TABLE 1. Baseline characteristics of the study population.

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | Total population  (N = 94) | | No hospitalization(s) for VOC  (N = 57) | Hospitalization(s) for VOC  (N = 37) | p-value |
| Age – (IQR) | 11.8 (9.3-14.1) | | 13.0 (9.5-15.0) | 10.7 (9.3-14.1) | 0.01 |
| Gender (%)   * Male * Female | 60 (63.8)  34 (36.2) | | 36 (63.2)  21 (36.8) | 24 (64.9)  13 (35.1) | 0.87 |
| Genotype (%)   * HbSS/HbS β0 * HbSC/HbS β+ * Others | 57 (60.6)  35 (37.2)  2 (2.1) | | 24 (42.1)  31 (54.4)  2 (3.5) | 33 (89.2)  4 (10.8)  0 (0.0) | <0.01 |
| α-thalassemia(%) | | 33 (35.1) | 20 (35.0) | 13 (35.1) | 0.996 |
| Steady state laboratory  values – (IQR/SD) | | | | | |
| - Hb [mmol/L] | 6.2 (0.1) | | 6.5 (0.2) | 5.7 (0.1) | 0.00 |
| - MCV [fL] | 77.3 (70.7-88.3) | | 76.4 (70.8-86.8) | 88.1 (74.2-95.6) | 0.02 |
| - HbF [%] | 8.6 (3.3-13.9) | | 4.7 (2.5-11.6) | 10.5 (3.7-13.9) | 0.28 |
| - HbS [%] | 79.4 (68.6-87.1) | | 76.5 (44.8-86.6) | 81.3 (77.7-87.6) | 0.10 |
| Hydroxyurea (%)   * Start age of hydroxyurea – (IQR) | 47 (50.0)  11.8 (0.6) | | 18 (31.6)  11.9 (0.8) | 29 (78.4)  11.6 (0.9) | <0.01  0.87 |
| Number of completed  PedsQL per patient 4 (3-6) 6 (4-8) 3 (2-5)   * Median (IQR) | | | | | |
| Number of hospitalizations – (IQR)   * 1 (%) * 2-4 (%) * ≥ 5 (%) |  | |  | 2 (1-4)  15 (40.5)  15 (40.5)  7 (19.0) |  |
| Severity of hospitalization (%) | | | | | |
| Mild |  | |  | 39 (32.0) |  |
| Moderate |  | |  | 53 (43.4) |  |
| Severe |  | |  | 30 (24.6) |  |

Abbreviations: fL, femtolitre; IQR, interquartile range; N, number; mmol/L, millimol per litre; MCV, mean corpuscular volume; SD, standard deviation; VOC, vaso-occlusive crisis

TABLE 2. Mean HRQoL scores of the study population as measured by PedsQLTM.

|  |  |  |  |
| --- | --- | --- | --- |
| HRQoL score –  Mean (SD) | No hospitalization(s) for VOC  (n = 57, PedsQL n = 237) | Hospitalization(s) for VOC  (n = 37, PedsQL n = 218) | p-value |
| Total | 80 (1.3) | 75 (1.5) | 0.02 |
| Physical | 81 (1.4) | 73 (1.9) | 0.00 |
| Emotional | 79 (1.9) | 76 (2.3) | 0.20 |
| Social | 87 (1.4) | 84 (1.7) | 0.09 |
| School | 69 (1.6) | 67 (1.9) | 0.39 |
| Psychosocial | 79 (1.4) | 75 (1.6) | 0.13 |

Abbreviations: N, number; PedsQL, Pediatric Quality of Life Inventory; SD, standard deviation; VOC, vaso-occlusive crisis;

TABLE 3. Results of impact of occurrence of hospitalization for VOC on HRQoL scores in the last 3, 6, 9 and 12 months adjusted for age and genotype.

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | β | Sig. | CI  LCI UCI | |
| *Three months interval (PedsQL n=397)* | | | | |
| Total | -4.8 | 0.11 | -10.7 | 1.1 |
| Physical | -5.6 | 0.10 | -12.4 | 1.1 |
| Emotional | -5.3 | 0.20 | -13.6 | 2.9 |
| Social | -3.3 | 0.19 | -8.3 | 1.6 |
| School | -5.8 | 0.13 | -13.5 | 1.9 |
| Psychosocial | -4.5 | 0.14 | -10.6 | 1.6 |
| *Six months interval (PedsQL n=422)* | | | | |
| Total | -4.2 | 0.04 | -8.3 | -0.2 |
| Physical | -6.1 | 0.02 | -11.0 | -1.3 |
| Emotional | -4.8 | 0.14 | -11.2 | 1.6 |
| Social | -1.3 | 0.50 | -5.4 | 2.7 |
| School | -3.3 | 0.21 | -8.5 | 1.9 |
| Psychosocial | -2.9 | 0.20 | -7.3 | 1.6 |
| *Nine months interval (PedsQL n=438)* | | | | |
| Total | -3.6 | 0.05 | -7.2 | -0.10 |
| Physical | -5.1 | 0.02 | -9.3 | -0.8 |
| Emotional | -4.4 | 0.12 | -10.0 | 1.1 |
| Social | -0.9 | 0.62 | -4.3 | 2.6 |
| School | -3.4 | 0.14 | -8.0 | 1.2 |
| Psychosocial | -2.81.6 | 0.15 | -6.6 | 1.1 |
| *Twelve months interval (PedsQL n=455)* | | | | |
| Total | -3.2 | 0.05 | -6.4 | 0.0 |
| Physical | -3.8 | 0.04 | -7.5 | -0.2 |
| Emotional | -3.5 | 0.20 | -9.1 | 2.0 |
| Social | -0.6 | 0.73 | -4.1 | 2.9 |
| School | -4.4 | 0.03 | -8.4 | -0.4 |
| Psychosocial | -2.8 | 0.13 | -6.4 | 0.9 |
| Dependent variable: all PedsQL scores  Linear mixed model with random intercept and random slope (for time) and covariance between random intercept and random slope.  Abbreviations: β, regression coefficient; CI, confidence interval; LCI, lower confidence interval; n, number; PedsQL, Pediatric Quality of Life Inventory; SE, standard error; Sig., significance; UCI, upper confidence interval | | | | |

TABLE 4. Results of impact of frequency of hospital admissions for VOC on all HRQoL scores in the last 12 months adjusted for age and genotype.

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | β | Sig. | CI  LCI UCI | |
| *Twelve months interval (PedsQL n = 455)* | | | | |
| Total | -2.3 | 0.04 | -4.4 | -0.2 |
| Physical | -2.7 | 0.05 | -5.4 | 0.0 |
| Emotional | -2.7 | 0.15 | -6.5 | 1.1 |
| Social | -1.7 | 0.08 | -3.7 | 0.3 |
| School | -2.6 | 0.05 | -5.2 | 0.0 |
| Psychosocial | -2.0 | 0.10 | -4.3 | 0.4 |
| Dependent variable: all PedsQL scores  Linear mixed model with random intercept and random slope (for time) and covariance between random intercept and random slope.  Abbreviations: β, regression coefficient; CI, confidence interval; LCI, lower confidence interval; n, number; PedsQL, Pediatric Quality of Life Inventory; Sig., significance; UCI, upper confidence interval | | | | |