

Effect of Moderate and Severe Hemophilia A on Daily Life in Children and Their Caregivers: A CHES Paediatrics Study Analysis

Kate Khair,^{1,2} Francis Nissen,³ Mariabeth Silkey,³ Tom Burke,² Aijing Shang,³ Martynas Aizenas,³ Oliver Meier,³ Jamie O'Hara,^{2,4} and Declan Noone²

¹Haemnet, London, UK; ²HCD Economics, Daresbury, UK;

³F. Hoffmann-La Roche Ltd, Basel, Switzerland; ⁴University of Chester, Chester, UK

The CHES Paeds study collects real-world data on the burden-of-illness in children with HA

Background

- HA is a congenital bleeding disorder caused by a deficiency in clotting FVIII and characterized by uncontrolled bleeding and progressive joint damage¹
- CHES Paeds is a retrospective study assessing the **burden-of-illness of moderate and severe hemophilia** in children with HA with/without FVIII inhibitors²
 - The study was conducted in 5 European countries: France, Germany, Italy, Spain and the United Kingdom²

Aim of this analysis

- To assess the impact of disease burden on the daily life of children with HA and their caregivers



Retrospective data were captured via a survey of physicians, children, and their caregivers

1. Recruitment of children with HA

- Male children with moderate and severe HA were recruited across 5 European countries
- Recruitment was stratified 1:1:1 according to age (0–5 years:6–11 years:12–17 years); and approximately 2:1 according to disease severity (severe:moderate)
- Data were captured for the preceding 12-month period



2. Physician perspective

- Physicians completed online case report forms for treated children



3. Child and caregiver perspective

- The child and/or their caregiver completed a paper-based questionnaire utilizing 5-point Likert scales
 - For children with HA aged 0–7 years, the questionnaire was completed by the caregiver reporting proxy data, while for children with HA aged 8–17 years, children and caregivers completed different sections

Burden-of-illness data were collected on 196 children with moderate and severe HA

- All children included in this study are male

	Moderate HA (n=50)	Severe HA (n=146)	All participants (N=196)
Mean age, years (SD)	9.4 (5.0)	10.5 (4.2)	10.2 (4.4)
Age category, n (%)			
Age 0–5	13 (26.0)	22 (15.1)	35 (17.9)
Age 6–11	18 (36.0)	63 (43.2)	81 (41.3)
Age 12–17	19 (38.0)	61 (41.8)	80 (40.8)
Median BMI, kg/m² (IQR)	19.4 (17.0–22.8)	20.4 (17.1–22.9)	20.0 (17.0–22.9)
With FVIII inhibitors, n (%)			
No	48 (96.0)	128 (87.7)	176 (89.8)
Yes	2 (4.0)	18 (12.3)	20 (10.2)
Current treatment strategy, n (%)			
On demand	7 (14.0)	35 (24.0)	42 (21.4)
Prophylaxis	32 (64.0)	110 (75.3)	142 (72.4)
Response missing	11 (22.0)	1 (0.7)	12 (6.1)
Has adapted hemophilia treatment, n (%)			
No	29 (58.0)	50 (34.2)	79 (40.3)
Yes	18 (36.0)	89 (61.0)	107 (54.6)
Not known	2 (4.0)	5 (3.4)	7 (3.6)
Response missing	1 (2.0)	2 (1.4)	3 (1.5)

Percentages may not add up to exactly 100% due to rounding.

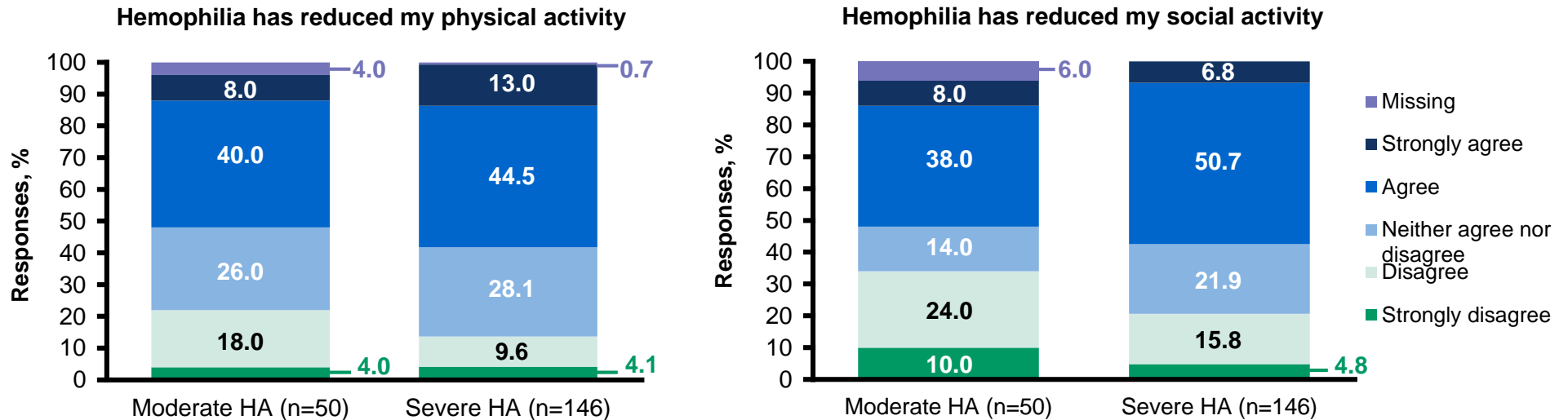
BMI, body mass index; FVIII, factor VIII; HA, hemophilia A; IQR, interquartile range; SD, standard deviation.

Some families cared for more than one child with HA

- For female carriers of HA, there is a 50% chance that any of their male children will inherit the condition¹
- In the CHES Paeds study, 24.5% of families had more than one child with HA
 - A total of 9.7% of children with HA had at least two siblings with HA

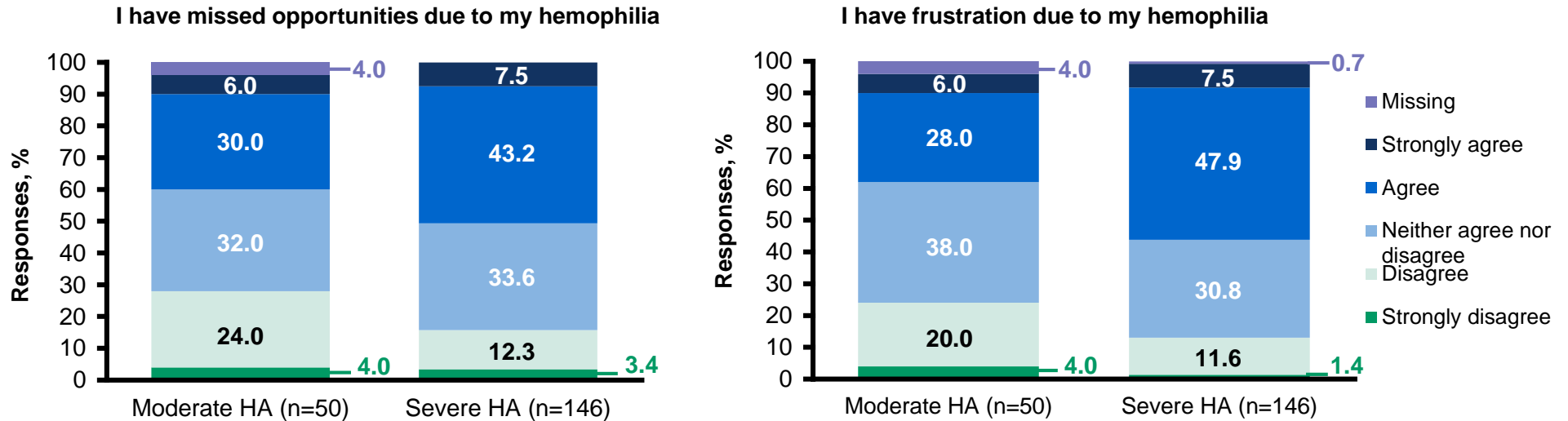
	Moderate HA (n=50)	Severe HA (n=146)	All participants (N=196)
>1 child in the family has hemophilia, n (%)			
No	41 (82.0)	103 (70.5)	144 (73.5)
Yes	8 (16.0)	40 (27.4)	48 (24.5)
Response missing	1 (2.0)	3 (2.1)	4 (2.0)
Number of siblings with hemophilia, n (%)			
0	41 (82.0)	103 (70.5)	144 (73.5)
1	7 (14.0)	22 (15.1)	29 (14.8)
2	1 (2.0)	14 (9.6)	15 (7.7)
3–5	0 (0.0)	4 (2.8)	4 (2.0)
Response missing	1 (2.0)	3 (2.1)	4 (2.0)

Children with HA have reduced physical and social activities as a result of their disease



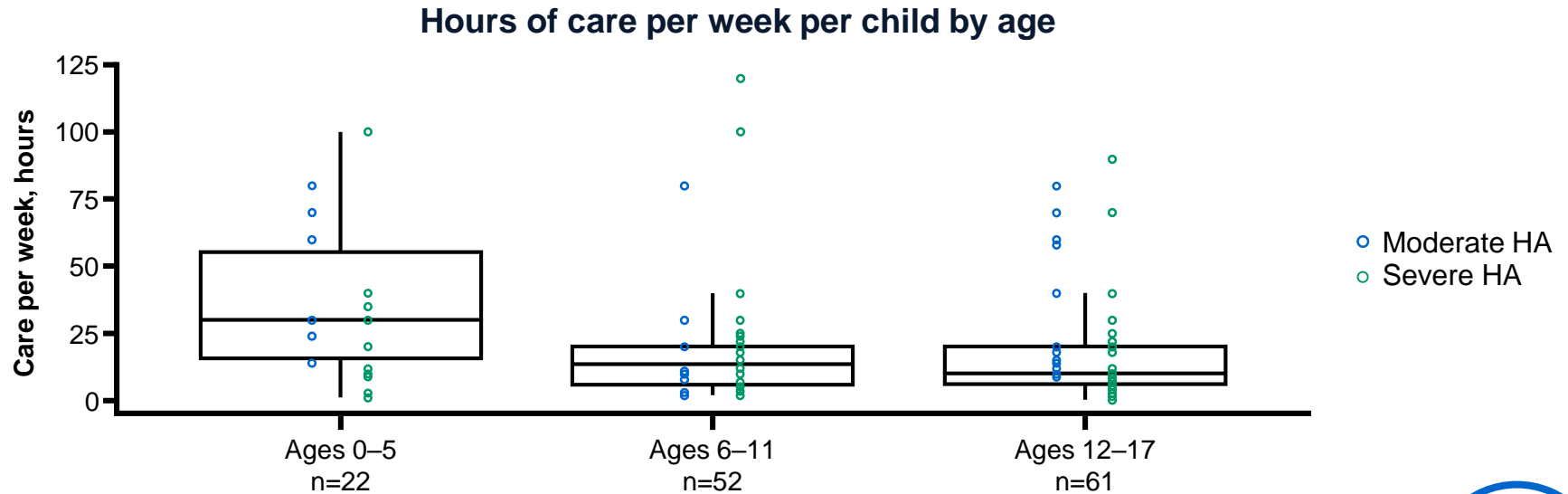
- According to the child or caregiver, due to HA:
 - **48.0%** of children with **moderate HA** and **57.5%** with **severe HA** have **reduced physical activity**
 - **46.0%** of children with **moderate HA** and **57.5%** with **severe HA** have **reduced social activity**

Children with HA felt that HA had caused them to miss opportunities, and felt frustration due to their HA



- According to the child or caregiver, due to HA:
 - **36.0%** of children with **moderate HA** and **50.7%** with **severe HA** felt they had **missed opportunities**
 - **34.0%** of children with **moderate HA** and **55.4%** with **severe HA** felt **frustration**

Caregivers were also impacted by the disease burden of HA



- Median (IQR) hours of care provided per week decreased from 30.0 (15.5, 55.0) for children aged 0–5 to 10.0 (6.0, 20.0) for children aged 12–17 years



Caregivers' work was impacted by their caregiving duty

Impact on caregivers' work due to their caregiving duty



- Of those who responded, due to HA:
 - **17.4%** (n=4/23) of caregivers to children with **moderate HA** and **25.0%** (n=20/80) with **severe HA** have **lost work due to their caregiving duty**
 - This was more than twice as common for caregivers in families with **multiple children with HA (42.9%, n=9/21 responses)** compared with families with **one child with HA (18.5%, n=15/81 responses)**



- Of those who responded, due to HA:
 - Caregivers of children with **moderate and severe HA** (n=24) have lost a median (IQR) of **15.0 (7.25–20.0) hours per week** of work

Conclusions (1)



The CHES Paeds study addresses the **paucity of data** on the impact of **disease burden** on the daily lives of **children with HA and their caregivers**



These data indicate that **both children and caregivers make sacrifices in their daily lives due to HA**

- Many children with HA reported **reduced physical and social activities, fewer opportunities and feelings of frustration** due to their HA
- Caregivers reported spending a substantial number of hours **caring for the hemophilia-related needs** of their child, and losing work due to their caring responsibilities

Conclusions (2)



This study is limited by its **cross-sectional design** and **limited number of respondents** to some questions, particularly those on caregiver burden

- Responses on the hours of work lost may be subject to selection bias, as caregivers who have lost work may be more likely to respond to this question
- Some caregivers may have left employment due to their caregiving responsibility, and only caregivers with the time or need to attempt paid employment were able to respond to the question



Overall, **the burden of disease was similar** in children with moderate and severe HA

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