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POSTER ABSTRACTS

904.OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Understanding Treatment Burden in Pediatric Hemophilia: Development of the Child Hemophilia Treatment Experience Measure (Child Hemo-TEM)

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Background: Currently available hemophilia treatments have resulted in significant improvements in quality of life. However, children with hemophilia (CwH) may still experience considerable treatment burden due to technical, physical, logistical, and emotional aspects of treatment administration.
Aims: The objective of the current study was to examine the treatment experiences of CwH with the aim of developing a patient-centered measure of hemophilia to assess treatment burden in CwH, in accordance with the United States Food and Drug Administration guidelines for patient-reported outcome (PRO) measure development.
Methods: Concept elicitation (CE) data included a review of the current hemophilia literature and 1-1.5-hour interviews with 4 clinical experts, 25 CwH, and 25 caregivers of CwH. A clinical expert was defined as a hematologist or nurse who currently had at least a 50% patient load in hemophilia and had been providing care to CwH for ≥ 5 years. CwH had to: be male; be between the ages of 8 to < 12 years; have a diagnosis of hemophilia A or B, with or without an inhibitor, and a factor level of < 2%; and currently be receiving, by caregiver administering or self-administering (with or without assistance), either on-demand or prophylactic treatment for hemophilia via factor replacement therapy or a bypassing agent. For caregivers to be eligible, their CwH had similar eligibility criteria except that they had to be < 12 years of age.
The child, caregiver, and clinician CE interview transcripts were analyzed using Dedoose software. The information was synthesized and coded based on grounded theory principles. Based on this analysis, a theoretical model of the treatment experiences of CwH was developed and items reflecting the major and minor aspects of, distal consequences, and modifiers to the experience were included. Items reflecting the major concepts were generated and cognitive debriefing (CD) was conducted with 12 children and 15 caregivers.
Results: For the CE, the average age of the child participants was 9.6 years (median 10, range 8-11). Sixty-four percent (*n*=16) of the sample was White/Caucasian, all had severe hemophilia, the majority (*n*=23, 92%) had hemophilia A, and had been treated with factor for an average of 8.9 years (median 9, range 7-11). The average age of caregivers' CwH was 6.3 years (median 6, range 2-11). Forty-four percent of children (*n*=11) were White/Caucasian, all had severe hemophilia, the majority (*n*=22, 88%) had hemophilia A, and had been receiving treatment for an average of 6.0 years (median 5.5, range 2-11.5).
The treatment experience issues most frequently reported by 50% or more by either or both child participants or caregivers of CwH were: *Ease of Use:* inserting the needle; *Adherence:* missing or delaying treatments and difficulty keeping track of schedule/remembering to do treatment; *Emotional Impacts and Treatment Concerns:* feeling nervous, anxious or scared; proud/good about oneself; emotionally resolved/used to treatment; and stress; *Physical Impacts:* pain and scarring/scar tissue; and *Interference with Daily Life:* needing to stop activities to do treatment (see Table 1 for list of issues reported by ≥ 10% of either one or both child or caregiver and percent endorsements).
Based on the theoretical model (see Figure 1), a preliminary measure was generated, and the CD was completed. As a result of the CD, a decision was made to develop only a caregiver observer-reported outcome (ObsRO) measure to capture the experiences of children aged < 12 years. The decision to not develop a PRO was based on the interviews with children aged 10 to < 12 years, which resulted in 10 out of the 12 respondents using an incorrect timeframe when answering one or more items, about one-third answered one or more items related to the impact of a bleed and not their treatment, and over half of the children had issues with comprehension with one or more items and/or trouble navigating layout/skip pattern. Following CD, the validation-ready version of the measure, the Child Hemo-TEM, was finalized.
Conclusion: This study has identified key aspects of the treatment experience of CwH, which support thecontent validity of the Child Hemo-TEM ObsRO. Compared to generic measures, condition- and age-specific instruments, should have greater

face validity, be more responsive to change over time, and may be more useful to both clinicians and researchers to assess the hemophilia treatment burden of CwH.

Disclosures Brod: *Novo Nordisk A/S:* Consultancy. **Busk:** *Novo Nordisk A/S:* Current Employment, Current equity holder in publicly-traded company. **Neergaard:** *Novo Nordisk A/S:* Current Employment.

Table 1. Treatment Experience Issues (≥10% reporting) for Child and Caregiver of Child with Hemophilia

Adherence Issues, n (%)	Child who Ever Self-Injects Total (*n*=19)	Caregiver of Child who Ever Self-Injects Total (*n*=4)
Miss or delay treatment[a]	15 (79)	2 (50)
Miss or delay by accident	*9 (47)*	*2 (50)*
Miss or delay on purpose	*9 (47)*	*1 (25)*
Miss or delay due to factors beyond child's control	*4 (21)*	*1 (25)*
Difficulty keeping track of schedule/remembering to do treatment	8 (42)	2 (50)
Recognizing when on demand treatment is needed	0 (0)	1 (25)
Ease of Use Issues, n (%)	**Child Total (*n*=25)**	**Caregiver Total (*n*=25)**
Inserting the needle	17 (68)	3 (12)
Treatment steps	6 (24)	5 (20)
Finding a vein	6 (24)	2 (8)
Needing to do or receive multiple sticks	5 (20)	7 (28)
Treating outside home	4 (16)	1 (4)
Reconstitute/mix medicine	3 (12)	0 (0)
Emotional Impacts of Treatment, n (%)	**Child Total (*n*=25)**	**Caregiver Total (*n*=25)**
Nervous/anxious/scared	16 (64)	10 (40)
Proud/good about oneself	14 (56)	2 (8)
General annoyance	5 (20)	4 (16)
Happy	4 (16)	3 (12)
Emotionally resolved/feels used to treatment	3 (12)	16 (64)
Excited	3 (12)	5 (20)
Frustrated	3 (12)	5 (20)
Safe/protected	3 (12)	1 (4)
Calm	3 (12)	4 (16)
Confident	2 (8)	5 (20)
Crying	2 (8)	12 (48)
Stress	2 (8)	18 (72)
Sad	1 (4)	3 (12)
Upset	1 (4)	7 (28)
Anger	0 (0)	5 (20)

Treatment Concerns, n (%)	Child Total (*n*=25)	Caregiver Total (*n*=25)
Having to poke oneself or being poked	20 (80)	14 (56)
Making mistake/something going wrong during treatment	12 (48)	1 (4)
Missing the vein	*9 (36)*	*1 (4)*
Blowing a vein	*3 (12)*	*0 (0)*
Inserting needle	8 (32)	3 (12)
Treatment frequency	8 (32)	15 (60)
Amount of time child needs to spend to receive treatment	6 (24)	12 (48)
Port access issues	4 (16)	3 (12)
Needing to do or receive multiple sticks	0 (0)	6 (24)
Someone other than caregiver administering treatment	0 (0)	3 (12)
Treatment-related Physical Impacts, n (%)	**Child Total (*n*=25)**	**Caregiver Total (*n*=25)**
Pain	20 (80)	20 (80)
Bruising	12 (48)	10 (40)
Blown veins	10 (40)	9 (36)
Scarring/scar tissue	9 (36)	17 (68)
Soreness	8 (32)	5 (20)
Coldness	7 (28)	5 (20)
Treatment-related mobility issues	6 (24)	0 (0)
Taste in mouth during infusion	5 (20)	4 (16)
Infections	4 (16)	7 (28)
Port access issues	4 (16)	7 (28)
Stinging	4 (16)	1 (4)
Pinch sensation	3 (12)	2 (8)
Burning sensation	0 (0)	3 (12)
Physical discomfort	0 (0)	6 (24)
Interference with Daily Life, n (%)	**Child Total (*n*=25)**	**Caregiver Total (*n*=25)**
Treatment schedule/feeling like it is a bad time to receive treatment	10 (40)	11 (44)
Arrives late to school or other activities due to treatment	9 (36)	4 (16)
Need to stop activities to do treatment	6 (24)	13 (52)
Missing out on social activities	6 (24)	5 (20)
Overnight activities	5 (20)	5 (20)
Restrictions on physical activities or sports	3 (12)	6 (24)
Ability to travel	3 (12)	4 (16)
Avoidance of bathing and water activities	0 (0)	4 (16)

[a]Numbers in italicized sub-rows are not mutually exclusive - four child participants and one caregiver participant reported multiple reasons for the child delaying or missing treatments that were attributable to accidental, intentional and/or factors beyond child's control.

Fig. 1 Preliminary Theoretical Model of Hemophilia Treatment Impacts for Children with Hemophilia

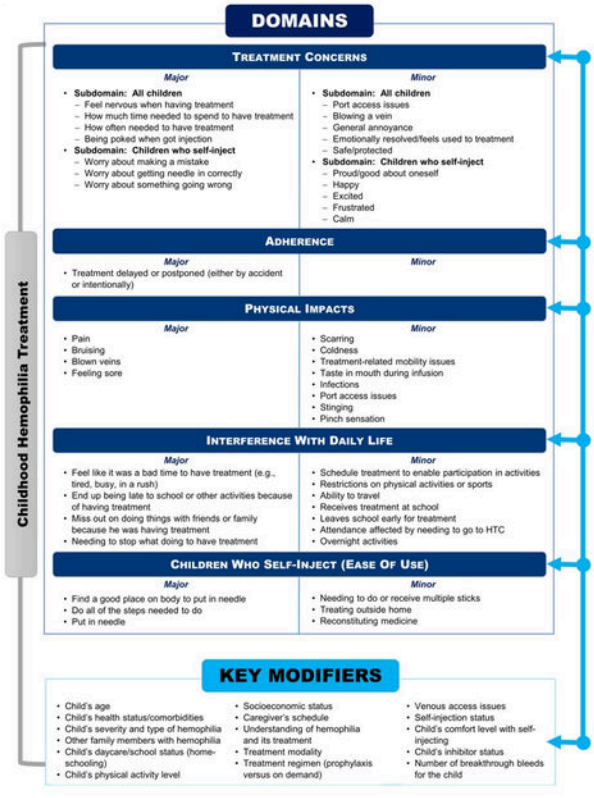

Figure 1

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