stage one of the brigatinib treatment arm of a multicenter, phase II, adaptive platform-basket trial for progressive, NF2-related, benign nervous system tumors (NCT04374305). Interviews included 7-point global impressions of change (GIC) ratings. Transcripts were coded by two analysts using a hybrid inductive/deductive framework; data was summarized in matrices to generate larger themes using the Framework Method. ***Results:*** 16/20 trial enrollees (69% female, ages 15-54 years) participated in interviews May 2021-March 2022. Participants rated their change in overall status after up to one year of treatment as much improved (3/16), minimally improved (10/16), no change (1/16) or minimally worse (2/16). Participants' GIC ratings were based on objective change in tumor volume, subjective change in symptoms, and intangible benefits such as sense of hope and agency in trying a new medication where otherwise there are no FDA-approved options. Most participants felt tumor volume stability was an improvement over previously accelerated growth rates; this importantly allowed them to avoid or postpone surgery to remove tumors. Participants also valued prevention of symptomatic decline, minimal impact of side effects on social roles and activities, and the convenience of oral medication. ***Conclusions:*** In contrast to NF2 clinical trials which have exclusively used tumor volume shrinkage of ≥20% as the primary outcome, trial participants report that the smallest worthwhile effect of an oral treatment for progressive NF2-related tumors is stabilization of tumor volume and symptoms, as long as there is also minimal impact of side effects on social roles and activities.

PCR84
THE URGENCY OF GENERATING REAL WORLD EVIDENCE DATA TO BRIDGE AN EXISTING GAP BETWEEN RANDOMIZED CLINICAL TRIALS AND CLINICAL PRACTICE IN RARE AND ORPHAN KIDNEY DISEASES: AN ONGOING RESEARCH FROM MAIN AVAILABLE PLATFORMS

Merante D[1] Schou H[2]
[1]*Global Clinical Development Expert, Zug, Switzerland,* [2]*Real World Evidence, Zug, Switzerland*

Objectives: This research describes tools and methodologies to fill an existing data gap between randomized phase 3 clinical studies (RCTs), Real World Evidence (RWE) data and clinical practice when a drug is near to be/already approved, but yet to be reimbursed with an assigned price for a rare or orphan kidney disease. ***Methods:*** This original research advocates the urgent use of new methodologies to predict when patients suffering from rare and orphan kidney diseases, can achieve a more adequate benefit/risk assessment profile of an approved/to be approved drug. For the scope, an ongoing extensive literature review from main available sources, including PUBMED, ENBASE, COCHRANE, CLINICALTRIALS.GOV, DR EVIDENCE and similarly available platforms with posted clinical study results will be completed. ***Results:*** Expected outcomes:

- Developing a novel methodology to fill the highlighted clinical gap, such as a risk assessment tool to assist Health Care Professionals (HCP) to support patient treatment algorithms.
- Developing a predictive/treatment algorithm based on both RCTs, RWE data from available registry platforms.
- Conducting a subsequent clinical study for verification and validation of clinical and laboratory results for improving therapeutic appropriateness of available and new medicines.
- Producing HCP feedback, truly patient-focused data, regulatory and payers' feedback.
- Incorporating new clinical evidence in current scientific management and treatment guidelines. ***Conclusions:*** Results of this ongoing qualitative research are expected in the first half of 2023. The main objectives are 1) to generate meaningful clinical data and an assessment tool based on new technologies to achieve a more adequate patient-focused benefit/risk drug profile in this diseases area. 2) to further improve quality of available data for future pharmacological treatments. Ultimately the research is expected to generate a truly patient-focused self-management approach through increased disease awareness and education and personalized treatment/risk disease algorithms. The generated results are expected to better reflect clinical practice and patient-focused disease management.

PCR85
SUSTAINED HUMANISTIC BURDEN AND WORK IMPACT IN ADULTS WITH SICKLE CELL DISEASE WITH RECURRENT VASO-OCCLUSIVE CRISES: RESULTS FROM A GLOBAL LONGITUDINAL SURVEY

Drahos J,[1] Boateng-Kuffour A,[1] Calvert M,[2] Valentine A,[3] Mason A,[4] Li N,[1] Pakbaz Z,[5] Shah F,[6] Ainsworth N,[7] Martin A[7]
[1]*Vertex Pharmaceuticals Incorporated, Boston, MA, USA,* [2]*University of Birmingham, Birmingham, UK,* [3]*Sick Cells, Washington, DC, USA,* [4]*Sickle Cell Care Manchester, Manchester, UK,* [5]*University of California Irvine School of Medicine, Orange, CA, USA,* [6]*NHS Blood and Transplant, London, UK,* [7]*QC Medica, Liverpool, UK*

Objectives: Sickle cell disease (SCD) is characterized by painful vaso-occlusive crises (VOCs). This longitudinal study examined the impact of SCD on participants' health-related quality of life (HRQoL) and work productivity. ***Methods:*** A longitudinal survey was administered at three timepoints (month 0 [M0], 3 months [M3], 6 months [M6]) in the US, the UK, France, Germany, and Italy and included multiple patient-reported outcome (PRO) measures: FACT-G, EQ-5D-5L, ASCQ-Me, FACIT-Fatigue (only M3), pain 11-point NRS, and WPAI. Key eligibility criteria included experiencing severe, recurrent VOCs, defined as ≥2 VOCs/year for 2 years that required an interaction with a medical facility and administration of pain medication or red blood cell transfusion. Descriptive analyses were conducted at M3. These data were compared with M0 data (previously reported), where available. ***Results:*** The survey was completed by 118 adult participants with SCD in the US (n=73), the UK, France, Germany, and Italy (n=45) at M3. Overall, 72.9% were female, their mean age was 34.7 years (standard deviation [SD] =11.2), and only 17.8% had full-time employment. Annual mean number of severe VOCs was 6.0 (SD=4.9). At M3, the mean FACT-G score was 59.8 (SD=19.6). Participants had a mean EQ-5D-5L utility score of 0.54 (SD=0.31). On the ASCQ-Me, participants reported worse pain, stiffness, emotional, and social impact compared with the US general SCD adult population. The mean FACIT-Fatigue score was 24.2 (SD=10.2) and comparable with that of patients with anemia and cancer (mean=23.9 [SD=12.6]). The average pain 11-point NRS score was 4.5 (SD=2.4). According to the WPAI, employed participants had 31.9% (SD=32.6%) absenteeism, 44.8% (SD=29.0%) presenteeism, and 63.7% (SD=28.1%) reduction in work productivity in the past week. PRO findings were comparable between M0 and M3. ***Conclusions:*** Despite receiving currently available treatments, adults with SCD and recurrent VOCs continue to experience substantial and sustained humanistic burden and indirect impacts.

PCR86
USING QUALITATIVE METHODS TO DEVELOP GROUNDED RELEVANT CONTENT FOR PREFERENCE SURVEYS IN A SPECIFIC RARE BLEEDING DISORDER (SRBD)

Grant S,[1] Gold A,[2] Pavisic I,[3] Doma R,[4] Rudell K[3]
[1]*Parexel International LLC, Uxbridge, Middlesex, UK,* [2]*Parexel, Lafayette, CA, USA,* [3]*Parexel, London, UK,* [4]*Parexel International, Durham, NC, USA*

Objectives: Literature attributing the factors to patient and Health Care Professional (HCP) treatment preferences in a specific rare bleeding disorder (sRBD) is lacking. This study (i) explores qualitatively with key medical experts the relevance of existing factors published in the literature attributing to sRBD (ii) identifies any other factors that pertain specifically to the rare bleeding disorder and (iii) aims to inform the development of a patient and HCP preference survey. ***Methods:*** A literature review preceded qualitative focus groups to understand the full scope of attributes and levels published in a sRBD. Findings of the literature review informed the design of the interview guide. Two focus groups of key medical experts from Japan, Italy, Germany, US, and UK were conducted. A content analysis was performed to identify common and unique concepts pertaining to a sRBD which further refined end selection of the attributes and levels for the patient and HCP preference surveys. ***Results:*** Focus groups resulted in the following eight attributes: Joint health improvement; number of treated bleeding events; concerns about safety of long-term treatment; risk of treatment side effects, frequency of intravenous infusions, hospital resource use, patient adherence, patient management costs considered as factors of relevance to bleeding disorders. Of these 8 factors, three were excluded (joint health improvement; patient adherence, patient management costs) as they were considered less relevant to the sRBD. The final selection of attributes and associated levels were further adjusted for appropriateness after two iterations of expert input. ***Conclusions:*** Qualitative input from medical experts in a disease area where little is known, has advanced understanding beyond the published literature about specific factors that might impact treatment preferences for a sRBD, both from a patient and HCP perspective. This information will improve the relevancy and appropriateness of content for the design of a future HCP and preference survey.

PCR87
IMPACT OF SIGNS AND SYMPTOMS OF CHRONIC REFRACTORY GOUT ON PATIENT HEALTH-RELATED QUALITY OF LIFE

Oladapo A,[1] Pillinger M,[2] Kragh N,[3] Johnson N,[4] Yousefian C,[5] Brooks D,[5] Sharp H,[5] Strand V[6]
[1]*Swedish Orphan Biovitrum AB, Boston, MA, USA,* [2]*NYU Grossman School of Medicine, New York, NY, USA,* [3]*Swedish Orphan Biovitrum AB, Stockholm, Sweden,* [4]*Endpoint Outcomes (a Lumanity company), Long Beach, CA, USA,* [5]*Endpoint Outcomes (a Lumanity company), Boston, MA, USA,* [6]*Stanford University, Palo Alto, CA, USA*

Objectives: Chronic refractory gout (CRG), also known as uncontrolled or refractory gout, is a debilitating inflammatory rheumatic condition. Patients experience severely painful and unexpected disease flares which, over the longer term, are associated with joint damage and chronic pain. The objective of this study was to gain an in-depth understanding of the impact that CRG has on patient health-related quality of life (HRQoL). ***Methods:*** A conceptual model of the impact of CRG on patient HRQoL was derived from a targeted literature review of CRG signs and symptoms covering the 10-years prior to August 2021. Twenty patients with CRG then participated in qualitative concept elicitation interviews; the results from these interviews were used to refine the conceptual model. Interviews included open-ended questions around frequency, duration, and severity for each identified concept, and bothersome impacts were scored on a scale of 0–10. ***Results:*** Most patients (60%) reported having >3 severe gout flares and many had 1–3 tophi (45%) within the past