**PIH40**

**DESIGNING A DISEASE-SPECIFIC PATIENT REPORTED OUTCOME (PRO) FOR A RARE DISEASE: ASSESSING THE RELIABILITY, VALIDITY, AND RESPONSIVENESS OF THE HUNTER SYNDROME - FUNCTIONAL OUTCOMES FOR CHILDREN**

OBJECTIVES: Hunter syndrome (HS) is a rare X-linked progressive multi-systemic lysosomal storage disease. The 55 item child and 68 item parent versions of the 6 domain HS-FOCUS questionnaire were used to monitor the safety and efficacy with enzyme replacement therapy (ERT) in clinical trials. The objective was to validate HS-FOCUS according to the FDA PRO Guidance. METHODS: HS-FOCUS data collected from 50 children and 50 parents/ caregivers over 12 months extended the clinical trials’ data spanning 6 months. The inter-item correlations suggested that some items were less informative or redundant. The construct validity correlating the HS-FOCUS with Childhood Health Assessment Questionnaire (CHQ) showed moderate to high correlations in related concepts, especially in activity-related domains. Correlations with lung function (FEV1) ranged from 0.06 (School/Work) to 0.48 (Breathing). Significantly score differences were found in most domains among tertiles based on overall well-being and painVAS. Responsiveness showed large effect sizes, especially for Sleep, Breathing and School/work domains (0.50-1.07). CONCLUSIONS: Developmental disease-specific PROs for rare diseases is challenging due to the nature of the evidence base. This study demonstrates HS-FOCUS to be a reliable, valid, and responsive instrument which can be applied in clinical trials or disease registries. The questionnaire can be streamlined by reducing item redundancy without compromising its validity. Simultaneously, an item bank may be generated which could serve as a basis for developing questionnaires for other mucopolysaccharidosis disorders.

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**PIH41**

**ARE DIFFERENT SPANISH VERSIONS OF PRO MEASURES NECESSARY? THE CASE OF PROS FOR RARE DISEASES**

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OBJECTIVES: Numerous changes in wording for idiomatic reasons indicating respondents preferences point to the need for validated Spanish versions in collaboration with the author; 2) the effect of MCCs in addition to PCCs compared to PCCs only; and 3) the effect of MCCs in addition to PCCs compared to PCCs only. The expectation of patients in the treatment of BPH is an important variable that needs to be studied. The objective was to assess the expectation of changes in health-related behaviours on academic achievement and on HRQOL of children and adolescents aged 8–18 years in a 3 years follow-up study. METHODS: A representative sample of Spanish children and adolescents aged 8 – 18 years in a 3 years follow-up study. RESULTS: The expectation of patients in the treatment of BPH is an important variable that needs to be studied. The objective was to assess the expectation of changes in health-related behaviours on academic achievement and on HRQOL of children and adolescents aged 8–18 years in a 3 years follow-up study.

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**PIH42**

**ARE DIFFERENT SPANISH VERSIONS OF PRO MEASURES NECESSARY? THE CASE STUDY OF THE PedsQL™ 4.0 GENDER CORE SCALES**

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OBJECTIVES: The PedsQL™ Measurement Model is a modular approach to measuring health-related quality of life (HRQoL) in healthy children and adolescents and those with acute and chronic health conditions. Several Spanish versions for use in different countries were created by translation and back-translation. The objectives of this study were to compare these Spanish versions, quantify and qualify the differences and conclude on the need (not) for country-specific Spanish versions. METHODS: Our study focused on the Teen form (13-18) and included the following steps: 1. Collection of all available validated Spanish versions in collaboration with the author; 2. Identification of the differences in items, and response scales; and 3. Coding of the differences as Cultural (C), Lexical (L), Idiomatic (I) and Syntactical (S). RESULTS: Six validated Spanish versions were retrieved: Spanish for US, Spain, Argentina, Chile, Mexico and Peru. The Spanish version for US was the first translated, the US Spanish version served as a comparator. All versions showed differences. Spanish for Spain showed the highest number of differences (57), then Chile (47), Argentina (36), Peru (20) and Mexico (18). None of the differences were cultural. In 65% of the cases, the divergences were idiomatic. For instance, the expression “Se me hace difícil” (It is hard for me) used in US Spanish was replaced in all corresponding items by “Me cuesta” (Literally “It costs me”) in Spain, Argentina and Chile. The time recall sentence and item 3 of the School Functioning Scale showed most of the differences. One of the reasons could be the use of a very idiomatic form in the original US English version: “I have trouble keeping up.” It should be noted that items of the Emotional Functioning Scale showed very few differences. CONCLUSIONS: Numerous changes in wording for idiomatic reasons indicating respondents preferences point to the necessity of developing specific Spanish versions of the PedsQL™.

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**PIH43**

**CHANGES IN HEALTH-RELATED BEHAVIOURS AND THEIR IMPACT ON ACADEMIC ACHIEVEMENT AND HEALTH-RELATED QUALITY OF LIFE (HRQOL) AMONG SPANISH ADOLESCENTS**

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OBJECTIVES: The object of the study was to assess the effect of changes in health-related behaviours on academic achievement and on HRQOL of children and adolescents aged 8 – 18 years in a 3 years follow-up study. METHODS: A representative sample of Spanish children and adolescents aged 8 – 18 years was included. The HS-FOCUS questionnaire was used to evaluate item performance, reliability, validity, and responsiveness. RESULTS: Altogether, 55 children and 84 parents completed the HS-FOCUS at baseline and follow up visits. High percentage of lowest item response (> 60%) and high average inter-item correlations suggested that some items were less informative or redundant. The construct validity correlating the HS-FOCUS with Childhood Health Assessment Questionnaire (CHAQ) showed moderate to high correlations in related concepts, especially in activity-related domains. Correlations with lung function (FEV1) ranged from 0.06 (School/Work) to 0.48 (Breathing). Significantly score differences were found in most domains among tertiles based on overall well-being and pain VAS. Responsiveness showed large effect sizes, especially for Sleep, Breathing and School/work domains (0.50-1.07). CONCLUSIONS: Developmental disease-specific PROs for rare diseases is challenging due to the nature of the evidence base. This study demonstrates HS-FOCUS to be a reliable, valid, and responsive instrument which can be applied in clinical trials or disease registries. The questionnaire can be streamlined by reducing item redundancy without compromising its validity. Simultaneously, an item bank may be generated which could serve as a basis for developing questionnaires for other mucopolysaccharidosis disorders.

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**PIH44**

**HEALTH-RELATED QUALITY OF LIFE FOR PATIENTS WITH CHRONIC CONDITIONS: REVEALING THE PROFILE OF THE BURDEN ASSOCIATED WITH COMORBID PHYSICAL AND MENTAL CONDITIONS IN RESPONDENTS FROM FIVE EUROPEAN COUNTRIES**

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OBJECTIVES: Studies often examine the impact of specific chronic conditions (CC) in isolation or in conjunction with another specific CC. Our aims were to compare: 1) the effect of physical CCs (PCC) in addition to mental CCs (MCC) to the effect of PCCs only; 2) the effect of MCCs in addition to PCCs to the effect of MCCs only; and 3) effects across country. METHODS: Data from the 2010 EU National Health and Wellness Survey were used (N=57,805; France, Germany, Italy, Spain, and the UK). Respondents were categorised as: 1) ‘Healthy’ (no mental or physical CC); 2) ‘Physical’ (psychiatric indicated 1 physical but no mental CCs); 3) ‘Mental’ (psychiatric indicated 1 mental but no physical CCs); 4) ‘Physical and Mental’ (1+ mental + 1+ physical). Differences across groups were assessed using multivariable regression with SF-12v2 Health Survey summary measures (PCS & MCS) as outcomes and controlling for age and gender. RESULTS: Relative to ‘Healthy’ respondents, the presence of 1 PCC was associated with a significant decrement in both PCS (3.04 to 5.18, p<0.05) and MCS (1.23 to 2.53, p<0.05). The presence of a mental CC was also associated with decreased MCS (7.10 to -13.30, p<0.05) and PCS (1.23 to -2.53, p<0.05). Interestingly, the impact of co-morbid conditions, physical or mental, varied as a function of other existing conditions (physical or mental). The impact of adding 1 PCC to an existing MCC was associated with a larger decrement in PCS (-4.34 to -6.92, p<0.05) and MCS (-8.54 to -11.70, p<0.05) as compared to adding 1 PCC to those who were healthy. Results were consistent across gender and country. CONCLUSIONS: These findings (which are largely consistent an earlier US study) highlight the complexity of managing patients with co- or multi-morbid CCs as the measurable burden of CCs varies with the presence of other conditions.
OBJECTIVES: Herpes zoster (HZ) and post-herpetic neuralgia (PHN) are painful conditions that can have a substantial negative impact on patients' lives. UK-specific data on the incidence of HZ and PHN based on linked electronic health records (EHR) were limited, however. A large-scale UK cross-sectional study, therefore, has recently been developed to address these limitations and further inform the scientific community. Findings from this study are summarised.

METHODS: A combined total of 388 HerP and PHN patients over the age of 50 were recruited from 49 sites throughout the UK. Participants were required to complete verified PRO measures of pain and health-related quality of life (HRQoL), including the Zoster Brief Pain Inventory (ZBPI) and the Medical Outcomes Study Short-Form 36 (SF-36).

RESULTS: Pain was a prominent symptom among patients, with more than 50% reporting experiencing pain in the preceding 24 hours at levels typically considered to have a significant impact on HRQoL (i.e. 28/81 worst pain - 5). This was reflected in SF-36 domain and summary scores that were significantly lower in HZ and PHN patients compared to age-matched norms (p < 0.05). When compared to age-matched normative samples, meaningful differences were observed among HZ and PHN samples across SF-36 domains assessing aspects of physical and mental well-being. In both groups, HZVQ was inversely associated with levels of reported pain. CONCLUSIONS: Findings indicate that the acute presentation of HZ and the development of PHN are the most common complication of HZ and that can persist for several months, are painful experiences that can have a significant impact on the physical and mental wellbeing of HZ/PVN sufferers.

PIH46 SATISFACTION WITH PRESCRIPTION AND OVER-THE-COUNTER MEDICATIONS: RESULTS FROM A NOVEL PATIENT REGISTRY

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OBJECTIVES: There is a growing debate regarding switching prescription (Rx) drugs to over the counter (OTC) drugs for chronic conditions as a viable option in the brand lifecycle management and cost control. Further, patients are increasingly involved in making informed treatment-related decisions. Using a novel patient registry, the current study assessed satisfaction across ten widely used Rx and OTC medications and evaluated the impact of Rx versus OTC status on treatment satisfaction for these medications. METHODS: The registry recruited patients from multiple sources: physicians, pharmacists, and online referrals, to report ongoing medications on www.MediGuard.org. A random sample of these patients was contacted to complete the Treatment Satisfaction Questionnaire for Medication (TSQM) Version-1, a 14-item reliable and valid instrument to capture patients' satisfaction with medication. The TSQM yields scores on four domains: effectiveness, side-effects, convenience, and global satisfaction. The study included patients on any of the ten Rx Levodopa, metoprolol, aspirin, clopidigrel, oxaprozin, acetaminophen, aspirin, naproxen, cardiovascular diseases (12.5%). The leading specific causes of DALYs were unipolar depression (11.7%), dementias (10.0%), tuberculosis (4.7%) among males, and unipolar depression (11.7%), dementia (10.0%), hearing loss (4.2%) and cerebrovascular disease (3.5%) among females.

CONCLUSIONS: Measuring DALYs specifically for Spain provides a systematic analysis of health losses at a population level. The findings from this study suggest that chronic non-communicable diseases would benefit from increased evidence-based public health actions.