<u>Humanistic Burden of Rare KidNey Diseases: Understanding the Impact of FSGS and IgAN</u>

on Patients and Caregivers Study (HONUS) Rationale and Study Design

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Background

- Focal Segmental Glomerulosclerosis (FSGS) and IgA Nephropathy (IgAN) cause chronic glomerular disease worldwide, with annual incidences of 0.2 to 1.8 cases per 100,000 persons and one case per 100,000 persons for FSGS and IgAN, respectively^{1,2}
- The clinical and economic burden of FSGS and IgAN on healthcare systems is significant
- Few studies exist describing the humanistic burden of FSGS and IgAN on patients and their caregivers or partners in care and henceforth, carepartners³

Objective

 To explain the study design and methodology to be used to understand the impact of FSGS and IgAN on patients and their care-partners

Methods

Study design and data source

 This study will be a multi-national (Figure 1), cross-sectional survey, designed in consultation with patients and clinical community members. The minimum targeted sample size will be 290 participants.

Inclusion criteria

 Adult patients and their care-partners or care-partners/parents of children/adolescents who: (1) are ≥18 years of age; (2) have/care for someone with a diagnosis of FSGS or IgAN; (3) can give informed consent; (4) are located in the United States (US), United Kingdom (UK), Germany, France, Spain, or Italy

Exclusion criteria

 Patients and care-partners/parents who: (1) have FSGS or IgAN secondary to another condition; (2) have a history of malignancy other than adequately treated basal cell or squamous cell skin cancer; (3) have a co-existing glomerular disease (e.g., membranous nephropathy or lupus nephritis); (4) are currently participating in a kidney disease clinical trial and potentially receiving active treatment as part of the trial

Survey elements

 Patients will be recruited by patient advocacy groups and clinics/medical centers (Figure 2) to complete a comprehensive survey covering multiple elements including health-related quality of life (HRQoL) and the impacts of FSGS and IgAN (Figure 3 and 4)

Statistical analyses

- Characteristics and outcomes of patients and care-partners/parents will be summarized descriptively and separately for FSGS and IgAN. Total scores, composite scores, and domain/scale scores of the survey instruments will be calculated based on their scoring manuals.
- Continuous variables will be summarized using mean, median, standard deviation, and range while categorical variables will be summarized using frequency and percentage

Desired impact

 Our hope is that results of HONUS will support multiple stakeholders with information to facilitate awareness, communication, education and support (Figure 5)

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Figure 1. Geographic scope of participant recruitment

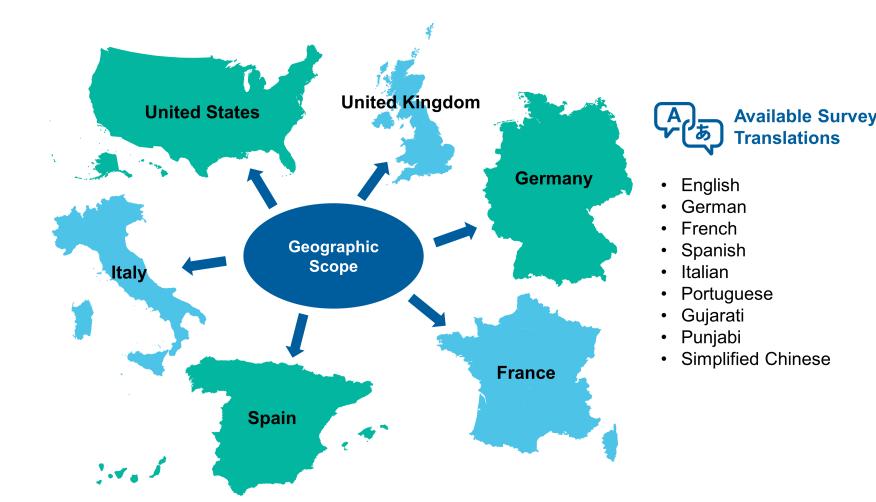


Figure 2. Planned steps for the recruitment of participants by patient advocacy groups and clinics/medical centers



 Clinic research staff screen existing patient lists for participants who have consented to email communications and participation in research

unique survey link including

study information, screening,

Abbreviations

Specific Health Problem

and consent

 Identified potential participants are sent email link including study information, screening, and consent

CPFQ, Cognitive and Physical Functioning Questionnaire; **FSGS,** Focal Segmental

Life; IgAN, IgA Nephropathy; KDQoL, Kidney Disease Quality of Life; PedsQL,

Pediatric Quality of Life Inventory; PHQ-9, Patient Health Questionnaire 9-item

Glomerulosclerosis; GAD, General Anxiety Disorder; HRQoL, Health-Related Quality of

survey; SF-12, 12-item Short-Form survey; UK, United Kingdom; US, United States;

WPAI, Work Productivity and Activity Impairment Questionnaire; **WPAI:SHP,** WPAI:

- activities
- invitation with unique survey
- study invitation letter with potential participants' clinic appointment letter/email
- Clinic research staff follow up with potential participants during their clinic visit to confirm interest
- Interested participants are sent email invitation with unique survey link including study information, screening, and consent

Figure 3. Participant populations and survey data elements



Group 1: Adult Patients

- Demographics Clinical characteristics
- Cognitive impairment
- Productivity Disease-related symptoms
- Other impacts: Education
- Career Employment
- Relationships Personal finances · Lifestyle

Group 2: Care-Partners of Adult Patients

- HRQoL
- Personal finances

Patient HRQoL

- Productivity
- Other impacts:
 - Education
 - Employment Relationships

Figure 4. Data collection domains for patients and parents/care-partners

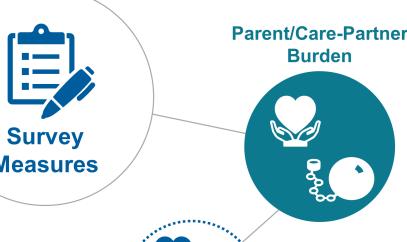
· Lifestyle

Group 3: Parents/Care-Partners of **Pediatric/Adolescent Patients**

- Patient Demographics Patient Clinical
- characteristics
- Patient HRQoL
- Disease-related symptoms reported by care-partners
- Care-partner burden (e.g., HRQoL, productivity)
- Other impacts: Education
 - Career **Employment**

 - Personal finances
- Lifestyle







HRQoL

- KDQoL-36⁴ domain scores Depression and anxiety
- scale scores: Total GAD-7⁵ score
- Total PHQ-9⁶ score • CPFQ⁷ cognition item
- Ranking of most burdensome symptoms Rating of fear and
- uncertainty for the future (5-point Likert scale) Productivity impairment for patients

Likert scale)

 WPAI⁸:SHP domain scores Other impacts (5-point)

Pediatric/Adolescent **Patients (Reported by**

Parents/Care-Partners)

- PedsQL Parent Proxy total and scale scores
- Ranking of most burdensome symptoms Rating of fear and

uncertainty for the future

(5-point Likert scale) Productivity impairment for

 Other impacts (5-point Likert scale)



and Parents/Care-Partners of **Pediatric/Adolescent Patients** Parent/Care-partner burden

Care-Partners of Adult Patients

SF-12⁹ composite and scale

- Depression and anxiety scale
- · Total GAD-7 score Total PHQ-9 score
- Rating of fear and uncertainty for the future (5-point Likert scale) WPAI⁸ domain scores
- Other impacts (5-point Likert

Figure 5. Desired multi-stakeholder impact of HONUS **Clinical Community**

- Support patient/family communication Educate the broader
- clinical community Contribute to informed decision making





Patients and Care-Partners

affected families

Validate and support individual experiences and

IGA NEPHROPATHY FOUNDATION





Limitations & Conclusion

Limitations

- · Although the study has been designed to be as comprehensive and robust as possible, we acknowledge inherent limitations:
- Patients and care-partners/parents who are willing and able to participate may be a selected population influenced by factors such as level of wellbeing or severity of illness, time constraints, or internet access
- The relatively small sample size of the study means that the findings in the survey may not represent all FSGS or IgAN patients and care-partners/parents nor the full spectrum of disease
- Due to small sample sizes, formal statistical testing may be limited
- Self-reported survey responses could be subject to inconsistencies as patient or care-partner/parent reporting of diagnosis and disease history may differ from their clinician's assessment

Conclusion

HONUS will provide evidence quantifying the humanistic burden of FSGS and IgAN from patient and care-partner perspectives

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