

# BSH18

## Paediatrics

### BSH18-522

#### Prevention of Morbidity in Sickle Cell Disease Phase II (POMS 2b paediatric cohort): Improvement of Pain and Quality of Life in Children with Sickle Cell Disease with Auto-adjusting Continuous Positive Airways Pressure

Baba Inusa<sup>1</sup>, Hanne Stotesbury<sup>\*2</sup>, Melanie Koebel<sup>2</sup>, Jamie Kawadler<sup>2</sup>, Jo Howard<sup>3</sup>, David Rees<sup>4</sup>, Subarna Chakravorty<sup>4</sup>, Maria Pelidis<sup>1</sup>, Swee Lay Thein<sup>5</sup>, Fenella Kirkham<sup>2</sup>, April Slee<sup>2</sup>

<sup>1</sup>Evelina Children's Hospital, <sup>2</sup>UCL Great Ormond Street Institute of Child Health, <sup>3</sup>Guy's Hospital, <sup>4</sup>King's College Hospital, London, United Kingdom, <sup>5</sup>National Heart, Lung, and Blood Institute, Bethesda, United States

**Please indicate your preferred method of presentation:** Oral or Poster

**Has this abstract been presented at a British Haematology meeting before?:** No

**Has this abstract been presented at an overseas meeting?:** No

**Please select your position from the list:** Consultant

#### **Abstract Content: Background:**

Low daytime and night-time oxygen saturation compounded by obstructive sleep apnoea (OSA) may impact patient-reported outcomes, e.g. sleep and pain. Continuous Positive Airways Pressure (CPAP) is an accepted treatment for OSA in the general population and prevents dips in oxygen saturation. A secondary aim of this single-blind, randomised, controlled phase II trial is to compare Auto-adjusting CPAP (APAP) with standard care to standard care alone in children with HbSS to determine whether APAP improves quality of life, sleep, and pain scores.

#### **Methods:**

Subjects were randomised to APAP or Standard Care. Pain assessed by the Pain Burden Inventory and Quality of Life (QoL) assessed by the PedsQL were collected at baseline and after 6 months of treatment.

Due to low pain and disease burden at baseline, outcomes were dichotomised. For PedsQL subscales and total score, the minimum clinically important difference was calculated as MCID=Baseline Standard Deviation /2. If the baseline score was within the MCID of the maximum 100 points, no clinically important improvement was possible. However, it is clinically significant to maintain high QoL scores, so a positive outcome was defined as maintaining a score within the MCID of 100. For subjects with a baseline score < MCID, an improvement of at least MCID was defined as a good outcome. Worsening or improvement less than the MCID was considered a poor outcome.

For Pain Burden Inventory, a good outcome was defined as indicating "never" at baseline and follow-up, or reduction of at least 1 frequency category.

Proportions were compared using Fisher's Exact Test.

#### **Results:**

Most of the PedsQL subscales numerically favoured APAP but did not reach statistical significance. Pain impact significantly improved (for APAP 78.6% improved or maintained compared with 33.3% for standard care;  $p=0.025$ ). Results were in the same direction but not statistically significant for Pain Hurt (64.3% vs 53.3%), Worry I (78.6% vs 60%), Worry II (85.7% vs 66.7%), and Emotions (50% vs 38.5%), while there was no difference in Pain management (both 57.1%). The Treatment subscale favours placebo, with 35.7% improved or maintained in the APAP arm compared with 53.3% in the placebo arm and the same was true for Communication I (64.3% vs 80%) and Communication II (42.9% vs 46.7%). For the Pain Burden Inventory, there was a higher proportion who improved or maintained status for APAP compared with standard care for Days with pain (62.5% vs 56%), Slept poorly (78.9 vs 73.7%), Missed School (78.9% vs 77.8%), Had to leave school early (100% vs 93.3%), and Unable to do things (78.9 vs 73.7%) while Trouble with self care (83.3% vs 87.5%) and Felt sad, mad or upset (75% vs 87.5%) favoured the standard care arm. The total PEDS-QL score favoured APAP (57.1 vs 40%) as did the total for the Pain Burden Inventory (65.2% vs 56%) but neither was statistically significant.

#### **Discussion:**

Pain burden was low at baseline and APAP did not make it worse. Improving sleep-disordered breathing appear to be associated with improvement in patient reported outcomes, particularly in terms of pain. It is unsurprising that the Treatment subscale of the PEDS-QL favoured placebo as the children on APAP now had a complicated procedure, including wearing a mask, every night. The focus should be on improving the intervention so that it is acceptable to the majority of children who could benefit.

**Disclosure of Interest:** None Declared

**Keywords:** APAP, paediatric, Pain, Quality of Life, sickle cell anaemia, Sickle cell disease