



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

904. OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Development of a Patient Reported Outcome Daily Diary to Assess Symptom Burden in Sickle Cell Disease

Gale Harding, MA¹, Julia Correll, MPH¹, Nirmish Shah, MD², Danellys Borroto, PharmD³, George J Joseph, BPharm, MS, PhD^{3,4}, Ankur Dashputre, PhD MS³

¹Evidera, Bethesda, MD

²Duke University School of Medicine, Duke Medical Ctr, NC

³Bausch Health US LLC, Bridgewater, NJ

⁴BioNTech US Inc., Cambridge, MA

Introduction

Sickle cell disease (SCD) is a genetic vascular disease that causes pain, fatigue and multiple complications, ultimately affecting health-related quality of life of patients (Osunkwo et al. Am J Hematol 2021). The purpose of this study was to conduct qualitative interviews to inform the development of a patient reported outcome (PRO) daily diary to capture key symptoms and impacts of vaso-occlusive crisis (VOCs) experienced at home, based on methods consistent with US Food and Drug Administration (FDA) guidance for incorporating patient input and clinical outcome assessments into clinical research (FDA Fed Reg 2009, FDA PFDD Draft Guidance 3 2022).

Methods

This was a cross-sectional, non-interventional, qualitative study involving 16 concept elicitation (CE) interviews with adults (18-65 years), 15 cognitive interviews with adults (two rounds of n=12 and n=3), and 8 hybrid CE/cognitive interviews with adolescents (12-17 years). Up to 50% of adult participants were able to participate in both CE and cognitive interviews. Adult participants were recruited by clinical sites in the United States (US) and adolescent participants were recruited by a recruitment vendor. Patients were eligible to participate if they had a confirmed diagnosis of SCD, at least two VOCs in the past 12 months, a hemoglobin level of ≥ 4 gm/dL in past six months, and did not have a blood transfusion in the past three months. All interviews were conducted in English according to the approved study protocol and interview guide. Key symptoms and impacts were identified during CE interviews as those that were reported by at least 75% of sample and experienced daily by at least 50% of sample. The daily diary items were refined based on cognitive interviews to ensure patient understanding, medical expert input (n=1) and medical expert advisory board input (n=4) to ensure clinical relevance.

Results

Adult CE and cognitive interview participants (n=25 unique participants, with 6 participating in both CE and cognitive interviews) were mostly female (n=18; 72.0%), with a mean age of 38.0 ± 9.4 years (range 20 to 56 years). Among adolescent participants, nearly all were female (n=7, 87.5%), with a mean age of 14.6 ± 2.1 years (range 12 to 17 years). All adult and adolescent participants self-identified as African American. Twenty percent (n=5) of adult participants had a history of taking crizanlizumab-tmca, 16.0% (n=4) had a history of taking voxelotor, 8.0% (n=2) had a history of taking both, and 56.0% (n=14) had never taken crizanlizumab-tmca or voxelotor. Twelve and a half percent (n=1) of adolescent participants had a history of taking crizanlizumab-tmca, 50.0% (n=4) had a history of taking voxelotor, and 37.5% (n=3) had never taken crizanlizumab-tmca or voxelotor.

Findings from CE interviews identified the following key concepts: pain severity, pain crisis, tiredness, stiffness, impact on daily activities, impact on work or school, and impact on sleep. Four items were included based on medical expert/advisory board input to address swelling, ability to keep up with peers (for adolescents only), ability to control pain, and overall SCD severity. The current draft PRO daily diary includes four symptoms, four SCD-related impacts, and overall SCD severity, which will undergo further qualitative testing.

Conclusion

Results from this qualitative study provides further insight into the daily experience of patients with SCD, including their experience with pain crises. Findings from this study were used to develop a daily diary PRO tool for use in future clinical trials. Future work includes additional CE/cognitive interview with adults and adolescents to refine the PRO as needed and establish content validity and psychometric validation.

Disclosures Harding: *Bausch Health US LLC*: Consultancy. **Correll:** *Bausch Health US LLC*: Consultancy. **Shah:** *Pfizer*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months, Honoraria, Membership on an entity's Board of Directors or advisory committees, Other, Patents & Royalties, Research Funding, Speakers Bureau; *Bausch Health*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months; *Emmaus*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months, Membership on an entity's Board of Directors or advisory committees, Other, Speakers Bureau; *Bluebeard Bio*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months; *Vertex*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months; *Agios*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months; *Forma*: Consultancy, Current equity holder in private company, Current equity holder in publicly-traded company, Current holder of *stock options* in a privately-held company, Divested equity in a private or publicly-traded company in the past 24 months; *Alexion*: Membership on an entity's Board of Directors or advisory committees, Other, Speakers Bureau. **Joseph:** *BioNTech US Inc*: Current Employment, Current equity holder in publicly-traded company; *Bausch Health US LLC*: Ended employment in the past 24 months. **Dashputre:** *Bausch Health US LLC*: Current Employment, Current equity holder in publicly-traded company.

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