The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

904.OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Patient-Defined Symptom Burden and Treatment Utilization in a Home-Reported Outcomes (HRO) Study in Sickle Cell Disease (SCD)

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Introduction: Sickle cell disease (SCD) is a complex genetic disorder exhibited by diverse symptoms and treatment needs. Past studies have assessed the impact of SCD on patients' symptoms and disease management through patient-reported outcome (PRO) surveys, qualitative interviews, and eDiaries. Challenges associated with point-in-time data capture can hinder researchers' ability to capture the individualistic SCD experience. With the emergence of therapies like voxelotor (Oxbryta),which binds to sickle hemoglobin to reduce hemolysis and anemia, real-world evidence data can be used to close the gap between traditional endpoints and patients' experience of SCD.

Objectives: The Ascend Study was designed with a flexible patient-reported data collection approach to understand day-to-day burden by collecting home-reported outcomes (HROs) through the Folia Health mobile and web-based platform. The objectives of the Ascend Study were to 1) Identify the most important symptoms-of-interest to individuals living with SCD; and 2) Understand treatments-in-use among participants taking and not taking voxelotor.

Methods: The Advarra IRB-approved study used clinic referrals and community-based organizations to enroll a target of 75 participants. Individuals were eligible if diagnosed with SCD of any genotype residing in the US, aged 13 years and above (self-reported), and caregivers of children aged 4 to 12.

Participants selected symptoms and treatments relevant to their individual SCD experience to routinely track in the platform for 3 months on a weekly basis, reporting treatment usage and changes in symptom severity on a 1 to 10 Likert scale. Monthly check-in surveys were conducted to review overall health. Participants could monitor symptom severity history, set treatment reminders, and generate graphs and insight reports in-platform. The study team analyzed active participants, defined as those who completed the study start survey, routine tracking weekly, and at least 2 of 3 monthly check-in surveys.

Summary statistics of symptom tracking and treatment usage (including dosage) and subgroup analyses were performed for individuals taking voxelotor and those not taking it. Voxelotor usage was captured through an initial survey, including respective therapy start date (See Table 1).

Results: 66 (80.4%) of 84 participants enrolled became active participants and, on average, selected 4.16 (SD=3.25) treatments as part of their routine and tracked a total of 88 distinct treatments. Folic acid was the most frequently selected (n=56) and tracked treatment. 11 types of pain medications were tracked by participants. The average person tracked 4.05 (SD=3.24) different treatments during the study, 2.19 (SD=1.24) of those pain-related. On average, participants taking voxelotor had lower total exposure to NSAID and opioid medication.

Participants selected 6.41 (SD=2.84) symptom types and tracked 8.5 (SD=6.07) symptoms during the study. Majority of participants selected fatigue, chronic pain, and trouble concentrating (see Table 2). Participants on voxelotor tracked symptoms at an average of 3.58/10 severity (SD=1.07); those not on voxelotor tracked an average of 5.06/10 severity (SD=0.75). Fatigue was the most frequently tracked symptom overall (3,648 tracks), with an average of 55 fatigue tracks per participant over the 3-month period. This was closely followed by chronic pain (3,572 tracks). 91% (n=60) of all participants tracked each of these symptoms at least once. 'Chest tightness or pain' emerged as another important symptom that was tracked by 15 of 66 participants, at an average of 59 tracks per participant.

Conclusion: Overall, the ASCEND study underscores the importance of understanding the holistic experience of individuals with SCD and highlights the need for individualized care approaches. Collecting HROs through a novel mobile and web-based platform gives direct insight into the symptom profiles and treatment patterns in SCD, which can lead to a focus towards improved management strategies and better quality of life for individuals living with this complex genetic disorder. These findings have the potential to contribute to the development of more patient-centered approaches to SCD management and help inform providers about the most important symptoms and treatment needs of their patients.

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Table 2. Top 10 Initially Selected Symptoms at Routine Setup based on self-reported voxelotor (Oxbryta) use

	All Participants (N=66)		Taking Voxelotor (Oxbryta) (N=31)			Not Taking Voxelotor (Oxbryta) (N=35)		
Overall Rank	Symptom Name	# of Participants	Rank	Symptom Name	# of Participants	Rank	Symptom Name	# of Participants
1	Fatigue	60 (91%)	1	Fatigue	29 (94%)	1	Chronic Pain	33 (94%)
2	Chronic Pain	60 (91%)	2	Chronic Pain	27 (87%)	2	Fatigue	31 (89%)
3	Trouble concentrating	48 (73%)	3	Shortness of breath	20 (65%)	3	Trouble concentrating	29 (83%)
4	Shortness of breath	44 (67%)	4	Trouble concentrating	19 (61%)	4	Shortness of breath	24 (69%)
5	Joint pain	35 (53%)	5	Joint pain	17 (55%)	5	Joint pain	18 (51%)
6	Headache	28 (42%)	6	Anxiety	12 (39%)	6	Headache	17 (49%)
7	Bone pain	25 (38%)	7	Headache	11 (36%)	7	Bone pain	15 (43%)
8	Anxiety	23 (35%)	8	Bone pain	10 (32%)	8	Loss of appetite	12 (34%)
9	Loss of appetite	22 (33%)	9	Loss of appetite	10 (32%)	9	Anxiety	11 (31%)
10	Depression	19 (29%)	10	Depression	9 (29%)	10	Depression	10 (29%)

*Symptoms in **bold** reflect those that were suggested for tracking during routine setup.*

Table 1. Demographics of Participants in the Ascend Study by self-reported voxelotor (Oxbryta) use

	Taking voxelotor (Oxbryta) (N=31)	Not taking voxelotor (Oxbryta) (N=35)
Age: Mean (SD)	30.2 (10.6)	30.7 (11.4)
Recruitment channel: N(%)		
Clinic	15 (48.3)	7 (20)
Community	16 (51.6)	28 (80)
Genotype: N(%)		
HbSS	18 (58.0)	19 (54.3)
HbS_beta[+]-thalassemia	0 (0.0)	4 (11.4)
HbS_beta[0]-thalassemia	2 (6.5%)	2 (5.7)
HbSC	5 (16.1)	7 (20)
Unknown	4 (12.9)	2 (5.7)
None of the above	2 (6.5)	1 (2.9)
Length of time on voxelotor (Oxbryta): N (%)		
<3 months before study start	1 (3.2)	N/A
3-6 months before study start	5 (16.1)	N/A
6-12 months before study start	8 (25.8)	N/A
12-18 months before study start	6 (19.4)	N/A
18 months + before study start	11 (35.5)	N/A

Figure 1