Poster No. 2532

Problem Joints and their Clinical and Humanistic Burden in Children and Adults with Moderate and Severe Hemophilia A: CHESS Paediatrics and CHESS II

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Disclosures

PM: no relevant financial relationships to disclose; **CH**: has provided consultancy and served on a speakers bureau for Bayer, Pfizer, Shire, a Takeda company, Sobi, Biogen, CAF-DCF, CSL Behring, LFB, Novo Nordisk, Roche and Octapharma, has served on a speakers bureau for Kedrion, and has received research funding from Bayer, Pfizer, Shire, a Takeda company and Sobi; **TB**: employee of HCD Economics and the University of Chester, and has provided consultancy for F. Hoffmann-La Roche Ltd**; SA**: employee of HCD Economics; **FN**: employee of F. Hoffmann-La Roche Ltd, has provided consultancy for Actelion, and has received research funding from Novartis and GSK; **HD**: employee of HCD Economics and has received research funding from F. Hoffmann-La Roche Ltd**; MA** and **OM**: employees of and holders of stock in F. Hoffmann-La Roche Ltd**; JO**: employee of and holder of stocks in HCD Economics, and has provided consultancy for F. Hoffmann-La Roche Ltd**.**

Key takeaways

- Patient-centric outcome measures are required to gain an understanding of the wider burden associated with chronic joint morbidity
- A correlation between clinical/humanistic burdens with chronic joint morbidity, as measured by 'problem joints', was found across children and adults with severe **and** moderate hemophilia A

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The CHESS II study collects real-world data on the burden-of-illness in adult PwHA

Background

- Chronic joint damage is most often associated with SHA; however, more recent research has illustrated that people with MHA also experience hemophilic arthropathy and functional impairment[1,2]
- Recent data from the Joint Outcome Continuation Study also highlight the need to manage joint health in children as well as adults[3]

Objective:

To gain a patient-centric understanding of the clinical and humanistic burden of joint damage, as measured by problem joints, in children (1–7) with **MHA** or **SHA** in **CHESS Paeds**, and adults (20+) with **MHA** or **SHA** in **CHESS II**

Methods

- Data were drawn from 'The Cost of Haemophilia in Europe: a Socioeconomic Survey' (*CHESS Paeds* and *CHESS II*) studies of children and adults with hemophilia A and B, from across five and eight European countries, respectively
- We report 12 months' retrospective physician-reported data on ABR, prevalence of (ISTH-defined) target joints (TJ) and hospitalizations, and patient/carer-reported EQ-5D-Y/5L, stratified by **MHA** and **SHA** and by number of problem joints (PJ)*: 0 PJ, 1 PJ, and 2+ PJ

To account for the possibility that persons aged 18 or 19 in CHESS II may have participated in CHESS Paeds, these individuals were excluded from the analysis.
*A problem joint (PJ) is defined as having chronic joint pain and/or limited range of movement due to compromised joint integrity (i.e. chronic synovitis and/or hemophilic arthropathy).
ABR, annualized bleed rate; MHA, moderate hemophilia A; PJ, problem joints; PwHA, persons with hemophilia A; SHA, severe hemophilia A; TJ, target joints.

1. Berntorp E, et al. *Haemophilia* 2017;23:105–14;
2. Scott MJ, et al. *Haemophilia* 2019;:205–12;
3. Warren BB, et al. *Blood Adv* 2020; 4:6–9.

Baseline characteristics and prevalence of problem joints (0 PJ, 1 PJ, 2+ PJ)

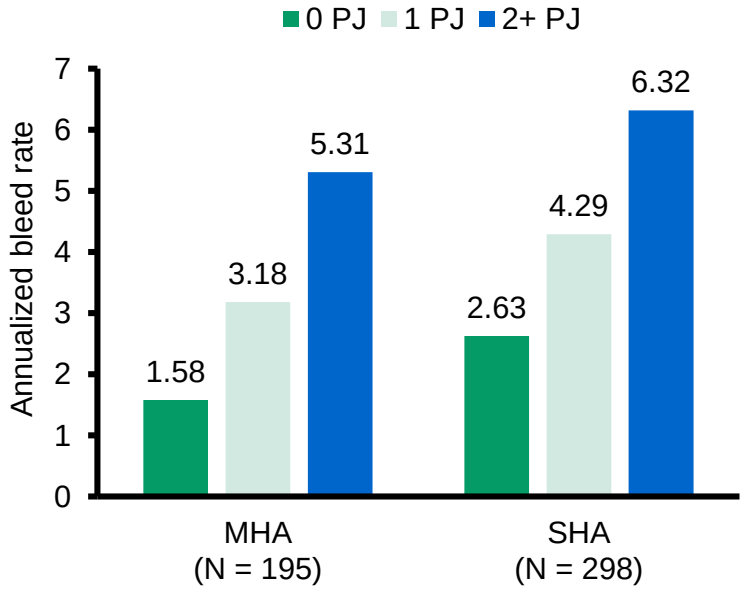
	CHESS II			CHESS Paediatrics		
	MHA	SHA	All	MHA	SHA	All
	N = 195	N = 298	N = 493	N = 321	N = 464	N = 785
Age, mean (SD)	39.70 (14.76)	37.89 (13.56)	38.61 (14.06)	10.69 (4.59)	10.08 (4.64)	10.33 (4.63)
BMI, mean (SD)	24.57 (2.90)	24.53 (2.94)	24.55 (2.92)	20.92 (6.38)	23.59 (21.51)	22.50 (17.07)
Inhibitor status, N (%)						
Yes	5 (2.6)	20 (6.7)	25 (5.1)	34 (10.6)	48 (10.3)	82 (10.4)
No	190 (97.4)	278 (93.3)	468 (94.9)	287 (89.4)	416 (89.7)	703 (89.6)
Has a target joint, N (%)						
No	149 (76.4)	163 (54.7)	312 (63.3)	280 (87.2)	363 (78.2)	643 (81.9)
Yes	46 (23.6)	135 (45.3)	181 (36.7)	41 (12.8)	101 (21.8)	142 (18.1)
No. of problem joints, N (%)*						
0 PJ	**118 (60.5)**	**152 (51.0)**	270 (54.8)	**277 (86.3)**	**382 (82.3)**	659 (83.9)
1 PJ	**45 (23.1)**	**83 (27.9)**	128 (26.0)	**29 (9.0)**	**66 (14.2)**	95 (12.1)
2+ PJ	**32 (16.4)**	**63 (21.1)**	95 (19.3)	**15 (4.7)**	**16 (3.4)**	31 (3.9)

*Among adults with 2+ PJ In CHESS II, the mean (SD) no. of problem joints in MHA and SHA was 2.44 (0.84) and 2.46 (0.80), respectively; among children with 2+ PJ in CHESS Paeds, the mean (SD) no. of problem joints in MHA was 2.27 (0.46) and in SHA was 2.25 (0.58)...
BMI, body mass index; MHA, moderate hemophilia A; PJ, problem joint; SD, standard deviation; SHA, severe hemophilia A.

Association between the number of PJs and ABR

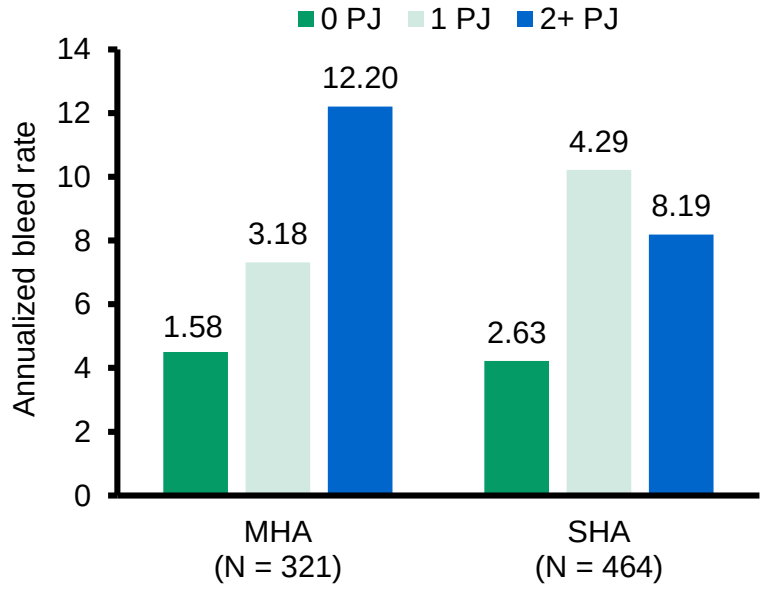
CHESS II

ABR consistently increased with **PJ**, in both **MHA** and **SHA**



CHESS Paeds

ABR and **PJ** showed a clearer trend in **MHA**, relative to **SHA**

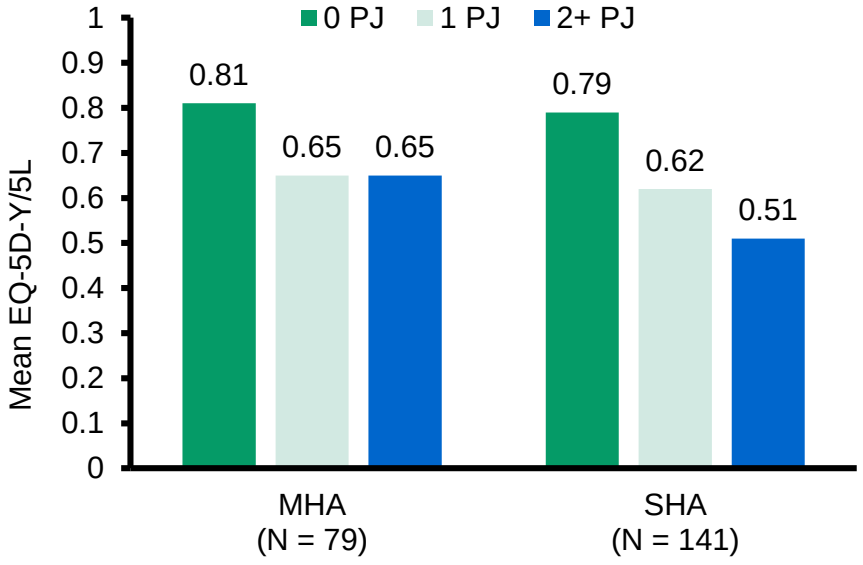


ABR, annualized bleed rate; PJ, problem joint; MHA, moderate hemophilia A; SHA, severe hemophilia A.

Impaired quality of life (EQ-5D-Y/5L) with PJs

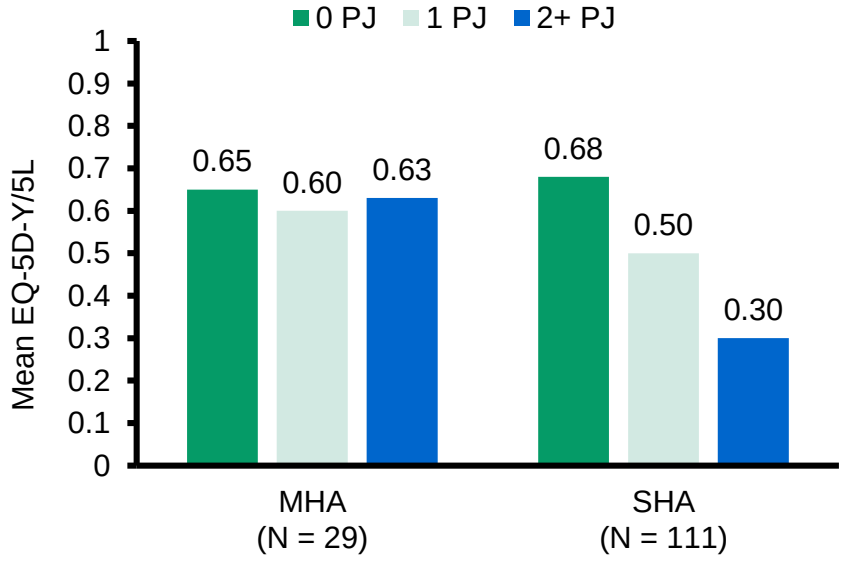
CHESS II

Quality of life was impaired **(**i.e., lower EQ-5D-5L) with **PJs**; in particular, among people with **SHA**, compared to **MHA**



CHESS Paeds

Impaired **quality of life** was associated with number of **PJs** in children with **SHA,** while no trend was observed in **MHA**



PJ, problem joint; MHA, moderate hemophilia A; SHA, severe hemophilia A.

Conclusions

- This analysis of data from CHESS Paeds and CHESS II indicates an association between chronic joint damage, as measured by the '**problem joint**' definition, and worsening clinical and quality of life outcomes, across both **MHA** and **SHA**
- Both children and adults with **MHA** exhibited a **clinical burden** indicated by **ABR**, and a **humanistic burden** in their **EQ-5D-Y/5L** scores, that was increased by **having one or more problem joints**
- The CHESS II and CHESS Paediatrics studies address the **lack of available data on the clinical, economic, and humanistic burden of hemophilia, across disease severity**
- Nonetheless, there are limitations inherent within a cross-sectional design and the retrospective nature of data collection, and further analyses should explore wider elements of **burden** and **unmet need**

ABR, annualized bleed rate; MHA, moderate hemophilia A; SHA, severe hemophilia A.

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