Poster IR009

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Assessing Alignment Between Standardized Patient-Reported Outcomes and Home-Reported Outcomes (HROs) in IgAN: Demonstrating the Value of HROs in Capturing the Full Patient Experience

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KEY FINDINGS & CONCLUSIONS

- While the PROs EQ-5D-5L and FACIT-F collectively cover only six domains (mobility, self-care, usual activities, pain/discomfort, anxiety/depression, and fatigue),[8,9] this study of HROs enabled participants to report on 40 symptoms and observations across 11 domains
  - HROs not only expand domain-level understanding but also provide granularity on individual symptom severity
- The alignment between HRO-tracked fatigue severity and FACIT-F scores at baseline supports the validity of home-reported tracking, with both reflecting moderate fatigue
- Thus, HROs add meaningful context by capturing individual experiences that standardized PROs may miss
- The ongoing IgAN Bridge study will continue to track HROs and PROs to gain a more comprehensive and patient-centered understanding of lived experiences with IgAN

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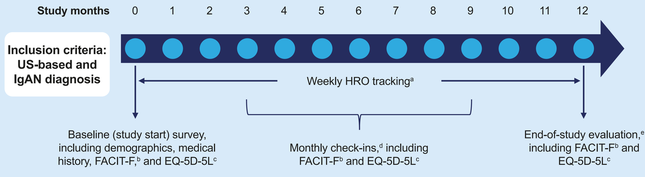
INTRODUCTION

- Immunoglobulin A nephropathy (IgAN) is the most common form of primary glomerulonephritis globally, with an incidence of ~2.5 cases per 100,000 adults per year[1,2]
- Marked heterogeneity exists in multiple disease aspects for IgAN, including disease progression[3]
  - IgAN progresses to kidney failure in ~50% of patients within 10–20 years[4,5]
- There are limited data on how patients manage their disease burden outside of clinical settings[7]
- Patient-reported outcomes (PROs), such as EuroQol 5-Dimension 5-Level (EQ-5D-5L) and Functional Assessment of Chronic Illness Therapy – Fatigue (FACIT-F), are standardized instruments that assess certain impacts of disease, such as mobility, pain, and fatigue[8,9]
  - These tools are used to assess patient perspectives in IgAN clinical trials,[10] but because they only evaluate specific parameters, they may miss important aspects of the IgAN experience
- In contrast, home-reported outcomes (HROs), which may be reported as often as daily and are defined by each patient's priorities, capture a broader range of symptoms, measurements, and treatments that PROs may overlook
- Collecting HROs alongside standardized PROs can provide a comprehensive yet individualized understanding of the patient experience with IgAN
- Here, we present a preliminary analysis assessing alignment between standardized PROs and HROs in the ongoing IgAN Bridge study

METHODS

- The IgAN Bridge study is an ongoing, remote, prospective, 12-month observational study (NCT06952426)[11] using the Folia Health mobile app to collect HROs, supplemented by PROs, from patients with IgAN in the US
- The primary objective is to gain a comprehensive understanding of the patient-reported burden of IgAN using HRO symptom tracking data
- The study will enroll at least 300 participants, comprising ~75% self-reporting adults and ~25% caregivers reporting on behalf of children with IgAN. Details of the study design have been presented previously[12]
- During the IgAN Bridge study, participants are required to (**Figure 1**):
  - Report on their demographics, health status, and treatment satisfaction at enrollment
  - Customize their mobile app profile at enrollment by selecting symptoms and treatments to track as HROs
    - Participants can choose symptoms and observations outside of the predefined selections
    - Each symptom is scored on a scale of 1 (least severe) to 5 (most severe)
    - Changes in symptoms, treatment plans, treatment utilization, and any flare events are required to be tracked at least once per week
  - Complete monthly surveys assessing health-related quality of life (HRQoL), including FACIT-F and EQ-5D-5L, starting at enrollment
  - Complete an end-of-study evaluation survey
- Data tracked by study participants will be automatically summarized into trend reports that can be used during medical appointments to inform their care directly

Figure 1. Study Design



[a]Changes in symptoms (incidence, severity), changes to treatment plans, treatment utilization (including non-use), and any flare events (heightened symptom severity vs baseline) are required to be tracked at least once per week. [b]The FACIT-F total scores range from 0 to 52, with higher scores indicating less fatigue. [c]The EQ-5D-5L Index scores range from less than 0 to 1, with higher scores indicating better HRQoL, and VAS scores range from 0 to 100, with higher scores indicating better self-rated health. [d]The monthly check-in questions include topics such as missed school or work, satisfaction with treatment (and any changes to treatment), and flares. [e]The end-of-study evaluation comprises monthly check-in questions with additional questions regarding study experience and perceived value.

EQ-5D-5L, EuroQol 5-Dimension 5-Level; FACIT-F, Functional Assessment of Chronic Illness Therapy – Fatigue; HRO, home-reported outcome; HRQoL, health-related quality of life; IgAN, immunoglobulin A nephropathy; US, United States; VAS, visual analog scale.

RESULTS

Baseline Characteristics

- Enrollment began in March 2025, and as of September 2025, preliminary analysis has been conducted on 45 participants
- Participants had a mean ± standard deviation (SD) age of 36.2 ± 12.8 years, 44% were female, 38% were White, and one-third reported a bachelor's degree as their highest level of education (**Table 1**)

Table 1. Demographic Characteristics of Participants at Baseline

Characteristic	Enrolled participants (N = 45)
Mean age ± SD, years	36.2 ± 12.8
Sex, n (%)	
Male	7 (16%)
Female	20 (44%)
Did not answer	18 (40%)
Race or ethnic group, n (%)	
Asian	5 (11%)
Black or African American	0
White or Caucasian	17 (38%)
Other[a]	1 (2%)
Prefer not to answer	1 (2%)
Selected more than one race	3 (7%)
Did not answer	18 (40%)
Highest level of education, n (%)	
High School Diploma	7 (16%)
Bachelor's degree	15 (33%)
Master's degree	4 (9%)
Trade School	1 (2%)
Did not answer	18 (40%)

[a]Includes Native American or Alaska Native, Native Hawaiian or Other Pacific Islander, or a different race or ethnic group not listed. SD, standard deviation.

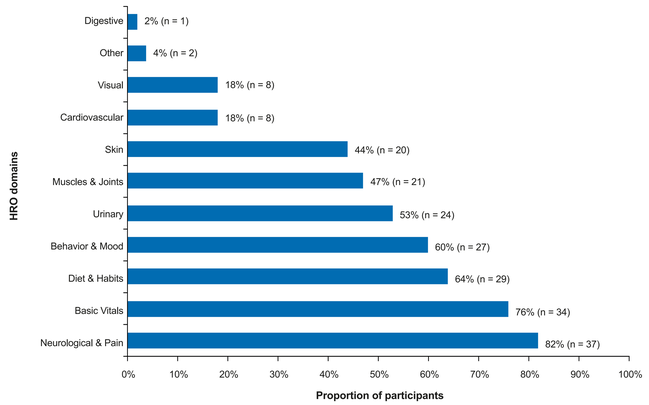
HRO Domain Categories

- Participants selected 40 IgAN symptoms and observations at baseline to track as HROs, which mapped to 11 domains
  - The most commonly tracked domains included Neurological & Pain (82%), Basic Vitals (76%), Diet & Habits (64%), Behavior & Mood (60%), and Urinary (53%) (**Figure 2**)
  - The most commonly tracked symptoms and observations included fatigue (76%), weight measurement (58%), anxiety (51%), changes in urine/pee (49%), difficulty sleeping (44%), blood in urine (42%), brain fog (40%), swelling (40%), and back pain (38%); additionally, 76% selected blood pressure for tracking (**Table 2**)
- Of the 40 participant-selected IgAN symptoms and observations, average symptom severity scores at baseline were available for 16 (36%) (**Table 2**)
  - Average ± SD severity scores for the frequently selected symptoms and observations – fatigue (2.94 ± 1.12), anxiety (2.82 ± 1.14), brain fog (2.94 ± 1.25), swelling (1.50 ± N/A), and back pain (2.93 ± 1.16) – indicate mild to moderate symptom burden at baseline

Fatigue Tracking by PROs and HROs

- Twenty-two (49%) participants completed the baseline FACIT-F survey with a mean ± SD score of 29.77 ± 16.05, indicating a mild to moderate level of fatigue
- Thirty-four (76%) participants selected fatigue as an HRO and reported a baseline average severity score of 2.94, aligning with a similar level of mild to moderate burden

Figure 2. Domain Mapping of IgAN Symptoms and Observations Selected for Tracking as HROs (N=45)



HRO, home-reported outcome; IgAN, immunoglobulin A nephropathy.

Table 2. IgAN Symptoms and Observations Selected for Tracking as HROs (N=45)

Domains	Items within domains: Symptoms and observations	Number of participants selecting each item, n (%)	Baseline average severity score[a]
Basic Vitals	Blood pressure	34 (76%)	–
	Cholesterol	1 (2%)	–
Behavior & Mood	Anxiety	23 (51%)	2.82
	Difficulty sleeping	20 (44%)	–
	Brain fog	18 (40%)	2.94
	Depression	15 (33%)	1.50
	Irritability	12 (27%)	2.92
	Daily stress	1 (2%)	2.00
Cardiovascular	Shortness of breath	8 (18%)	2.50
Diet & Habits	Weight measurement	26 (58%)	–
	Daily sodium intake	15 (33%)	–
	Daily potassium intake	9 (20%)	–
	Exercise/physical activity	1 (2%)	–
Digestive	Nausea	1 (2%)	–
Muscles & Joints	Joint pain	14 (31%)	3.08
	Muscle weakness	14 (31%)	2.07
	Muscle cramping	10 (22%)	2.00
Neurological & Pain	Fatigue	34 (76%)	2.94
	Back pain	17 (38%)	2.93
	Abdominal pain	9 (20%)	3.12
	Bone pain	6 (13%)	2.33
	Flank pain	1 (2%)	3.00
	Lightheadedness	1 (2%)	–
Skin	Swelling	18 (40%)	1.50
	Cold sensitivity	8 (18%)	–
	Heat sensitivity	8 (18%)	–
	Itching	1 (2%)	3.00
	Skin rash	1 (2%)	–
Urinary	Changes in urine/pee	22 (49%)	–
	Blood in urine/pee	19 (42%)	–
	Protein in urine (dipstick)	2 (4%)	–
	Frequency of urination	1 (2%)	–
	Foamy or bubbly urine	1 (2%)	–
	Protein in urine/pee	1 (2%)	–
Visual	Vision distortion	5 (11%)	–
	Vision loss	5 (11%)	–
	Visual aura	1 (2%)	–
	Visual floaters	1 (2%)	–
Other	eGFR	2 (4%)	–
	Urine protein-to-creatinine ratio	2 (4%)	–

For symptoms or observations tracked as presence/absence (Yes/No), count, or for measurements where severity would not apply (eg, weight measurement, protein in urine/pee), a baseline severity is not available.

[a]Each symptom is scored on a scale of 1 (least severe) to 5 (most severe).

eGFR, estimated glomerular filtration rate; HRO, home-reported outcome; IgAN, immunoglobulin A nephropathy.

Limitations

- All data collected will be self-reported, and as participants have different lived experiences of IgAN, this may impact on the longitudinal tracking patterns and volume of data each participant contributes
- Selection bias could affect the type of participants, as the study requires baseline technology literacy

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Abbreviations

eGFR, estimated glomerular filtration rate; EQ-5D-5L, EuroQol 5-Dimension 5-Level; FACIT-F, Functional Assessment of Chronic Illness Therapy – Fatigue; HRO, home-reported outcome; HRQoL, health-related quality of life; IgAN, immunoglobulin A nephropathy; N/A, not available; PRO, patient-reported outcome; SD, standard deviation; US, United States; VAS, visual analog scale.

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Disclosures

RK and **BN** are employees of Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA. **SM**, **CZ**, **AH**, **HA**, and **SV** are employees of Folia Health, Boston, MA, USA.