evaluations of PROs are commonly used in trials and are key to aid patient-centered decision-making.

POSB351
A PROSPECTIVE OBSERVATIONAL STUDY TO DETERMINE THE SAFETY, EFFICACY, AND TOXICITY PROFILE OF PACLITAXEL IN PATIENTS RECEIVING WEEKLY AND THREE WEEKLY DOSES

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Objectives: A prospective observational study to determine the safety, efficacy, and toxicity profile of paclitaxel in patients receiving weekly and three weekly doses. ***Methods:*** In this study, a sample size of 80 patients, in which 20 in each hands(once weekly paclitaxel and 3 weekly paclitaxel) were assessed for the dose intensity, density, efficacy and toxicity profile is narrow in Indian population.The details are regarding dose, indications,regimen and the duration were collected and were assessed for standard dose intensity and relative dose intensity. We also looked at the clinical effectiveness (in the span of 6 months time span) and the side effects encountered by the patients such as pheripheral neuropathy, neutropenia, blackening of nails. ***Results:*** In the study to understanding the effect of paclitaxel once weekly and 3 weekly once effect and assessing the standard and relative dose intensities it gives out the rationale and the dose intensities with prescribing the doses of paclitaxel once weekly, 80mg and 3 weekly once, 175mg,it was found that the standard dose intensity of once weekly paclitaxel is more with the 3 doses (240mg) that 3 weekly once dose. Side effects that was being particularly examined were peripheral neuropathy that accounted for 95% patients who were on once weekly paclitaxel and 80% in case of 3 weekly once paclitaxel. Blackening of nails were also found in both the groups and neutropenia was also identified to be a common adverse reaction. ***Conclusions:*** In the assessment determining the safety, efficacy, and toxicity profile of paclitaxel in patients receiving weekly and three weekly doses, weekly paclitaxel was found to have a good standard dose intensity. This little data will provide an meaningful insight to the future studies regarding the same due to the unavailability of literature.

POSB352
MINING SOCIALMEDIA TO UNDERSTAND UNMET NEED AND TREATMENT EXPERIENCE IN PATIENTS WITH ATYPICAL HEMOLYTIC UREMIC SYNDROME (AHUS)

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Objectives: The objective of this study was to understand the patient journey, quality of life (QoL), unmet needs, and experiences with available treatments from the aHUS patients' perspective. ***Methods:*** Data through social media sources including Twitter, forums, Facebook, blogs, and news outlets were analyzed to find aHUS relevant information from 2017-2020. The search was restricted to the English language for conversations originating from the US and the UK on open channels. Identified data from the searches were triaged by combination of automated relevancy keyword algorithm and manual review, and subsequently analyzed post anonymizing. ***Results:*** A total of 3,849 relevant posts were analyzed. Patients/caregivers/advocacy group conversations were mainly focused on sharing or seeking experiences, and information on aHUS. Delay in establishing a clear diagnosis of aHUS was a common theme encountered from patient journey posts and also one of the largest unmet medical needs. Multiple blood investigations and renal biopsy required for diagnosis caused emotional stress on patients/caregivers. Approximately 40% of the patients reported kidney dysfunction and many were on dialysis or underwent kidney transplants along with supportive therapies. Eculizumab is the first approved treatment for aHUS and a preferred option. However, concerns over the high drug cost, inconvenience in long-term management associated with intravenous infusion administration, uncertainty on the duration of treatment, and insurance coverage, were some of the unmet needs affecting the QoL of both patients and caregivers. Additionally, symptoms like fatigue, inability to resume work, lack of socializing, and sentiments like depression and anxiety, even after receipt of treatment with eculizumab, caused functional and emotional turmoil on the QoL. ***Conclusions:*** This study provides insights into how patients perceive and live with a rare disease like aHUS. These insights can help better understand patients' perspectives and unmet needs that can help with drug development and patient support programs.

POSB353
QUALITY OF LIFE AND SYMPTOM BURDEN OF PAROXYSMAL NOCTURNAL HEMOGLOBINURIA AMONG PATIENTS RECEIVING C5 INHIBITORS IN THE UNITED STATES AND EUROPE

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Objectives: Paroxysmal nocturnal hemoglobinuria (PNH) is a rare disease with the most prominent symptoms being hemolytic anemia, hemoglobinuria, thrombosis and fatigue. Complement C5 inhibition with eculizumab (Ecu) or ravulizumab (Ravu) is the current standard of care. Despite improvements in morbidity and mortality, some patients continue experiencing hemolysis and suboptimal quality of life (QoL). This study aimed at investigating the symptom burden of PNH in patients currently treated with a C5 inhibitor, globally. ***Methods:*** Adult patients with a self-reported diagnosis of PNH were recruited through patient advocacy groups in the United States (US) and Europe (EU: UK, France and Germany): Aplastic Anemia and MDS International Foundation, Patient Support, HPN France, and Stiftung Lichterzellen, respectively. Patients treated with Ecu or Ravu completed an online cross-sectional questionnaire. FACIT (Functional Assessment of Chronic Illness Therapy) was used to measure fatigue scores. Descriptive statistics are reported here. ***Results:*** A total of 122 adult patients in the US, and 71 in EU, were enrolled. Current medications included Ecu (EU:69%, US:29%) or Ravu (EU:31% US:71%); most patients were on treatment for ≥3 months: EU:98.6% US:96.7%. The most recent hemoglobin level was <12g/dL (mean±SD; EU:10.19±1.97, US:10.17±2.04) for the majority of patients (EU:85.7%, US:84.2%), indicating that most remained anemic. Among patients who ever received a red blood cell transfusion and were on treatment for ≥1 year, (EU:35.0%, US:35.2%) received ≥1 transfusion in the previous year, and 18% (EU) and 22% (US) received ≥4. Fatigue was the most commonly reported symptom (EU:63.4%, US:78.7%). FACIT scores (EU:35.0±13.7, US:32.1±13.4) were lower than in the general population (43.5) indicating higher levels of fatigue. Breakthrough hemolysis was experienced by US:40% and EU:39% patients. ***Conclusions:*** Despite C5 treatment, PNH patients experience a substantial burden of illness. There is a significant unmet need for treatments with better clinical and hematological outcomes for PNH patients.

POSB355
HEALTH-RELATED QUALITY OF LIFE IN FRIEDREICH ATAXIA: A SYSTEMATIC LITERATURE REVIEW

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Objectives: Friedreich ataxia (FA) is a rare autosomal recessive inherited disorder that progressively causes damage predominantly to the nervous and cardiac system. Quality of life is an important aspect in FA and accurate measures of health state utilities are required to evaluate the cost-effectiveness of therapies. This study aimed to review and synthesize existing evidence of health-related quality of life (HRQoL) and utility values for FA. ***Methods:*** A systematic literature review was performed in Medline and EMBASE up to January 2021 following PRISMA guidelines and guided by PICO criteria. Screening and data extraction were performed by two independent researchers. Studies published in English that evaluated HRQoL or utilities for patients with FA were included. Data extraction included study and patient characteristics, utility and HRQoL measuring approaches and their reported values. Activities of daily living (ADL) was not included in the analysis recognizing that ADL can contribute to HRQoL as one domain. ***Results:*** A total of 157 records were identified, of which 12 fulfilled the inclusion criteria: three reported utility values measured by EQ-5D, and ten reported HRQoL values from PedsQL (n=3) and SF-36 (n=7). The mean EQ-5D index was between 0.58-0.6. The PedsQL total scores ranged from 46-63 (parent proxy report) or up to 66 (child self-report). SF-36 was inconsistently reported across included publications – only two studies reported Mental Component Summary (48 and 52) and Physical Component Summary (33 in both). Physical function was the lowest score among all domains in reported studies, ranging from 16-26. No caregiver utilities were reported. ***Conclusions:*** Relatively few studies have been identified with a focus on generic HRQoL measurements in FA. Utilities were based on the EQ-5D as a preference-based measurement. Future initiatives to describe patient utility are needed. Caregiver utilities were not evaluated although they are an important aspect to evaluate the treatment in FA.

POSB356
HEALTH-RELATED QUALITY OF LIFE ACROSS KING'S AND MITOS STAGES AMONG AMYOTROPHIC LATERAL SCLEROSIS PATIENTS: RESULTS FROM A REAL-WORLD POINT-IN-TIME SURVEY

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Objectives: Amyotrophic lateral sclerosis is a rare, degenerative neuromuscular disease, leading to progressive loss of muscle function, and ultimately death. The King's and MiToS staging systems are used for assessing clinical progression, which is typically rapid. Real-world research quantifying health-related quality of life (HRQoL) at different disease stages is limited. ***Methods:*** The Adelphi ALS Disease Specific Programme (DSP™) is a point-in-time survey of neurologists and their ALS patients and caregivers in France, Germany, Italy, Spain, the UK, and the USA (Jul 2020–Mar 2021). Neurologists completed questionnaires on their consulting patients' demographics and clinical characteristics, including King's and MiToS staging. These ALS patients (and caregivers, if applicable) were invited to complete questionnaires