

# Is the Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH) appropriate to measure the impact of hemophilia and its treatment? First quantitative survey

Antoine Regnault,<sup>1</sup> Karina Raimundo,<sup>2</sup> Anisha M. Patel,<sup>2</sup> Jessica T. Markowitz,<sup>1</sup> Patrick Marquis,<sup>1</sup> Amy D. Shapiro,<sup>3</sup> Barbara A. Konkle,<sup>4</sup> Peter Trask,<sup>2</sup> Anna Ciesluk,<sup>1</sup> Michelle Rice,<sup>5</sup> Wendy E. Owens,<sup>6</sup> Kimberly Haugstad,<sup>6</sup> Jennifer Whiteley,<sup>2</sup> Robert F. Sidonio<sup>7</sup>

<sup>1</sup>Modus Outcomes, LLP, Cambridge, MA and Lyon, France; <sup>2</sup>Genentech, Inc., South San Francisco, CA, USA; <sup>3</sup>Indiana Hemophilia and Thrombosis Center, Indianapolis, IN, US; <sup>4</sup>Washington Center for Bleeding Disorders at Bloodworks Northwest, Seattle, WA, USA; <sup>5</sup>National Hemophilia Foundation, New York, NY, US; <sup>6</sup>Divitus LLC, Olympia WA, USA; <sup>7</sup>Aflac Cancer and Blood Disorders Center, Emory University, Atlanta, GA, USA.

## BACKGROUND

- Hemophilia management has evolved, leading to major changes in the experiences of people with hemophilia and enabling them to undertake activities they would previously not have considered (e.g., sports activities).
- Existing hemophilia-specific patient-reported outcome instruments are not adequate to capture these changes in people's lives appropriately, necessitating a new measure relevant to people with hemophilia.
- A new self-reported instrument, the **Comprehensive Assessment Tool of Challenges in Hemophilia (CATCH)**, was developed using qualitative and quantitative methods, including interviews with people with hemophilia and caregivers of children with hemophilia.
  - CATCH has three versions: Pediatric (ages 8–17 years), Adult (≥18 years old) and Caregiver (for parents/caregivers of children with hemophilia).

## OBJECTIVES

- To examine how well CATCH measures the impact of hemophilia and its treatment in adults and children with hemophilia, and caregivers of children with hemophilia.

## METHODS

- Online survey of people with hemophilia A or B (pediatric: 8–17 years; adult: ≥18 years) and caregivers of children (8–17 years) with hemophilia.
- Age group-specific versions of CATCH were completed by participants twice (1-week apart).
- Concepts/domains (or areas impacted by hemophilia) that were measured by CATCH are presented in **Table 1**.
- Rasch measurement theory (RMT) analysis was done for each concept to assess how well a set of items performs to generate reliable and valid measurement for the impact of hemophilia.

**Table 1. Concepts captured and number of items in the CATCH versions**

Concept	Number of items		
	Adult version	Pediatric version	Caregiver version
Daily activity risk perception	23 + 1 overall	18 + 1 overall	NA
Social activity risk perception	9 + 1 overall	7 + 1 overall	NA
Recreational activity risk perception	15 core items + larger bank + 1 overall	16 core items + larger bank + 1 overall	NA
Daily activity impact	23 + 1 overall	18 + 1 overall	NA
Social activity impact	11 + 1 overall	7 + 1 overall	NA
Recreational activity impact	15 core items + 17 items + 1 overall	16 core items + larger bank + 1 overall	NA
Work impact	9 + 1 overall	10 + 1 overall	NA
Preoccupation	9 + 1 overall	2 + 1 overall	12 + 1 overall
Treatment burden	7 + 1 overall	6 + 1 overall	7 + 1 overall
Pain	5	2	NA

\*Overall represents the overall impact of the disease on a given concept and "larger bank" represents additional items. For recreational activities, participants were instructed to answer only those items (or questions) that were relevant to them (i.e., if the item was about soccer, but the participant does not play soccer, they would skip that item). Response scale: 3–4 categories for Pediatric CATCH; 5 categories for Adult and Caregiver CATCH; 11-point numeric rating scale for pain. NA, not applicable.

## RESULTS

- The study included 184 people with hemophilia and 129 caregivers. Most participants had hemophilia A (73%) and moderate/severe disease (95%); 23% had inhibitors (**Table 2**).
- In general, CATCH performed well in its ability to measure the impact of hemophilia on patients' and caregivers' lives (**Table 3**).

**Table 2. Clinical data of the study participants (N=313)**

	Adults: Ages ≥18 (n=78)	Pediatric: Ages 8–17 (n=106)	Caregivers (n=129)
Mean age (SD)	37.0 (14.0)	12.2 (3.1)	40.1 (8.1)
Male – N (%)	69 (88.5%)	103 (97.2%)	NA
Parent of patient – N (%)	NA	NA	124 (96.1%)
Type of hemophilia – N (%)			
Hemophilia A	55 (70.5%)	77 (72.6%)	96 (74.4%)*
Hemophilia B	23 (29.5%)	29 (27.4%)	33 (25.6%)*
Severity of hemophilia – N (%)			
Mild	7 (9.0%)	3 (2.8%)	NA
Moderate	21 (26.9%)	24 (22.6%)	NA
Severe	50 (64.1%)	79 (74.5%)	NA
Presence of inhibitors – N (%)	15 (19.2%)	27 (25.5%)	NA
Number of bleeds in the past 30 days			
Range (min–max)	0–8	0–15	NA
Mean (SD)	1.3 (1.6)	1.3 (2.0)	NA
Number of bleeds in the past year			
Range (min–max)	0–51**	0–51**	NA
Mean (SD)	7.9 (10.2)	6.7 (10.0)	NA
Self-reported "joint problems due to hemophilia" – N(%)	56 (71.8%)	43 (40.6%)	NA
Treatment at HTC – N (%)	55 (70.5%)	87 (82.1%)	NA

\*Type of hemophilia of the patient; \*\*51 was maximum available option. HTC, hemophilia treatment center; SD, standard deviation; NA, not applicable/available.

### Adult CATCH

- The 5 response categories originally used for risk perception (no/low/moderate/high/very high risk) and activity impact (never/rarely/sometimes/often/always) did not perform as expected. They were rescored into 4 categories by merging the two highest categories (high/very high risk and often/always, respectively).
- With the 4 response categories, the adult version of the CATCH showed adequate measurement properties overall.
  - Good ability to produce consistent results across all domains except in recreational activity risk perception and impact.
  - Recreational activity risk perception and impact were more challenging to assess due to large amount of missing data by design, as participants only responded to items for relevant activities, and showed lower ability to produce consistent results.
  - Core set of 15 activities was identified to measure recreational activity risk perception and impact in adults with hemophilia (**Figure 1**).
  - Items were relevant to the the participant experience across all domains.
  - Minor issues: for some items in all domains except recreational activity risk perception and impact, most participants reported low risk/infrequent impact; participants' responses to treatment burden did not form a clear logical progression from less to more burdensome.

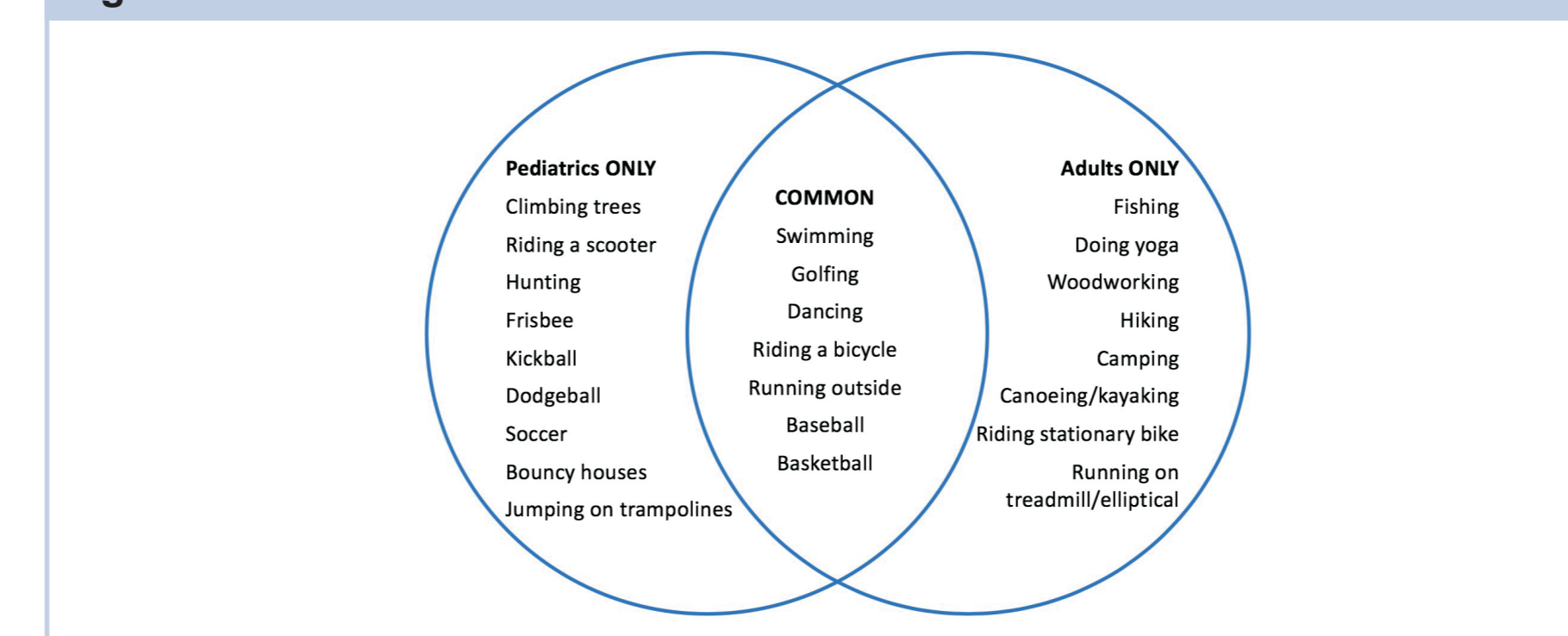
### Pediatric CATCH

- Overall, the pediatric version of CATCH showed adequate measurement properties, although often not as good as the adult version.
  - Adequate ability to produce consistent results across all domains except in preoccupation and recreational activity risk perception and impact.
  - Items were relevant to the the participant experience across most domains.
  - Many pediatric participants reported extremely low risks of bleed or infrequent impact for many of the daily and social activities.
  - Core set of 16 recreational activities was identified to measure recreational activity risk perception and impact in children with hemophilia (**Figure 1**).
  - Minor issues: most patients showed no impact, pain, or burden on some school impact, pain, and treatment burden items, respectively; participants' responses for school impact or treatment burden items did not form a clear logical progression.

### Caregiver CATCH

- Adequate ability to produce consistent results and items relevant to the caregiver experience.
- Few limitations: some items under preoccupation did not perform as expected (disordered thresholds and item fit); participants' responses to treatment burden did not form a clear logical progression from less to more burdensome.

**Figure 1. Core set of recreational activities**



**Table 3. Summary of validation study results**

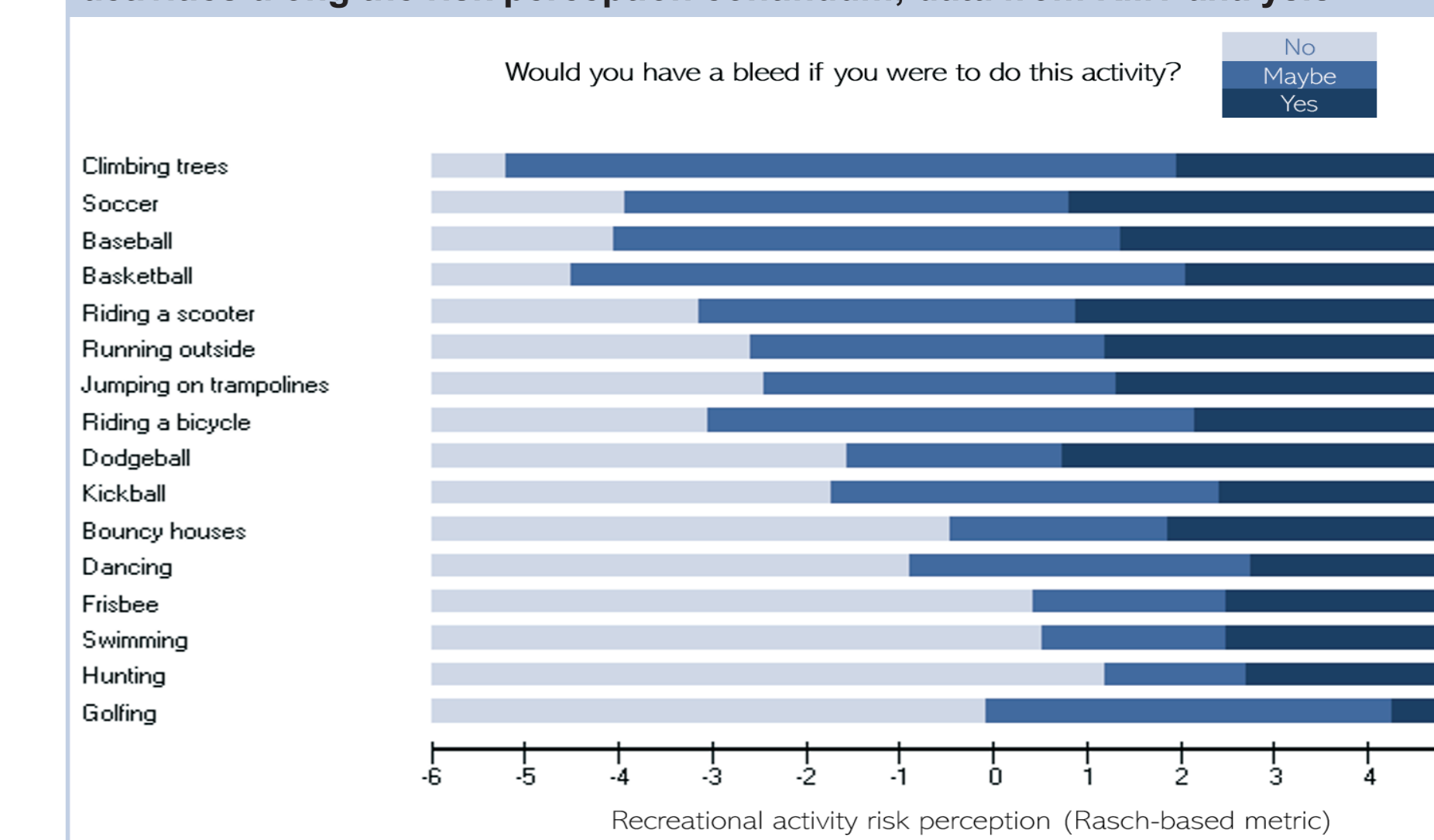
CATCH version	Daily/Social activity	Recreational activity	Work/School, Preoccupation, Pain, Treatment Burden
Adult	Good reliability (coefficients: 0.88–0.95); acceptable targeting; some floor effects	Challenging to assess; core set of 15 activities; low reliability (coefficients: 0.56–0.76)	Good reliability (coefficients: 0.88–0.92) and targeting; minor issues
Pediatric	Adequate reliability (coefficients: 0.71–0.93); floor effects	Core set of 16 activities; low reliability (coefficient: 0.51).	Adequate reliability (coefficients: 0.79–0.86) except in preoccupation (coefficient: 0.53); good targeting; minor issues
Caregiver	NA	NA	Adequate reliability (coefficients: 0.88–0.76); good targeting; few limitations

Coefficients indicate reliability coefficients. Reliability – ability to produce consistent results; Targeting – relevancy of items to the participants' experience; Floor effects – participants reported low risk and impact for activities. NA, not applicable.

### Interpreting CATCH data using the frames of reference

- The example in **Figure 2** demonstrates a logical progression in terms of risk perception for pediatric recreational activities.
- For most concepts in CATCH, RMT showed meaningful item structures.
  - In **Figure 2**, items range from safer (e.g., "swimming" and "frisbee") to less safe activities (e.g., "climbing trees" and "playing soccer") for risk perception.
- These meaningful item structures provide useful frames of reference when interpreting CATCH scores, and making inferences about participants' abilities based on their scores.

**Figure 2. CATCH item structure (hierarchy) for the pediatric core recreational activities along the risk perception continuum; data from RMT analysis**



## CONCLUSIONS

- These data provide good initial evidence that **CATCH is an appropriate and comprehensive measure of the impact of hemophilia and its treatment on people with hemophilia and their caregivers.**
- Built-in versatility, including different versions and independent measures for key concepts, make it a promising instrument for use in many contexts:
  - In **clinical research**, it could provide interpretable evidence on the benefit of new treatment for meaningful aspects to people with hemophilia.
  - In **clinical practice**, it could help characterize the impact of hemophilia on patients to inform treatment decisions.
- More data will be needed to consolidate the body of evidence on its performance and interpretation.

## ACKNOWLEDGEMENTS

This study was sponsored by Genentech, Inc. Third-party medical writing assistance was provided by Rebecca A Bachmann, PhD, of Gardiner-Caldwell Communications, and was funded by F. Hoffman-La Roche Ltd.