

Health-related quality of life and long-term joint damage in people with severe haemophilia A in Brazil (without Inhibitors)

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Introduction

- The health-related quality of life (HRQoL) of people with severe hemophilia A (PwSHA) is impacted by many factors including bleeding, treatment burden, and long-term joint damage
- This analysis aims to describe the impact of joint damage on HRQoL in a sample of adult (18+) PwSHA in Brazil receiving prophylaxis without inhibitor diagnosis

Methods

- Brazilian clinical data for adults were obtained via electronic case record forms (eCRF) collected during the CHES LATAM burden-of-illness study between September 2020 and May 2021

HRQoL

- EQ5D-5L data was captured cross-sectionally from a corresponding adult public patient involvement and engagement (PPIE) survey
- Supplemental data was captured from adult PwSHA via the CHES LATAM+ study using the same PPIE materials (between September and December 2022) and pooled with the CHES LATAM PPIE data
- EQ5D-5L index scores were calculated using a Peruvian value set as:
 - No Brazilian value set was available
 - Peru was used as an available South American EQ5D-5L value set

Joint Damage

- Long-term joint damage was captured in the eCRF and CHES LATAM+ as problem joints, defined as having chronic joint pain and/or limited range of movement due to compromised joint integrity.¹
 - PwSHA were stratified by number of problem joints in categories of 0 PJ, 1 PJ, 2 PJ, 3 PJ, and 4≤ PJ
- PwSHA not receiving prophylaxis or with active inhibitor were excluded from the analysis.

Patient-reported outcomes

- Annual bleed rate (ABR), adherence (where 0 is “Not at all” and 10 is “Fully”), and pain (where 0 is “The least pain you can imagine ” and 100 is “The least pain you can imagine ”) were all captured from the CHES LATAM and CHES LATAM+ PPIEs and are reported here by PJ.

Results

Clinical data – Joint damage

- Of the Brazil eCRF cohort, 123 PwSHA reported no active inhibitor and were receiving factor VIII prophylactically.
- Of these 61 had 0 PJ, 36 had 1 PJ, 16 had 2 PJ, 5 had 3 PJ, 5 had 4≤ PJ
- Table 1 presents the numbers and key demographics of the PPIE populations of CHES LATAM, CHES LATAM+ and the pooled sample.

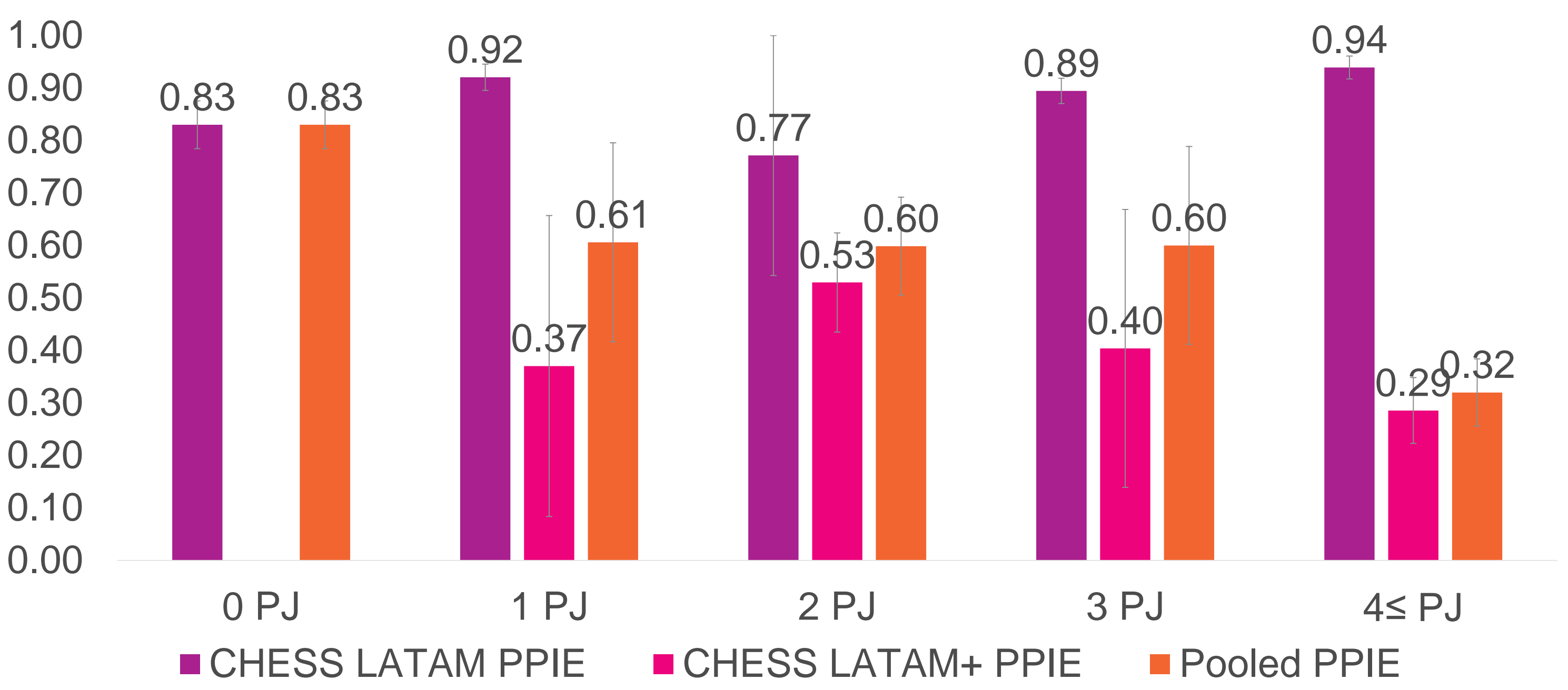
Table 1. Demographics (PPIE)

Demographic	0 PJ	1 PJ	2 PJ	3 PJ	4≤ PJ
CHES LATAM PPIE, n (%)	7 (43)	3 (19)	2 (13)	2 (13)	2 (13)
Age, mean years (sd)	32.8 (9.2)	31.1 (11.1)	27.3 (13.1)	32.5 (0.4)	34.0 (4.5)
Height, mean metres (sd)	1.72 (0.05)	1.74 (0.05)	1.79 (0.01)	1.75 (0.07)	1.72 (0.04)
Weight, mean kg (sd)	71.3 (7.6)	67.7 (3.5)	76.5 (7.8)	78.5 (0.7)	74 (8.5)
Annual bleed rate, mean (sd)	6.4 (6.0)	11.7 (11.5)	12.5 (17.7)	9.5 (0.7)	25 (35.4)
CHES LATAM+ PPIE, n (%)	0 (0)	4 (8)	5 (10)	3 (6)	36 (75)
Age, mean years (sd)	-	26.3 (5.9)	30.0 (8.3)	34.6 (7.0)	36.7 (9.0)
Height, mean metres (sd)	-	1.69 (0.24)	1.72 (0.73)	1.76 (0.56)	1.73 (0.66)
Weight, mean kg (sd)	-	70.8 (9.3)	75.6 (8.5)	84 (21.2)	81.0 (21.5)
Annual bleed rate, mean (sd)	-	8.3 (8.0)	6.6 (4.3)	16.7 (15.3)	13.6 (25.6)
Pooled PPIE, n (%)	7 (11)	7 (11)	7 (11)	5 (8)	38 (59)
Age, mean years (sd)	32.8 (9.2)	28.3 (8.1)	29.2 (8.7)	33.8 (5.1)	36.6 (8.8)
Height, mean metres (sd)	1.72 (0.05)	1.71 (0.05)	1.74 (0.07)	1.76 (0.05)	1.73 (0.06)
Weight, mean kg (sd)	71.3 (7.6)	69.4 (7.1)	75.9 (7.6)	81.8 (15.3)	80.7 (21.1)
Annual bleed rate, mean (sd)	6.4 (6.0)	9.7 (8.9)	8.3 (8.5)	13.8 (11.5)	14.2 (25.7)

HRQoL and problem joints

- Mean EQ5D-5L index score across PwSHA in the pooled sample was 0.41 (sd 0.41)
- PwSHA with 0 PJ had the highest mean EQ5D-5L index score (0.83) while PwSHA with 4≤ PJ had the lowest (0.32)
- Figure 1 presents EQ5D-5L scores of the CHES LATAM PPIE, CHES LATAM+ PPIE, and pooled PPIE sample stratified by number of problem joints.

Figure 1. EQ5D-5L index scores by number of PJ



Patient-reported outcomes and problem joints

- ABR was highest in those PwSHA with 4≤ PJ in the CHES LATAM (25.0) and pooled (14.2) PPIE samples. In contrast PwSHA with 3 PJ had the highest ABR in the CHES LATAM+ sample (16.7)
- Adherence increased gradually in the presence of more PJs in the CHES LATAM sample, however fluctuated between PJ groups in both the CHES LATAM+ and pooled PPIE samples
- Pain was highest in the 0 PJ group (61.3) however those with 4≤ PJ showed high pain levels in all three samples
- Table 2 presents patient-reported outcomes of the CHES LATAM PPIE, CHES LATAM+ PPIE, and pooled PPIE sample stratified by number of problem joints

Table 2. Patient-reported outcomes

Patient-reported outcome	0 PJ	1 PJ	2 PJ	3 PJ	4≤ PJ
CHES LATAM PPIE, n (%)	7 (43)	3 (19)	2 (13)	2 (13)	2 (13)
ABR, mean (sd)	6.4 (6.0)	11.7 (11.5)	12.5 (17.7)	9.5 (0.7)	25.0 (35.4)
Adherence, mean (sd)	3.0 (1.0)	5.3 (1.2)	5.0 (1.4)	5.0 (0)	6.0 (0)
Pain, mean (sd)	61.3 (18.7)	42.7 (25.8)	44.0 (50.9)	35.0 (21.2)	65 (49.5)
CHES LATAM+ PPIE, n (%)	0 (0)	4 (8)	5 (10)	3 (6)	36 (75)
Annual bleed rate, mean (sd)	-	8.3 (8.0)	6.6 (4.3)	16.7 (15.3)	13.6 (25.6)
Adherence, mean (sd)	-	4.0 (0)	4.8 (1.1)	3.0 (1.0)	4.2 (1.4)
Pain, mean (sd)	-	45.3 (26.5)	39.2 (34.6)	49.3 (35.0)	58.1 (23.0)
Pooled PPIE, n (%)	7 (11)	7 (11)	7 (11)	5 (8)	38 (59)
Annual bleed rate, mean (sd)	6.4 (6.0)	9.7 (8.9)	8.3 (8.5)	13.8 (11.5)	14.2 (25.7)
Adherence, mean (sd)	3.0 (1.0)	4.6 (1.0)	4.9 (1.1)	3.8 (1.3)	4.3 (1.4)
Pain, mean (sd)	61.3 (18.7)	44.2 (24.0)	40.6 (35.1)	43.0 (27.9)	58.4 (23.8)

Conclusions

- Pooled EQ5D-5L results indicated substantial impact on HRQoL as long-term joint damage increased.
- The analysis is the first to explore long-term joint damage and patient-reported outcomes using the problem joint metric in Brazil for PwSHA.
- PwSHA (CHES LATAM+) reported more problem joints than clinicians (CHES LATAM) suggesting that not all the burden associated with severe haemophilia A is seen at clinic.

References

1. McLaughlin, P., Hermans, C., Asghar, S., Burke, T., Nissen, F., Aizenas, M., Meier, O., Dhillon, H. and O'Hara, J., 2020. Problem joints and their clinical and humanistic burden in children and adults with moderate and severe hemophilia A: CHES paediatrics and CHES II. Blood, 136, pp.33-34.

Disclosures

Funding for this study was provided be BioMarin Pharmaceutical Inc. Medical writing support was provided by Prime HCD and funded by BioMarin Pharmaceutical Inc.