OBJECTIVES: Hemophilia is a chronic disease typically diagnosed in infancy, characterized by bleeding requiring regular or episodic infusion of clotting factor. Taking care of a child with hemophilia (CWH) may cause burden for caregivers. We aimed to develop a first "Hemophilia associated Caregiver Burden Scale" (HEMOCABTM) assessing the burden of hemophilia for caregivers of CWH. **METHODS:** Questionnaire development included: 1) item generation (two semi-structured focus groups with 11 caregivers, evaluation of existing caregiver burden scales for relevance by 16 HCPs); 2) feasibility testing (cognitive interviews with 12 caregivers) and 3) pilot-testing (psychometric analysis of HEMOCABTM). **RESULTS:** Item generation resulted in a preliminary version of HEMOCABTM consisting of 109 items. During feasibility testing, mean completion time was 19.3±6.3 minutes, some problems were revealed concerning item formulation, applicability of items for young children and missing items. The revised HEMOCABTM contained 108 questions grouped in 13 domains. HEMOCABTM was pilot-tested in 40 caregivers of CWH with a mean age of 39.32±8.9. The majority of CWH had hemophilia A (95%), were severely affected by hemophilia (77.5%) and 15% had inhibitors. Reliability estimation showed high internal consistency of total score with Cronbach's α=.97, and for 2 summary scores 'FREQUENCY' with α=.95 and 'BURDEN' α=.92; Cronbach's α for the sub domains ranged from α=.77 to .93. HEMOCABTM revealed good convergent validity with Impact on Family Scale (r=-.867 for total score). Known groups validity showed significant differences in all domains of HEMOCABTM, except for 'school' among caregivers of CWH with inhibitors vs. without. Type of treatment and disease severity showed some differences between groups. Based on item and scale analysis 49 items were deleted and the final HEMOCABTM consists of 59 items. **CONCLUSIONS:** HEMOCABTM is the first hemophilia-specific instrument for the assessment of caregiver burden and revealed good psychometric characteristics in terms of reliability and validity.

PRM81

USING THE CLINICAL SUMMARY SCORE FROM THE KANSAS CITY CARDIOMYOPATHY QUESTIONNAIRE AS AN ENDPOINT IN CLINICAL TRIALS: PSYCHOMETRIC SUPPORT

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OBJECTIVES: Symptoms and physical limitations can have an important impact on the day to day lives of heart failure patients. The Clinical Summary Score (CSS) of the Kansas City Cardiomyopathy Questionnaire (KCCQ), a patient-reported outcome instrument, provides a measure of symptoms and physical limitations associated with heart failure. The primary goal of this study was to evaluate the psychometric properties of the CSS and its utility as an endpoint in clinical trials. **METHODS:** Data from 3 randomized, controlled clinical trials with heart failure patients were included in analysis (ALOFT, PARAMOUNT, and PARADIGM trials). Studies were examined independently; within each study, data were collapsed across treatment groups. Study measures included the KCCQ, physician-rated New York Heart Association (NYHA) classification, patient global impression of change (PGIC), and/or NT-proBNP assay. **RESULTS:** Findings were similar across the 3 trials. Mean CSS scores at baseline ranged from 63-76 (on a 0-100 scale, with higher scores indicating better symptoms and physical functioning). Dimensionality assessments highlighted the complex nature of the scale, with evidence for first-, second-, and third-order factors. The CSS consistently discriminated between all four NYHA classifications (all pairwise comparisons p<.05). Correlations with BNP and NT-proBNP levels were statistically significant, but relatively small (-.12 and -.17 respectively). The CSS was sensitive to changes in patient status over time, as indexed by changes in the NYHA classification and PGIC. The responder definition – the amount of change within an individual patient that would be considered clinically meaningful - was in the range of 6 to 10 points, which is higher than what has been seen in studies of the KCCQ overall score. **CONCLUSIONS:** Data from multiple heart failure clinical trials confirm the psychometric characteristics of the CSS. This evidence supports the use of the CSS as an endpoint in clinical trials examining heart failure treatments.

PRM82

VALIDATION AND U.S. POPULATION NORMS OF HEALTH-RELATED PRODUCTIVITY QUESTIONNAIRE

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OBJECTIVES: To validate the Health-Related Productivity Questionnaire (HRPQ), a new health-related productivity instrument, and estimate the US population norms by age and gender. **METHODS:** An online survey was developed that consisted of four components: the HRPQ; a screener to determine relevant disease conditions-related and health-state questions; validated instruments such as Work Productivity and Activity Impairment (WPAI) and EQ-5D; and socio-demographic questions. The survey was administered by a third-party company for a 6-week period. Weightings were calculated to allow extrapolation of results from the 10,000 respondents to achieve values representing the general United States population. Validation analysis included concurrent and criterion validity, construct validity with group difference, extreme group comparison and factor analysis. Mean, median, standard error, 25th and 75thpercentiles were calculated for absenteeism, presenteeism measures for employed and household work and stratified by age, gender, age and gender. **RESULTS:** The HRPQ showed strong concurrent validity with WPAI (Pearson's r≥0.6, p-value<0.05). Correlations of total productivity at work and home from HRPQ with EQ-5D scores, were small to moderate, r=0.3-0.5 (p-value<0.05) and aligned with direction of the hypothesis. Several group difference analyses showed positive results for HRPQ. Presenteeism items heavily loaded on one factor and absenteeism and scheduled hours items loaded on second factor. General population estimates for average percent lost productivity at work was 14%, (absenteeism=4%, presenteeism=10%) and for household activities was 28% (absenteeism=18%, presenteeism=10%). Furthermore, 17% of people reported any kind of loss of workforce participation due to illnesses/treatments. Average work loss productivity was significantly higher in females vs males (16% vs 13%, p<0.05) and decreased with increase in age (20%, 17%, 14%, 11% and 7% in 18-29, 30-39, 40-49, 50-64 and >64 years old, respectively, p-value <0.05). **CONCLUSIONS:** HRPQ has good construct and criterion validity. Presenteeism remains higher for paid work, while absenteeism remains higher for unpaid work.

PRM83

MEASURING UPPER LIMB FUNCTION IN MULTIPLE SCLEROSIS: ENHANCING THE ABILHAND'S PERFORMANCE

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OBJECTIVES: ASCEND is a phase 3, randomized, double-blind, placebo-controlled trial assessing whether natalizumab slows disability progression in secondary progressive multiple sclerosis (SPMS) patients. The aim of this current analysis was to use Rasch Measurement Theory (RMT) methods to evaluate the ABILHAND (56 item version), a PRO assessing manual ability, in SPMS patients, and to explore an optimized scoring structure based on empirical post-hoc analyses. **METHODS:** Baseline blinded data from the 889 randomized patients in ASCEND were analyzed. In stage 1, RMT methods examined: scale-to-sample targeting, item fit, local dependency, and reliability. In stage 2, a post-hoc revision of the ABILHAND-56 scoring structure and conceptual grouping of items was conducted and re-evaluated using the same RMT methods. **RESULTS:** Stage 1 analyses showed adequate scale performance: minor item misfit (2/56); minimal dependency (4 pairs of items); good reliability (Person Separation Index = 0.93). However, there was ABILHAND-to-ASCEND sample mis-targeting (person location range [mean]: -7.31 to 1.83 [-3.62]; item location range [mean]: -5.75 to 4.27 [0.00]). In stage 2, all items were rescored on a dichotomous response scale (easy & difficult/impossible) in an attempt to improve targeting. Also, the 56 items were re-categorized into two conceptually clearer manual ability sub-scales: 'fine motor skills' and 'power'. These ABILHAND-56 revisions improved targeting: 'fine motor skills' (person range [mean]: -5.39 to 5.34 [-1.75]; item range [mean]: -4.15 to 3.54 [0.00]), 'power' (person range [mean]: -4.47 to 4.38 [-1.72]; item range [mean]: -4.15 to 3.54 [0.00]). Sample measurement and item fit were consistent with the original ABILHAND-56. **CONCLUSIONS:** The ABILHAND-56 revised scoring demonstrated improved psychometric performance and provides an initial evidence-base for the enhancement of ABILHAND-56's measurement performance in people with SPMS. Additional research will determine whether the two sub-scale structure of the revised ABILHAND-56 provides better interpretability of patient-reported manual ability.

PRM84

VALIDATION OF THE DEPRESSION AND FAMILY FUNCTIONING SCALE (DFFS)

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OBJECTIVES: Patient-reported outcomes (PROs) are necessary to assess disease impacts from the patient's perspective. In line with the Food and Drug Administration's (FDA's) guidance on PROs, the Depression and Family Functioning Scale (DFFS) was developed to assess the impact of major depressive disorder (MDD) on family functioning. Psychometric analyses were conducted to establish the reliability, validity, and responsiveness of the DFFS according to the FDA PRO guidance. **METHODS:** Data from PERFORM, a longitudinal multicenter, prospective, 2-year observational study in the United Kingdom and Spain, were analyzed (NBaseline=478; NMonth2=433). The 15 DFFS items use a 5-point rating scale to assess partner and family interactions and quality of relationships; higher scores indicate greater (worse) impacts. Test-retest reliability (intraclass correlations), construct validity (correlations and factor analysis), discriminating ability (analyses of variance), and responsiveness (effect size estimates) were evaluated. **RESULTS:** Factor analyses resulted in a single factor, confirmed by highly satisfactory Cronbach's alphas (0.85 at baseline, 0.89 at month 2). The DFFS demonstrated satisfactory test-retest reliability (intraclass correlation=0.75). Hypothesized correlations with other measures provided evidence of convergent and divergent validity. For example, the correlation of the DFFS with SF-12 mental component scores was –0.35 (baseline) –0.49 (month 2), and with SF-12 physical component scores, –0.05 (baseline) and –0.31 (month 2). Hypothesis tests were generally in the predicted direction and many were statistically significant, substantiating the discriminating ability of the DFFS. Effect size estimates of responsiveness were 0.44–0.84, demonstrating that the items were capable of detecting change. **CONCLUSIONS:** The psychometric analyses strongly support the reliability, validity, and responsiveness of the DFFS and its usefulness for assessing the impacts of depression on family functioning. It has the potential to provide important information not traditionally captured in clinical practice or research and will facilitate a more comprehensive evaluation of treatments of MDD.

PRM85

DOES DIFFERENTIAL FRAMING OF OPT-OUT ALTERNATIVES IN DISCRETE CHOICE EXPERIMENTS (DCES) MATTER? COMPARISON OF RANDOM UTILITY MAXIMIZATION (RUM) AND RANDOM REGRET MINIMIZATION (RRM) MODELS

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OBJECTIVES: We systematically investigate random utility maximization and random regret minimization modeling approaches to establish the impact of dif-

ferently framed opt-out alternatives in discrete choice experiments. We hypothesize that within the same experiment, when opt out alternatives are framed as a rejection of all the available alternatives, it is likely to have a detrimental impact on the performance of RRM model, while the performance of RUM model suffers more when the opt out is framed as a respondent being indifferent between the alternatives on offer. **METHODS:** We used two waves of data from a discrete choice experiment (N1 = 227; N2= 344); the first wave included an opt-out option implying a rejection of choice alternatives (i.e. none of these) while the second wave included an opt-out option implying a position of 'indifference' between the choice alternatives. We compared RUM and RRM models of different sophistications (e.g.; multinomial logit and mixed logit) in terms of parameter estimates, log likelihood and the Ben-Akiva and Swait test for non-nested models. **RESULTS:** In line with hypotheses, RUM models performed significantly better (P<0.01) than RRM models when opt–out alternative implied rejection of choice alternatives i.e. none of these. The RRM models performed significantly better (p<0.01) than the RUM models when the opt-out alternative implied a position of 'indifference'. RRM models had difficulty in handling the 'none of these' opt-out alternative while the RUM models had difficulty in handling the 'indifference' opt out alternative as was evident by the suspiciously large value of opt-out constant in the model parameter estimates for these cases. **CONCLUSIONS:** The framing of opt out alternatives influences the type of behavioral framework to be considered for modeling.

PRM87

THE INFLUENCE OF PAIN MANAGEMENT TREATMENT ATTRIBUTES ON CLINICAL DECISION MAKING IN POSTOPERATIVE PAIN MANAGEMENT

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OBJECTIVES: The introduction of patient satisfaction metrics for pain management as part of the Hospital Consumer Assessment of Healthcare Providers and Systems (HCAHPS) survey may impact decision making in postoperative pain management (POPM). The potential influence of patient satisfaction metrics, combined with evolving practice patterns that remain highly individualized to physicians and hospitals, creates a need to understand what treatment attributes influence physician selection of inpatient POPM strategies. **METHODS:** This adaptive approach conjoint analysis was a self-administered online survey of surgeons that treat POPM. Treatment attributes were developed based on qualitative interviews with clinicians, peer-reviewed literature, and input from the sponsor. The conjoint consisted of 9 attributes (patient mobility, adverse event profile, pain control, daily cost of pain medication, analgesic gaps, staff utilization, medication type, route of administration, and patient satisfaction), totaling 28 levels. When considering their answers, respondents were asked to think of a specific type of post-surgical patient. The study received central IRB approval. **RESULTS:** A total of 288 surgeons [male = 88%; mean (SD) years in practice 19(9)] were included in the study. Surgeons were screened to ensure they performed inpatient surgeries resulting in a need to provide inpatient POPM. Surgical subspecialties included general surgeons who performed abdominal/colorectal surgeries (n=97), gynecologic surgeons (n=95), and orthopedic surgeons (n=96). When selecting POPM strategies, surgeons valued (strength of preference) patient satisfaction with pain management (15.3%), a patient's ability to mobilize (14.9%); adverse event profile (14.6%), and pain control effectiveness (13.6%). There is variability in the utility rankings by specialty. **CONCLUSIONS:** When selecting a POPM strategy physicians consider a multitude of factors that go beyond the efficacy and safety profile of a solution. Patient satisfaction, which may be inclusive of but not limited to efficacy, will likely maintain or increase its influence on physician selection of POPM.

PRM88

DEVELOPMENT OF THE HYPERPHAGIA QUESTIONNAIRE FOR USE IN PRADER-WILLI SYNDROME CLINICAL TRIALS

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OBJECTIVES: The Hyperphagia Questionnaire (HQ) is a well-established, caregiver-reported questionnaire that focuses on food-seeking behaviors common among individuals with Prader-Willi syndrome (PWS). The goal of this research was to develop and psychometrically evaluate a modified version of the HQ for use within PWS clinical trials. **METHODS:** Following modification of the HQ based on industry and regulatory standards (e.g., Food and Drug Administration, 2009), the initial, 10-item version of the HQ for Clinical Trials (HQ-CT) was used in a phase 2 trial with the resulting data analyzed to identify the optimal scoring approach and evaluate instrument psychometric properties. Next, further evaluation by clinical experts in PWS and regulatory authorities resulted in removal of an item that focused more on patient-caregiver interactions than direct observation of patient behavior. Qualitative interviews were then conducted with PWS caregivers to inform final refinements, provide additional support for content validity, and assess usability of the electronic (ePRO) version of the 9-item measure. **RESULTS:** Initial modifications to the HQ reduced the recall period, optimized response scales, and limited item content to observable behaviors that have the potential to change during the course of a clinical trial. Psychometric evaluation supported the use of a single HQ-CT composite score as well as the questionnaire's reliability and validity. The HQ-CT total score was also able to demonstrate improvements in hyperphagia-related behavior between treatment groups. Finally, qualitative interviews with 6 caregivers supported the relevance and comprehensiveness of the content, item clarity, and the ePRO version of the HQ-CT's ease of use. **CONCLUSIONS:** Qualitative and quantitative evidence provide support for use of the 9-item HQ-CT total score for the assessment of food-seeking behaviors in PWS clinical trials. The HQ-CT is currently being used in a phase 3 PWS study.

PRM89

PATIENTS' EXPERIENCES WITH MYCOSIS FUNGOIDES/SÉZARY SYNDROME - CUTANEOUS T-CELL LYMPHOMA (MF/SS-CTCL): EVIDENCE FROM QUALITATIVE RESEARCH OF A PATIENT REPORTED OUTCOME (PRO) MEASURE DEVELOPED ON AN ONLINE RESEARCH PLATFORM

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OBJECTIVES: Patients' experience of MF/SS-CTCL remains understudied due to the rarity of the condition. Therefore, PatientsLikeMe (PLM) aimed to harness its internal platform, Open Research Exchange (ORE), to explore a novel method of data collection for the concept elicitation phase of PRO development. **METHODS:** Members of the MF/SS-CTCL community on PLM were invited to complete an online survey to share their experiences as patients. The 11 open-ended questions intended to elicit descriptions of MF/SS-CTCL quality of life impact, ability to cope and manage their condition, perception of symptoms and treatments, and sources of distress were administered to 21 patients. Of those, 10 participated in a follow-up interview. The data was analyzed by two trained raters and inter-rater reliability was used to validate and confirm their agreement. **RESULTS:** The sample (N=21) was predominantly female (67%), all white, and mostly reported MF (76%) rather than SS. The two raters achieved moderate to high inter-rater agreement for the qualitative analysis (0.68-0.80 Cohen's kappa for the 43 validated codes). Saturation for the sample was reached after 15 patients. Dominant themes that were elicited from the data were social impact (f=27), emotional impact (f=30), impact on daily activities (f=20), coping/management (f=64), severity and burden of symptoms and treatments (f=377, f=291), and overall treatment satisfaction (f=291). **CONCLUSIONS:** The online method proved to be an effective tool for understanding the experiences of a population living with a rare condition and complimented the interview data in eliciting dominant themes that were present in the literature. Utilizing ORE as the primary method of data collection, recruitment for this rare condition was found to be easier and faster than traditional methods and the sample size recruited was validated by concept saturation. Therefore, together, the survey and the follow-up interviews proved to be successful in eliciting relevant concepts from patients.

PRM90

CONTENT VALIDITY OF A PATIENT-REPORTED OUTCOME INSTRUMENT FOR PANCREATIC CANCER

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OBJECTIVES: To develop a patient-reported outcome (PRO) instrument that assesses important and relevant signs, symptoms, and impacts for adult patients with pancreatic cancer; and to evaluate patient comprehension and usability of the draft Pancreatic Cancer Symptom Assessment Form (PC-SAF) administered via electronic diary. **METHODS:** Concept elicitation (CE) interviews were conducted with 18 adult patients with pancreatic cancer from one of six US clinic sites to identify the most important and relevant signs, symptoms, and impacts of pancreatic cancer. Results from patient interviews, clinical expert interviews, and a targeted concept literature review informed the development of the draft PC-SAF, which was evaluated in cognitive interviews (CI) with N=14 adult patients with pancreatic cancer from one of two US clinic sites to assess patient comprehension and usability of the electronic instrument. **RESULTS:** Gastrointestinal (GI) symptoms (loss of appetite, early satiety, gas/bloating, constipation, diarrhea, nausea), pain and discomfort (in particular, in the abdomen and back), tiredness/low energy, weight loss and physical weakness, jaundice, and itching emerged as predominant concepts from the patient CE interviews. Patients commonly reported difficulty with activities of daily living, and noted physical (e.g., climbing stairs, walking, standing, carrying objects), work, social/relationship, emotional (e.g., depression, anxiety/fear), and sleep limitations. Patients also reported a negative impact on overall quality of life. Results from the CI indicate that patients had no comprehension problems for most items; however, minor revisions were made to the wording of a few items (e.g., early satiety, stomach pain, standing) to improve clarity. No concerns were raised about the usability of the electronic diary; patients reported that it was easy to use. **CONCLUSIONS:** Findings from this study support the content validity of the PC-SAF, a new PRO instrument to assess the key signs, symptoms, and impacts experienced by adult patients with pancreatic cancer.

PRM91

DEPRESSION AND QUALITY OF LIFE AMONG INDIVIDUALS WITH CHRONIC OBSTRUCTIVE PULMONARY DISEASE IN NORTH INDIA

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OBJECTIVES: Although presence of depression and poor health-related quality of life (HRQol) lead the poor clinical outcomes in patients with COPD. However, little is known about the prevalence or risk factors for depressive symptoms in patients with chronic obstructive pulmonary disease (COPD) in India. Therefore, the current study was conducted to estimates rates and factors associated with depression and poor health-related quality of life(HRQol) in the COPD patients attending a tertiary care health facility in north India. **METHODS:** A total of 126 patients (73.81% male) were enrolled using convenient sampling prospectively in this cross-sectional study. Eligible patients were assessed for socioeconomic status, anthropometric measures, COPD severity, dyspnea and health status using the Hindi version of St George's Respiratory Questionnaire (SGRQ). COPD was classified according to GOLD stages based on forced expiratory volume in one second (FEV1) in 126 stable patients. Depression was examined by administering the nine-item Hindi version of Patient Health Questionnaire-9 (PHQ-9). Linear regression model was used to identify the risk factors for depression and poor quality of life. Cronbach alpha was calculated to assess internal consistency of PHQ-9 and SGRQ. **RESULTS:** Among the patients