PSY46

PATIENT-REPORTED MEASURES OF VASO OCLUSIVE CRISIS OUTCOMES – GRADING THE LITERATURE

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OBJECTIVES: Patient-reported measures of Vaso Oclusive Crisis (VOC) outcomes reported in literature were identified and quality of evidence for each type of measure was assessed. METHODS: MEDLINE, Embase, Cochrane, and Web of Science (through August 2012) and reference lists of identified articles were searched for any patient-reported measures employed to assess VOC outcomes. Identified measures were categorized as pain, and measured disability. RESULTS: Quality of evidence for each measure was assessed using the Modified Jadad Scale. Our findings indicate the need to enhance patients’ self-efficacy and reduce the use of passive coping strategies in order to decrease pain-related disability. Future intervention studies improving self-efficacy and coping are needed to improve patients’ wellbeing and quality of life.

PSY47

PATIENT-REPORTED OUTCOME (PRO) CLAIMS IN PRODUCTS INDICATED FOR THE TREATMENT OF RARE DISEASES AND APPROVED BY THE EUROPEAN MEDICINES AGENCY (EMA)

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OBJECTIVES: 1) To identify products indicated for the treatment of rare diseases approved through the centralized procedure in Europe, and 2) To list the products which obtained a PRO labeling claim. METHODS: The search was performed on the EMA website (EMA website (11/16/13)). The European public assessment reports (EPARs) were browsed by type (i.e., orphan medicines). Products refused were excluded. Then, the PROLabels database was searched for each product released to identify any PRO claim in the corresponding lab. RESULTS: A total of 69 orphan medicines were identified on the EMA website, representing 11 different