Development of a Patient-Reported Outcome Measuring Patients' Experience With Immunoglobulin Treatment

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INTRODUCTION

- Primary immunodeficiency diseases (PIDDs) occur when part of the immune system is impaired or absent due to a genetic disorder, putting individuals at increased risk of infections[1]
- Patients with PIDDs may require lifelong immunoglobulin replacement therapy (IGRT)[1]
- Patients' experiences with IGRT treatment may depend on the mode of administration (subcutaneous [SC] or intravenous [IV]) or location of administration (home or clinic)
- Although previous studies have investigated patients' experiences with IGRT, there is no patient-reported outcome (PRO) measure designed to systematically evaluate the entire patient experience for those using both SC and IV IGRT across all locations of administration[2]

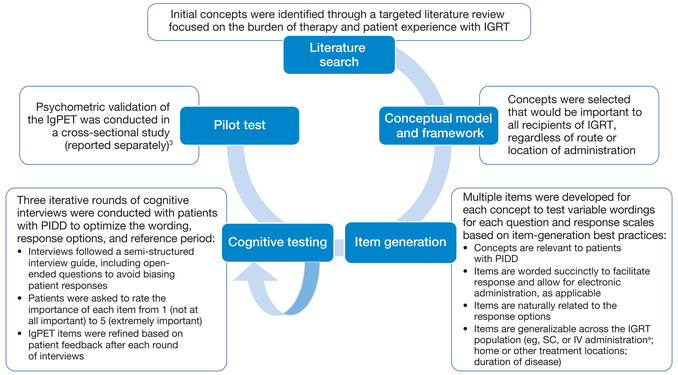
OBJECTIVE

- To develop a PRO measure evaluating patients' experiences with IGRT across modes of administration and treatment locations and obtain patient feedback on draft questionnaire items

METHODS

- The key steps in the development of the Ig Patient Experience with Treatment (IgPET) instrument are shown in **Figure 1**

Figure 1. Key steps in the development of the IgPET instrument



[a]Items could be generalized across routes of IGRT administration, including facilitated SC.
IgPET, Immunoglobulin Patient Experience with Treatment; IGRT, immunoglobulin replacement therapy; IV, intravenous; PIDD, primary immunodeficiency disease; SC, subcutaneous.

Patient interviews

- Recruitment
  - Patients with PIDD were identified and screened via email from a panel assembled by Rare Patient Voice, a specialty patient recruiting organization
  - Eligibility criteria:
    - Adult or adolescent ≥ 16 years of age
    - Self-reported PIDD diagnosis by a physician or other health care provider
    - Currently receiving IGRT for PIDD
    - Participant (and parent/legal guardian, if applicable) able to read, speak, and understand English
  - The study protocol was approved by the RTI International Institutional Review Board prior to recruitment
  - Verbal informed consent was obtained for all patients, or their parent/legal guardian, and reviewed with the patient at each interview
- Cognitive debriefing interviews
  - Cognitive debriefing is a technique used to determine whether patients understand the concepts and items as the developer of the PRO intended
  - Interviews were conducted by 2 RTI staff members during April and May 2017 via telephone
  - Interviews followed a semistructured interview guide, and lasted about 1.5 hours each for round 1, and 1 hour each for rounds 2 and 3
  - A copy of the draft items was sent to patients before the scheduled interview and displayed during the interview via a web-based meeting
    - 2 sets of response items were tested in all 3 rounds of interviews:
      - o A 5-point verbal response scale (VRS) ranging from "strongly agree" to "strongly disagree" for items related to treatment experiences
      - o A 5-point VRS ranging from "not at all" to "an extreme amount" for items related to treatment impact and global items
  - Interviews were audio recorded and transcribed for analysis

Analysis

- Patient feedback on individual items and overall themes was summarized and used to inform revisions to the draft IgPET items after each round of interviews
- Descriptive statistics were calculated for patient demographics and clinical characteristics

RESULTS

Participants

- A total of 21 patients with PIDD participated across 3 rounds of interviews
- Patient demographics and clinical characteristics collected at the time of screening are summarized in **Table 1**

Experience with IGRT

- Overall, patients reported that they did not consider their IGRT to be burdensome; instead, they regarded their IGRT as lifesaving therapy that gave them the opportunity to live a relatively normal life
- Patients were generally satisfied with their current IGRT, treatment frequency, and location of treatment

Table 1. Patient demographics and clinical characteristics

Characteristic	Round 1 (n = 7)	Round 2 (n = 6)	Round 3 (n = 8)	Total (N = 21)
Sex, n (%)				
Male	2 (28.6)	0 (0.0)	0 (0.0)	2 (9.5)
Female	5 (71.4)	6 (100)	8 (100)	19 (90.5)
Age in years, mean (range)	31.9 (17–53)	47.5 (37–58)	48.0 (32–70)	42.5 (17–70)
Years since diagnosis, mean (range)	8.4 (2–13)	6.7 (2–10)	9.5 (1–26)	8.3 (1–26)
Type of treatment, n (%)				
IV	4 (57.1)	3 (50.0)	4 (50.0)	11 (52.4)
SC	3 (42.9)	3 (50.0)	4 (50.0)	10 (47.6)
Treatment location, n (%)				
Home	6 (85.7)	5 (83.3)	5 (62.5)	16 (76.2)
Clinic/infusion center	1 (14.3)	1 (16.7)	3 (37.5)	5 (23.8)
Treatment administration, n (%)				
Self	2 (28.6)	3 (50.0)	5 (62.5)	10 (47.6)
Medical professional	4 (57.1)	2 (33.3)	3 (37.5)	9 (42.9)
Family member	1 (14.3)	1 (16.7)	0 (0.0)	2 (9.5)
Race/ethnicity, (%)				
White	7 (100)	6 (100)	7 (87.5)	20 (95.2)
Hispanic	0 (0.0)	0 (0.0)	1 (12.5)	1 (4.8)
Education, n (%)				
Less than high school	1[a] (14.3)	0 (0.0)	0 (0.0)	1 (4.8)
High school degree	1 (14.3)	0 (0.0)	1 (12.5)	2 (9.5)
Some college	0 (0.0)	2 (33.3)	2 (25.0)	4 (19.0)
College degree	3 (42.9)	2 (33.3)	2 (25.0)	7 (33.3)
Advanced degree	2 (28.6)	2 (33.3)	3 (37.5)	7 (33.3)

[a]Patient was a high school student (11th grade).
IV, intravenous; SC, subcutaneous.

Impacts of IGRT

- 20 of the 21 patients (95%) reported at least one negative impact related to their current IGRT:
  - Most frequently reported negative impacts were side effects (n = 11) and social/family activities (n = 6)
  - Less frequently reported negative impacts were daily activities, work/school, time, travel, needle sticks, and issues with ordering supplies/treatment and/or scheduling treatment visits

Cognitive debriefing

- 29 items were tested in round 1
- 20 items were tested in round 2
  - 2 additional items were tested in round 3 based on feedback from round 2: frustration with the process of ordering treatment and supplies and unhappiness with treatment nurses
- 20 items were tested in round 3
  - An item related to insurance was removed following round 3 because it was found to be more related to the current political climate than to the patient's individual IGRT
- 19 items were included in the final pilot IgPET instrument and were considered clear, easy to answer, and important to IGRT experiences (**Figure 2**)

Figure 2. Final IgPET item concepts

Frequency of treatments	Time required for treatments	Worry about side effects	Worry about treatment
Inconvenience of treatment scheduling	Time required for treatment preparation/setup	Interference with activities	Worry about costs
Time required for treatment planning	Feelings about treatment location	Interference immediately following treatment	Overall inconvenience of treatment
Frustrated with ordering process	Feelings about treatment nurses	Interference with work/school	Overall burden of treatment
Control over treatments	Number of needle sticks	Impact on travel	

IgPET, Immunoglobulin Patient Experience with Treatment.

- The IgPET items were found to be relevant and important to patients' IGRT experiences regardless of treatment modality, location, treatment administrator, or duration/severity of disease
- Patient-reported impacts of IGRT were generally similar across the modes and locations of treatment, with a few minor differences:
  - Some differences were observed among patients who received SC vs IV therapy; this was likely due to the frequency of infusions
  - Some differences were observed among patients who received treatment at home vs other treatment locations:
    - 2 patients who reported impacts on travel and family life were receiving IV therapy
    - 2 patients who reported time as an impact were receiving frequent SC treatments

CONCLUSIONS

- The IgPET instrument is a new PRO measure consisting of 19 items assessing concepts related to patient experiences with IGRT
- Patients reported being generally satisfied with their current IGRT treatment, frequency, and location of treatment, and did not find it to be burdensome; however, almost all patients did report some type of negative impact
- Limitations:
  - The sample size was small, potentially impacting generalizability of results to a broader population of patients with PIDD
  - Results may not be generalizable to adolescent or pediatric patients with PIDD or patients who live outside the US
- The psychometric properties and performance of the IgPET instrument have been evaluated in an observational study, including over 800 patients in the US and have been shown to support the reliability and validity of the IgPET instrument[3]
- Future studies may assess the potential for using the IgPET instrument in clinical practice

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DISCLOSURES
D DiBenedetti and E Evans are employees of RTI Health Solutions. LM Meckley is an employee of and a stockholder in Shire.

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