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Shortening the paediatric Haemophilia Activities List (pedHAL) based on pooled data from international studies

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Abstract

Introduction: The paediatric Haemophilia Activities List (pedHAL) was developed to measure activities and participation in children and youth with haemophilia. Results from international studies provide an opportunity to determine which items are universally important.

Aim: The aim of this study was to determine which items of the pedHAL are redundant to construct a shorter version of the pedHAL.

Methods: This study is a cross-sectional multicentre secondary analysis on pooled data of published studies using the pedHAL (7 domains, 53 items, optimum score: 100) in children with haemophilia A/B aged 4–18 years. To identify redundant items, the following aspects were evaluated: floor and ceiling effects, proportions of missing and 'not applicable' responses, inter-item correlations, component loadings in an exploratory factor analysis, internal consistency and item-total correlations.

Results: Data on 315 patients with haemophilia from 6 studies were evaluated. Median age was 12.2 years) (range 4.0–18.0), 87.3% had severe haemophilia and 80.3% received prophylaxis. Median (IQR) pedHAL sum score was 96.7 (88.0–100). After a stepwise procedure, 31 items were removed, resulting in a pedHAL_short of 22 items, representing all original 7 domains. Most remaining items belonged to the domains 'sitting/kneeling/standing' and 'functions of the legs'. The pedHAL_short sum score was similar to the original pedHAL sum score, with small differences in 5 domains.

Conclusion: This clinimetric study resulted in >50% reduction of the length of the pedHAL. The 22-item pedHAL_short reduces patient burden and is expected to capture the information on activities and participation. The pedHAL_short needs validation in other populations.

KEYWORDS
activities, haemophilia, participation, patient-reported outcome

1 | INTRODUCTION

The paediatric Haemophilia Activities List (pedHAL) assesses self-reported limitations in various activities of daily living, which are relevant to children and youth with haemophilia.[1] It was directly derived from the Haemophilia Activities List (HAL) for adults [2,3] and subsequently validated.[1,4] The pedHAL includes 53 items, distributed over seven domains similar to the HAL domains: 'sitting/kneeling/standing', 'functions of the legs', 'functions of the arms', 'use of transportation', 'self-care', 'household tasks' and 'leisure activities and sports'. All items belong to 'activities and participation', according the World Health Organization's International Classification of Functioning (ICF). 'Activity' is defined as 'the execution of a task or action by an individual' and 'participation' as 'involvement in a life situation'.[5] The pedHAL is recommended for both research purposes and clinical management of patients.[6]

After introduction of the pedHAL to clinical care and research in 2010, clinicians and researchers reported some items to be non-informative. Most ceiling effects were observed in the domains of 'functions of the arms', 'use of transportation', 'self-care' and 'household activities' in Dutch, English, Romanian and Lithuanian children. The domains 'sitting/kneeling/standing', 'functions of the legs' and 'leisure activities and sports' were the most informative domains.[4,7-9]

Shortening the questionnaire may enhance the feasibility of pedHAL use within the context of multiple outcome assessments in haemophilia care. With more studies using the pedHAL being conducted internationally over the past years, there is an opportunity to determine which items are universally important for different patient populations.

The aim of this study was to determine which items of the pedHAL are redundant in order to construct a shorter version of the pedHAL for the measurement of activities and participation in children and youth with haemophilia.

2 | MATERIALS AND METHODS

2.1 | Study design and study population

This study was a cross-sectional multicentre secondary analysis of pooled data of published studies using the pedHAL. The Medical Research Ethical Committee (MREC) of the University Medical Center Utrecht reviewed the study (protocol number 18-309/C).

2.2 | Pooling of published pedHAL data

A literature search identified five studies published between 2010 and April 2018, which used the pedHAL in children with haemophilia. In addition, two studies in preparation for publication were identified and included. Inclusion criteria were children with haemophilia A (FVIII) and B (FIX) of all severities, aged 4–18 years. Patients were excluded if more than half of the pedHAL items were missing. If both the children and parent proxy pedHAL scores were available, only the pedHAL completed by the child was included in the analyses. Authors of all seven papers were invited to share the original pedHAL data (scores per item) and de-identified patient characteristics. All but one authors accepted our invitation. Only data of children with haemophilia A (FVIII) and B (FIX) aged 4–18 years who participated in the selected studies were included.[1,4,7,9-11]

2.3 | Measurements

The pedHAL assesses self-reported limitations in activities and participation in children with haemophilia. It consists of a patient version (8–18 years) and parent version (4–18 years) both with 53 items, distributed over seven domains. Patients score the items on a 6-point Likert scale ('impossible', 'always', 'usually', 'sometimes', 'almost never', 'never'), with a 'not applicable (N/A)' scoring option. Domain scores and sum scores are converted to a normalized domain score ranging from 0 (worst possible functional abilities) to 100 (best possible functional abilities) in the scoring tool (available at www.vancreveldkliniek.nl). According to the pedHAL scoring manual, domain scores were only calculated if half or more of the items of a domain were scored on the 6-point Likert scale.

Patient characteristics analysed included age at pedHAL assessment, type of haemophilia (A or B), severity of the disease (mild [factor VIII/IX activity 0.06 – 0.40 IU/ml], moderate [factor VIII/IX activity 0.01 – 0.05 IU/ml] or severe [factor VIII/IX activity <0.01 IU/ml]), clotting factor regimens (prophylaxis yes/no and start prophylaxis before age of 3 years yes/no) and current inhibitor status.

2.4 | Statistical analyses

Patient characteristics were presented as proportions or medians (interquartile ranges [IQR:P25 - P75]). A Kruskal-Wallis test was performed to compare age according to treatment regimen (prophylaxis start <3 years vs. prophylaxis start ≥3 years vs. no prophylaxis). Descriptive analyses (median, IQR, range, mean and standard deviation [SD]) were performed for the pedHAL domain and sum scores. Based on reported limits of agreement (LoA) of test-retest data,[1] limitations in activities and participation were defined as ≤95 points for domain and sum scores.

Non-informative items were identified in a stepwise process (7 steps) according to the method of de Vet et al. (2011), from the COnsensus-based Standards for the selection of health Measurement INstruments (COSMIN) initiative.[12] After each step, non-informative items were deleted, before proceeding with the following step.

Step 1: Per item the proportions of each scoring option ('impossible', 'always', 'usually', 'sometimes', 'almost never' or 'never') was determined, excluding the missing and 'N/A' scored questions.

Proportions of minimum ('impossible') and maximum ('never' problems) scores were analysed to detect floor and ceiling effects. Items with ≥85% minimum or maximum scores were removed.

Step 2: The missing data and scores with 'N/A' were examined. The authors removed items which were scored >15% as 'missing' or 'N/A'.

Step 3: Inter-item correlations were evaluated. Inter-item correlations calculated with Spearman's rho <0.2 indicated items which do not correlate with any of the others and >0.9 indicated item redundancy. Items with inter-item correlations <0.2 and >0.9 were not included in the factor analysis.

Step 4: Component loadings on exploratory factor analysis were evaluated. Items were analysed on categorical level. Items with factor loadings <0.5 were removed. Model fit was evaluated with the Root Mean Square Error of Approximation (RMSEA); <0.08 indicates moderate model fit and <0.05 indicates good model fit.

Step 5: Inter-item correlations were evaluated for the second time. Inter-item correlations calculated with Spearman's rho >0.7 within one factor were indicators for item redundancy. Items which had a correlation >0.7 were reviewed by IK, KF and JJ and one of the items was removed.

Step 6: Internal consistency calculated with Cronbach's α and internal consistency after item deletion were compared. Cronbach's α should be 0.7 and 0.9; a higher Cronbach's α after item deletion was considered a reason to eliminate an item.

Step 7: Item-total correlations for the pedHAL total score were evaluated. Item-total correlations were calculated with Spearman's rho. Items with item-total correlations <0.3 were removed.

A sensitivity analysis was performed where only parent scores were analysed in cases where both were available (n = 72).

After removing non-informative items, a pedHAL$_{short}$ was created. Median (IQR) normalized sum and domain scores and percentages of scores ≤95 points were calculated for the pedHAL$_{short}$, similar to the calculation of sum and domain scores in the original scoring tool. The differences between the pedHAL and pedHAL$_{short}$ sum scores were calculated and shown in a box plot. In addition, a one-way analysis of variance (ANOVA) was performed to compare differences according to treatment regimen (prophylaxis start <3 years vs. prophylaxis start ≥3 years vs. no prophylaxis). A secondary exploratory factor analysis was performed for the pedHAL$_{short}$ to detect possible underlying constructs.

SPSS (version 25, IBM) was used for data analyses. Mplus (version 6.12, Muthen & Muthen) was used for the exploratory factor analysis.

3 | RESULTS

3.1 | Patient characteristics

From the data of six studies, 315 children with haemophilia A or B were included. The data are from the Netherlands (n = 84),[1,9] Romania (n = 28),[4] United Kingdom (UK) (n = 123),[7] Lithuania (n = 15)[10] and Canada (n = 65).[11] Patient characteristics are shown in Table 1. Median age at the time of completing the last pedHAL was

TABLE 1 Patient characteristics (n = 315)

Patient characteristics (n = 315)	Median (IQR), % (n)
Age (years)	12.2 (9.7–15.0)
Haemophilia A	87.0 (n = 274)
Severity haemophilia	
Mild	7.6 (n = 24)
Moderate	5.1 (n = 16)
Severe	87.3 (n = 275)
Prophylaxis	80.3 (n = 253)
Early prophylaxis (<3 years)	51.9 (n = 139)[a]
Inhibitor (current)	5.7 (n = 18)

[a]Missing data on prophylaxis (n = 47).

12.2 years (range 4.0 – 18.0) and was similar for patients with different treatment regimens (P = 0.22). The majority of the patients had severe haemophilia (87.3%). One patient was excluded because he completed less than half of the items of the questionnaire. The bulk of the questionnaires (81.3%) that were analysed were completed by the children, and the others were completed by parents.

3.2 | PedHAL domain and sum scores

Domain and sum scores are shown in Table 2. The median (IQR) pedHAL sum score was 96.7 (88.0 – 100.0). 'Positive' pedHAL sum scores (≤95 points) were observed in 43% of participants. The median (IQR) domain scores were lowest for the domains 'sitting/kneeling/standing' (97.8 [82.2 – 100.0]), 'functions of the legs' (97.8 [83.6 – 100.0]) and 'leisure activities and sports' (97.8 [80.0 – 100.0]). The other domains had median scores of 100.0. Domain scores were not calculated for 2 participants for the domains 'functions of the arms' and 'self-care', and for up to 60 participants for the domain 'leisure activities and sports', because more than half of the items were scored as missing or 'N/A'.

3.3 | Item reduction

The stepwise process to select non-informative items is shown in Table 3. The frequency tables generated for step 1 and 2 and the table with item-total correlations for step 7 in the item reduction process are shown in the Data S1.

1. Floor and ceiling effects.

Minimum and maximum scores were evaluated for all items. There was no floor effect in any pedHAL item. Ceiling effects were shown in 2/6 items of the domain 'functions of the arms' and in 6/9 items of the domain 'self-care'.

2. Missing data and scores with 'N/A'.

There were small numbers of missing responses (0–3) on the items. Missing and/or 'N/A' responses were scored in >15% of the children in 1/3 items of the domain 'use of transport', 2/3 items of the domain 'household tasks' and in 9/11 items of the domain 'leisure activities and sports'.

Domain	Domain scores				Score ≤95	Missing/ NA
	Median	(IQR)	Min	Max	(%)	N
Sitting/kneeling/ standing	97.8	(82.2–100)	10.0	100	45	0
Functions of the legs	97.8	(83.6–100)	0.0	100	44	3
Functions of the arms	100	(90.0–100)	10.0	100	34	2
Use of transport	100	(93.3–100)	0.0	100	26	17
Self-care	100	(96.8–100)	6.7	100	20	2
Household tasks	100	(93.3–100)	0.0	100	26	37
Leisure activities and sports	97.8	(80.0–100)	0.0	100	45	60
Sum score	96.7	(88.0–100)	19.2	100	43	0

3. Inter-item correlations (1).

None of the items had correlations with other items lower than 0.2 or higher than 0.9. All remaining items were used for the exploratory factor analysis.

4. Component loadings of the exploratory factor analysis.

Table 4 shows the component loadings of the exploratory factor analysis. The exploratory factor analysis suggested no items were eligible for item reduction. A 2-factor model was selected which included all remaining items, and the two factors were identified as arm activities and leg activities. The model fit of the 2-factor model was 0.07 (RMSEA), indicating moderate model fit. The factor loadings were >0.5.

5. Inter-item correlations (2).

Inter-item correlations were re-evaluated. In the domain 'sitting/kneeling/standing', 3/10 items, which had inter-item correlations >0.7 with other items, were removed. In the domain 'functions of the legs', 5/11 items were removed. The items 'running' and 'jumping' had a correlation of 0.73. The authors decided to remove the item 'jumping', which was considered less relevant in lifelong outcome assessment. The items 'walking upstairs' and 'walking downstairs' had a correlation of 0.81. The authors decided to remove the item 'walking downstairs', which was scored as less difficult than 'walking upstairs' by the participants. In the domain 'use of transport', the item 'using public transport' (1/3) was removed. In the domain 'self-care', 2/9 items were removed.

6. Internal consistency calculated with Cronbach's α.

The remaining 22 PedHAL items were strongly related (Cronbach's α of 0.97), which indicates redundancy of items. Only complete cases (n = 201, 63.8%) were included in the analysis. The Cronbach's α after deletion of separate items was equal or smaller, which did not identify candidate items for removal. Eventually, the authors decided to keep the remaining 22 items, because the Cronbach's alpha was already lowered by removing the 31 items.

7. Item-total correlations for pedHAL total scores.

All item-total correlations were high (Spearman's rho =0.55 – 0.76), thus identifying no candidates for item reduction.

The sensitivity analysis with parent proxy (n = 131) and child (n = 184) forms resulted in a shorter pedHAL$_{short}$ (20 items). The items 'walking or riding up a small hill or slope without help', 'stretching to reach something above your head' and 'putting on pants' were removed in step 5 (inter-item correlations), and 'putting on shoes and socks' was not removed in step 5.

3.4 | PedHAL$_{short}$ with 22 items

In Table 3, all items of the pedHAL$_{short}$ are shown. Domain and sum scores of the pedHAL and pedHAL$_{short}$ are shown in Table 5.

Twenty-two items remained after removing the items (n = 31) according to the seven steps. All domains were still represented in the pedHAL$_{short}$. Most items of the pedHAL$_{short}$ belonged to the domains 'sitting/kneeling/standing' (n = 7) and 'functions of the legs' (n = 6). For the domains 'use of transport', 'self-care' and 'household tasks', only one item remained in the pedHAL$_{short}$. The median (IQR) pedHAL$_{short}$ sum score was 97.3 (87.0 – 100.0), which was similar to the pedHAL sum score. The differences between the pedHAL and pedHAL$_{short}$ sum scores were similar in patients receiving prophylaxis started <3 years and started ≥3 years and patients receiving no prophylaxis (P = 0.82) (see Figure 1). The domains had median scores of 100.0, in exception of a median domain score of 97.1 for 'sitting/kneeling/standing'. Domain scores for 'sitting/kneeling/standing', 'functions of the legs', 'use of transport' and 'self-care' were higher than the original pedHAL domain scores. Domain score for 'functions of the arms' was lower than the original pedHAL domain score. The largest discrepancy in the proportions of abnormal domain scores (≤95) was observed for the domain 'use of transport' (pedHAL: 26% vs. pedHAL$_{short}$: 16%), which was a result of removing the item 'cycling'.

The secondary exploratory factor analysis with the 22-item pedHAL$_{short}$ resulted in a 1-factor model, indicating that a sum score containing all 22 items needs to be used.

TABLE 3 Flow chart of steps to reduce the number of pedHAL items and the remaining 22 pedHAL items

Sitting/kneeling/standing	Functions of the legs	Functions of the arms	Use of transport	Self-care	Household tasks	Leisure activities and sports
Step 1: Floor and ceiling effects (≥85% maximum scores)						
		3 Fine hand movements 6 Shaking hands with someone		1 Drying off your entire body 5 Wiping your bottom after using the toilet 6 Fastening a hood or doing up the top button on your … 7 Buttering bread or making a sandwich 8 Unscrewing the lid from a bottle of water, juice, etc. 9 Brushing your teeth		
Step 2: Missing and/or N/A (>15% missing / N/A)						
			1 Cycling		2 Outside chores 3 Other household chores	1 Going out 4 School sports: athletics 5 School sports: ball sports 6 Playing non-contact team sports 7 Playing contact team sports 8 Individual non-contact sports 9 Individual contact sports 10 Taking part in a sports event 11 Going to school camp or summer camp
Step 3: Inter-item correlations (r < 0.2 and r > 0.9)						
Step 4: Exploratory factor analysis (factor loadings <0.5)						
Step 5: Inter-item correlations (r > 0.7)						
5 Kneeling/ squatting 8 Standing still for a short period 10 Standing still for a very long periods	1 Walking short distances 3 Walking long distances 4 Walking on an uneven surface 8 Jumping 10 Walking downstairs		3 Using public transport (bus, train, metro, tram)	2 Putting on a t-shirt or jumper 4 Putting on shoes and socks		
Step 6: Internal consistency						
Step 7: Item–total correlations (r < 0.3)						

(Continues)

TABLE 3 (Continued)

pedHAL$_{short}$: 22 items

Sitting/kneeling/standing	Functions of the legs	Functions of the arms	Use of transport	Self-care	Household tasks	Leisure activities and sports
1 Sitting down	2 Walking longer distances	1 Carrying large or heavy objects with two hands	2 Getting in and out the car	3 Putting on pants	1 Chores around the house	2 Playing outside, alone or with others
2 Sitting on the ground	5 Walking on a soft surface	2 Stretching to reach something above your head				3 School sports: exercises and gymnastic equipment
3 Standing up from a chair with arm rests	6 Strolling	4 Writing				
4 Standing up from a chair without arm rests	7 Running	5 Leaning on your arms				
6 Squatting for longer periods	9 Walking upstairs					
7 Bending over forwards	11 Walking or riding up a small hill or slope without help					
9 Standing still for longer periods						

4 | DISCUSSION

This study analysed international pedHAL data in children with haemophilia with the aim of reducing the 53-item pedHAL questionnaire. A stepwise approach resulted in a pedHAL$_{short}$ of 22 items. The items of the pedHAL$_{short}$ belonged to the domains of the original pedHAL: 'sitting/kneeling/standing' (n = 7), 'functions of the legs' (n = 6), 'functions of the arms' (n = 4), 'use of transportation' (n = 1), 'self-care' (n = 1), 'household tasks' (n = 1) and 'leisure activities and sports' (n = 2). Differences between the original pedHAL and pedHAL$_{short}$ sum score were similar between treatment regimens.

4.1 | Internal and external validity

This is the first study presenting pedHAL data of 315 patients from heterogeneous populations. In these published pedHAL data, the majority of patients had received prophylactic treatment (80%) and half of the patients had early prophylaxis (51%). Ceiling effects are more likely to occur in intensively treated patients.

Despite the lack of cross-cultural validation studies of the pedHAL, the use of the pedHAL is recommended in international guidelines.[13] After development of the pedHAL in Dutch children, only one clinimetric study was performed in Romanian children.[1,4] In Romanian children, high proportions of 'N/A' responses were recorded in the domains 'household tasks' and 'leisure activities and sports'. This was confirmed by the present 'pooling' study including more children from different populations. It seems that especially these domains are culturally dependent and removing these items is expected to results in a questionnaire that will perform better in a multicultural and global context. The shift towards higher domain scores in some domains (ie 'sitting/kneeling/standing', 'functions of the legs', 'use of transport' and 'self-care') and lower domain scores for 'functions of the arms' were a result of the different reasons for removing items. For example, in the domain 'functions of the arms', items were only removed for ceiling effects and in the domain 'use of transport' the most difficult item 'cycling' was removed for a high number of 'N/A' responses. However, the sum scores of the pedHAL and pedHAL$_{short}$ were similar.

For two items with a high item-total correlation, rephrasing of the question may be considered. The items 'walking upstairs' and 'walking downstairs' had a high inter-item correlation of 0.81. 'Walking upstairs' was reported by the participants as being slightly more difficult. As both items are about walking stairs, 'walking stairs' may better capture the activity than choosing one of the two activities. For calculating the pedHAL$_{short}$ from the original pedHAL, any limitation reported on walking stairs could be scored as abnormal.

Internal consistency of the pedHAL$_{short}$ (Cronbach's α = 0.97) is still higher than the recommended Cronbach's α between 0.7 and 0.9. As the internal consistency improved after reduction of the 31 items and there was no clear indication for removing any other specific items, it was decided to retain the remaining items.

TABLE 4 Two-factor model of the remaining pedHAL items

	Arm activities	Leg activities
Sitting/kneeling/standing		
Sitting down	**0.725**	0.507
Sitting on the ground	0.614	**0.658**
Standing up from a chair with arm rests	**0.720**	0.556
Standing up from a chair without arm rests	**0.654**	0.562
Kneeling/squatting	0.547	**0.709**
Squatting for long periods	0.521	**0.726**
Bending over forwards	0.612	**0.630**
Standing still for a short period	0.523	**0.726**
Standing still for longer periods	0.338	**0.905**
Standing still for very long periods	0.256	**0.925**
Functions of the legs		
Walking short distances	0.638	**0.657**
Walking longer distances	0.430	**0.851**
Walking long distances	0.347	**0.855**
Walking on an uneven surface	0.531	**0.727**
Walking on a soft surface	0.573	**0.710**
Strolling	0.501	**0.725**
Running	0.480	**0.782**
Jumping	0.514	**0.740**
Walking upstairs	**0.730**	0.606
Walking downstairs	**0.766**	0.585
Walking or riding up a small hill or slope without help	**0.674**	0.634
Functions of the arms		
Carrying large or heavy objects with two hands	**0.824**	0.358
Stretching to reach something above your head	**0.790**	0.411
Writing	**0.650**	0.397
Leaning on your arms	**0.764**	0.389
Use of transport		
Getting in and out of the car	**0.845**	0.445
Using public transport	**0.763**	0.549
Self-care		
Putting on a t-shirt or jumper, etc.	**0.903**	0.312
Putting on pants	**0.905**	0.374
Putting on shoes and socks	**0.866**	0.385
Household tasks		
Chores around the house	**0.772**	0.497
Leisure activities and sports		
Playing outside, alone or with others	0.614	**0.621**
School sports: exercises and gymnastic equipment	0.610	**0.623**

Note: Interpretation: Two factors were identified in the factor analysis: arm activities and leg activities. The grey highlighted factor loadings shows to which factor the items were allocated. The highest factor loading of each factor was shown in bold.

To ensure that the pedHAL$_{short}$ contains all informative items, the 22-item pedHAL$_{short}$ was preferred above the 20-item pedHAL$_{short}$ resulting from the sensitivity analysis including all parent forms.

4.2 | Comparison with other studies

Similar domains were important in two studies not included in this pooled data. In boys with haemophilia from Lithuania and Portugal,

TABLE 5 Domain and sum scores of the pedHAL and pedHAL$_{short}$.

	pedHAL			pedHAL$_{short}$		
Domain	Median	IQR	Score ≤95 (%)	Median	IQR	Score ≤95 (%)
Sitting/kneeling/standing	97.8	82.2–100	45	97.1	85.7–100[*]	42
Functions of the legs	97.8	83.6–100	44	100	86.7–100[*]	39
Functions of the arms	100	90.0–100	34	100	85.0–100[*]	40
Use of transport	100	93.3–100	26	100	100–100[*]	16
Self-care	100	96.4–100	20	100	100–100[*]	18
Household tasks	100	93.3–100	26	100	100–100	19
Leisure activities and sports	97.8	80.0–100	45	100	80.0–100	44
Sum score	96.7	88.0–100	43	97.3	87.0–100	43

Note: Only complete cases within each domain were included in the comparison of the pedHAL scores with pedHAL$_{short}$ scores.
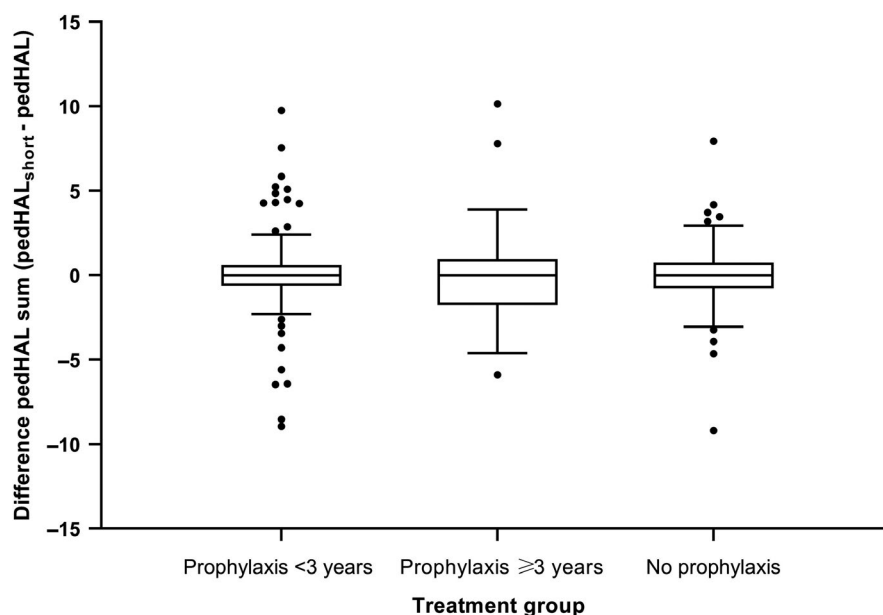
*$P < 0.05$ Wilcoxon's signed rank test.

FIGURE 1 Box plots of the differences in pedHAL and pedHAL$_{short}$ scores for patients with three different treatment regimens.

the most difficulties were reported in the domains 'sitting/kneeling/standing', 'functions of the legs' and 'leisure activities and sports'.[8,14] The fewest difficulties were reported in the domain 'self-care' in the Lithuanian data and in the domains 'household tasks' and 'self-care' in the Portuguese data, which were both less informative domains in the pooled data.[8,14] Exact scores were difficult to compare because both studies reported mean scores. The highest proportions of 'N/A' responses were in the domain 'leisure activities and sports' in the Lithuanian data, which was similar in the pooled data.[8] No other studies were available to further compare our findings. In adults, similar scoring patterns were shown by domain level.[15,16]

participation is desirable. This pooling study of international pedHAL data in children with mild to severe haemophilia with a wide range of treatment regimens suggested that 31 pedHAL items are redundant, resulting in a notable shortening of the questionnaire. The shorter version of the pedHAL includes the most relevant and informative items for children and youth with haemophilia. The pedHAL$_{short}$ can be derived from the original pedHAL, which allows for use in longitudinal studies. Only the sum score should be used for the pedHAL$_{short}$, since some domains only have 1 item in the pedHAL$_{short}$. Before introduction of the pedHAL$_{short}$ construct, validity and reliability of the questionnaire should be investigated in diverse populations.

4.3 | Clinical implications and future research

Within a context of multiple outcomes assessments in haemophilia care, a shorter assessment of limitations in activities and

5 | CONCLUSION

This clinimetric study resulted in a reduction of the pedHAL by more than half after a stepwise procedure of removing items. This short

version of the pedHAL (22 items) is expected to retain the most relevant and informative items on activities and participation for children with haemophilia, representing all domains of the original pedHAL. It detects similar proportions of abnormal sum scores.

CONFLICT OF INTEREST
K. Fischer has received speaker's fees from Bayer, Baxter/Shire, SOBI/Biogen, CSL Behring, Octapharma, Pfizer, NovoNordisk; performed consultancy for Bayer, Baxter, Biogen, CSL-Behring, Freeline, NovoNordisk, Pfizer, Roche and SOBI; and has received research support from Bayer, Pfizer, Baxter/Shire, and Novo Nordisk, Biogen. K Fischer is the epidemiologist for the EUHASS and PedNet registries. K. Fischer is member of the group that developed the pedHAL. IAR Kuijlaars and J. van der Net do not have any conflict of interest regarding this manuscript other than membership of the group that developed the pedHAL. The other authors have no competing interests.

AUTHOR CONTRIBUTION
IAR Kuijlaars, J van der Net and K Fischer contributed to the design of the study. IAR Kuijlaars performed the statistical analyses. IAR Kuijlaars wrote the first draft of the paper. All authors contributed to interpretation of the data, modification of statistical analyses and the writing of the manuscript.

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SUPPORTING INFORMATION
Additional supporting information may be found online in the Supporting Information section.