

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, care-partners³

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)

References

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Figure 1. Geographic scope of participant recruitment

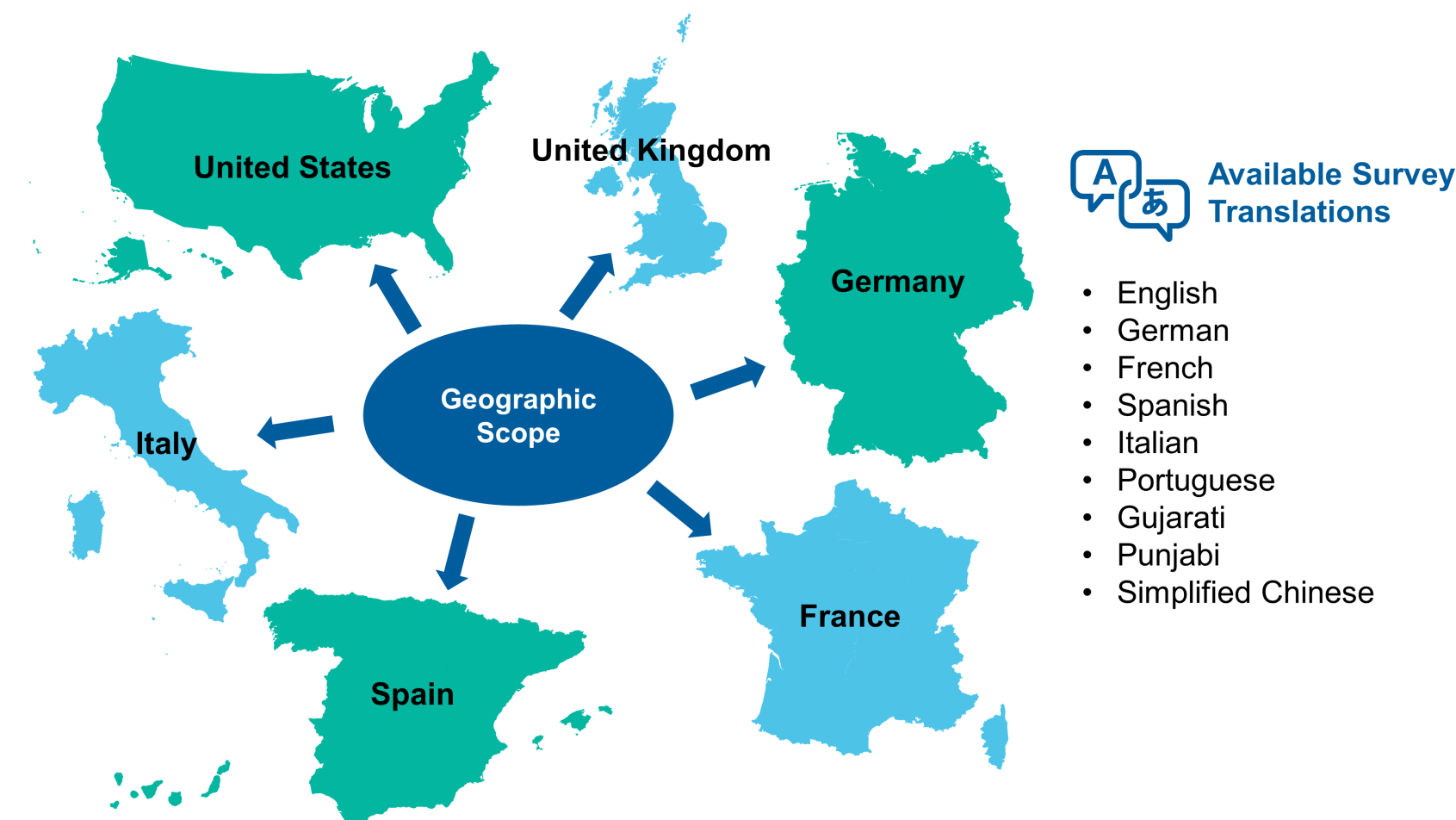
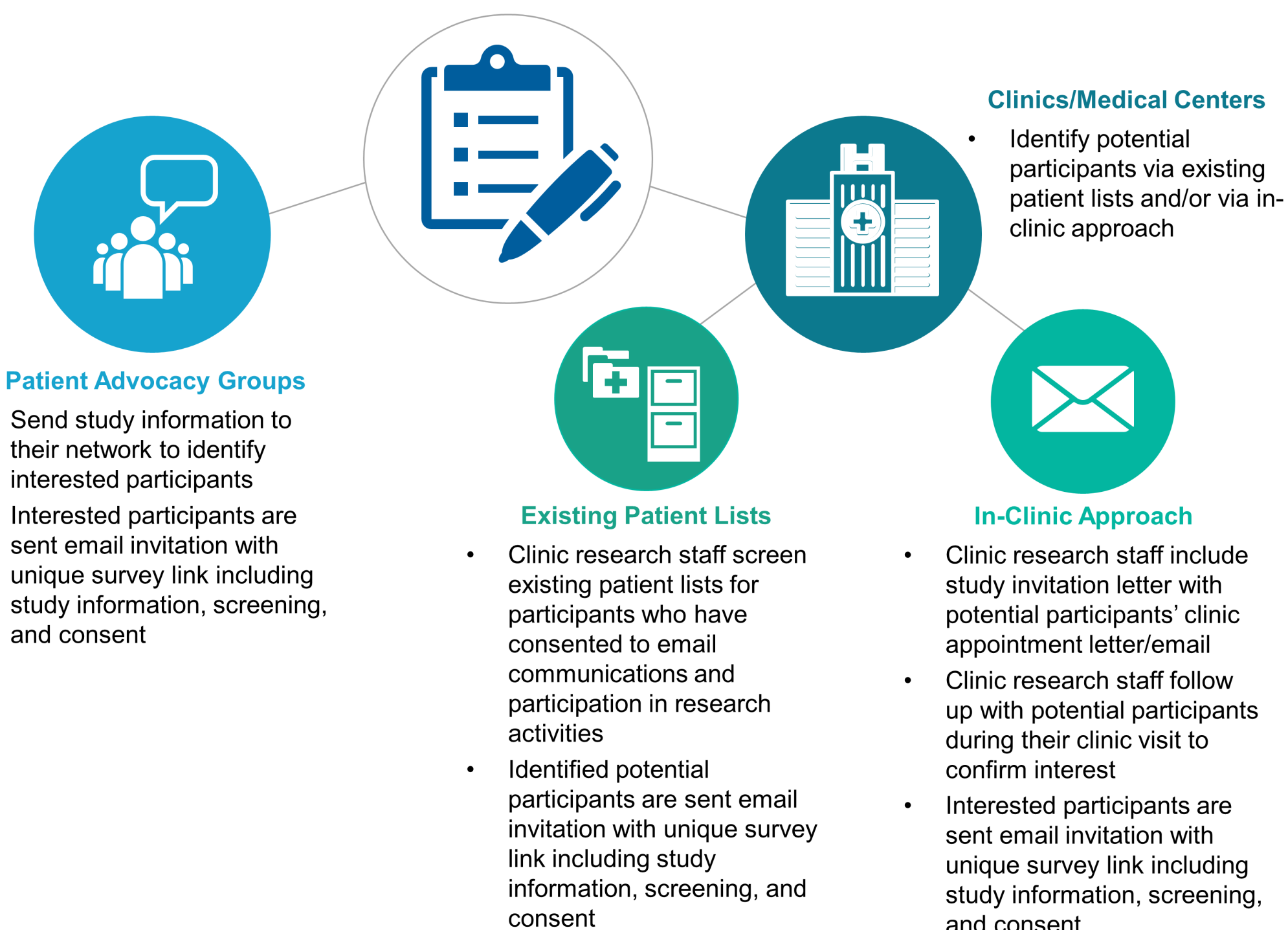


Figure 2. Planned steps for the recruitment of participants by patient advocacy groups and clinics/medical centers



Abbreviations

CPFQ, Cognitive and Physical Functioning Questionnaire; **FSGS**, Focal Segmental Glomerulosclerosis; **GAD**, General Anxiety Disorder; **HRQoL**, Health-Related Quality of Life; **IgAN**, IgA Nephropathy; **KDQoL**, Kidney Disease Quality of Life; **PedsQL**, Pediatric Quality of Life Inventory; **PHQ-9**, Patient Health Questionnaire 9-item survey; **SF-12**, 12-item Short-Form survey; **UK**, United Kingdom; **US**, United States; **WPAI**, Work Productivity and Activity Impairment Questionnaire; **WPAI:SHP**, WPAI: Specific Health Problem

Figure 3. Participant populations and survey data elements

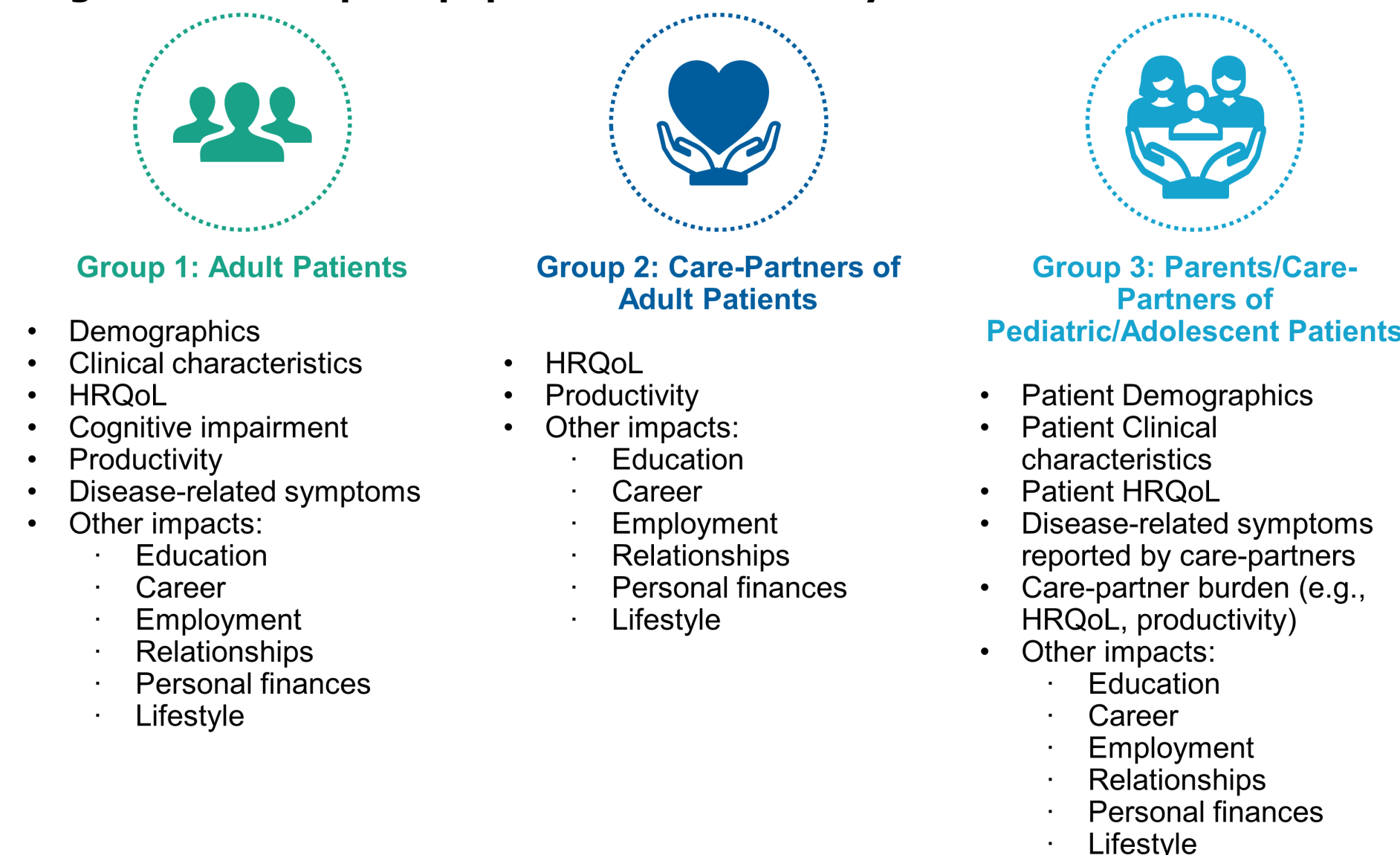
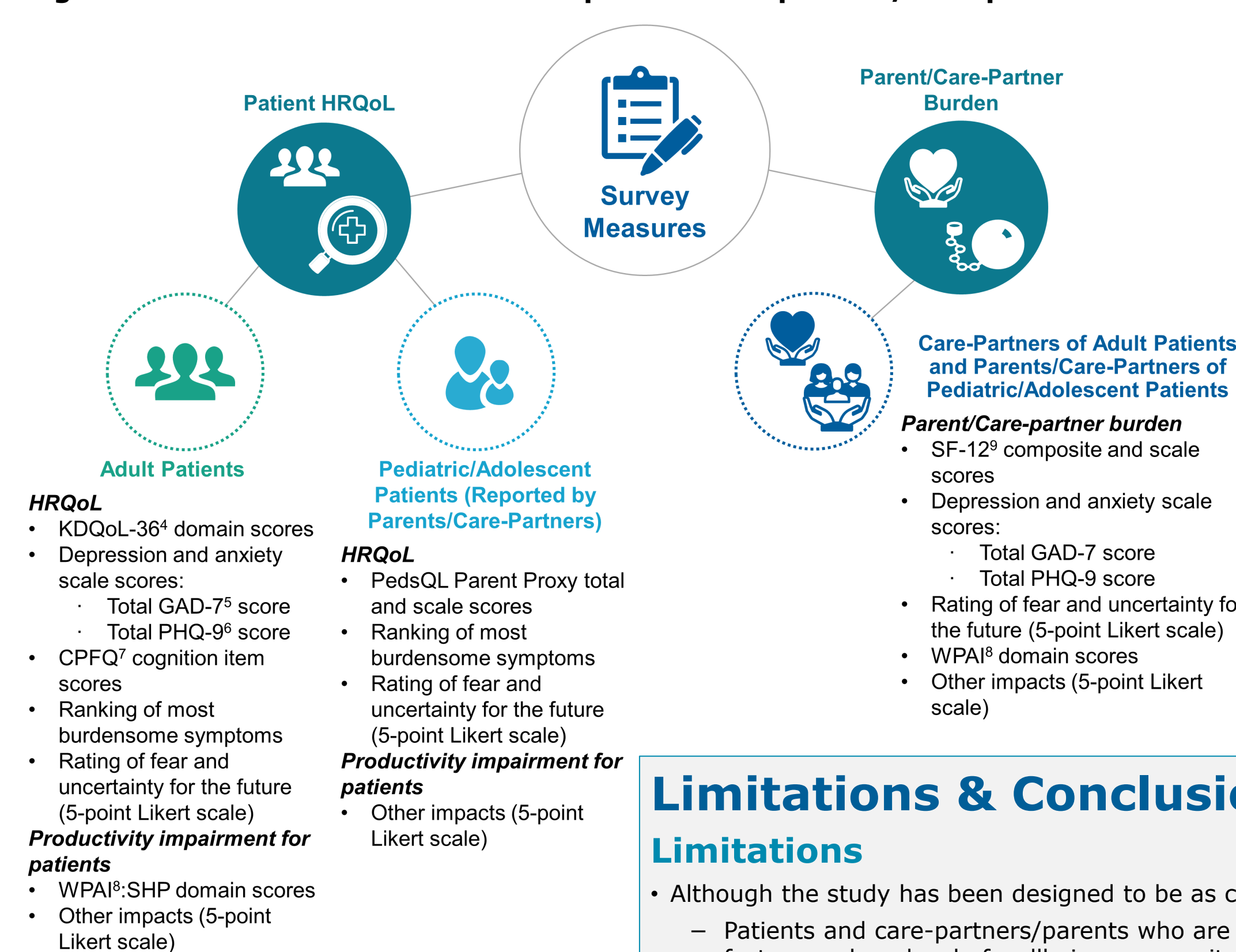


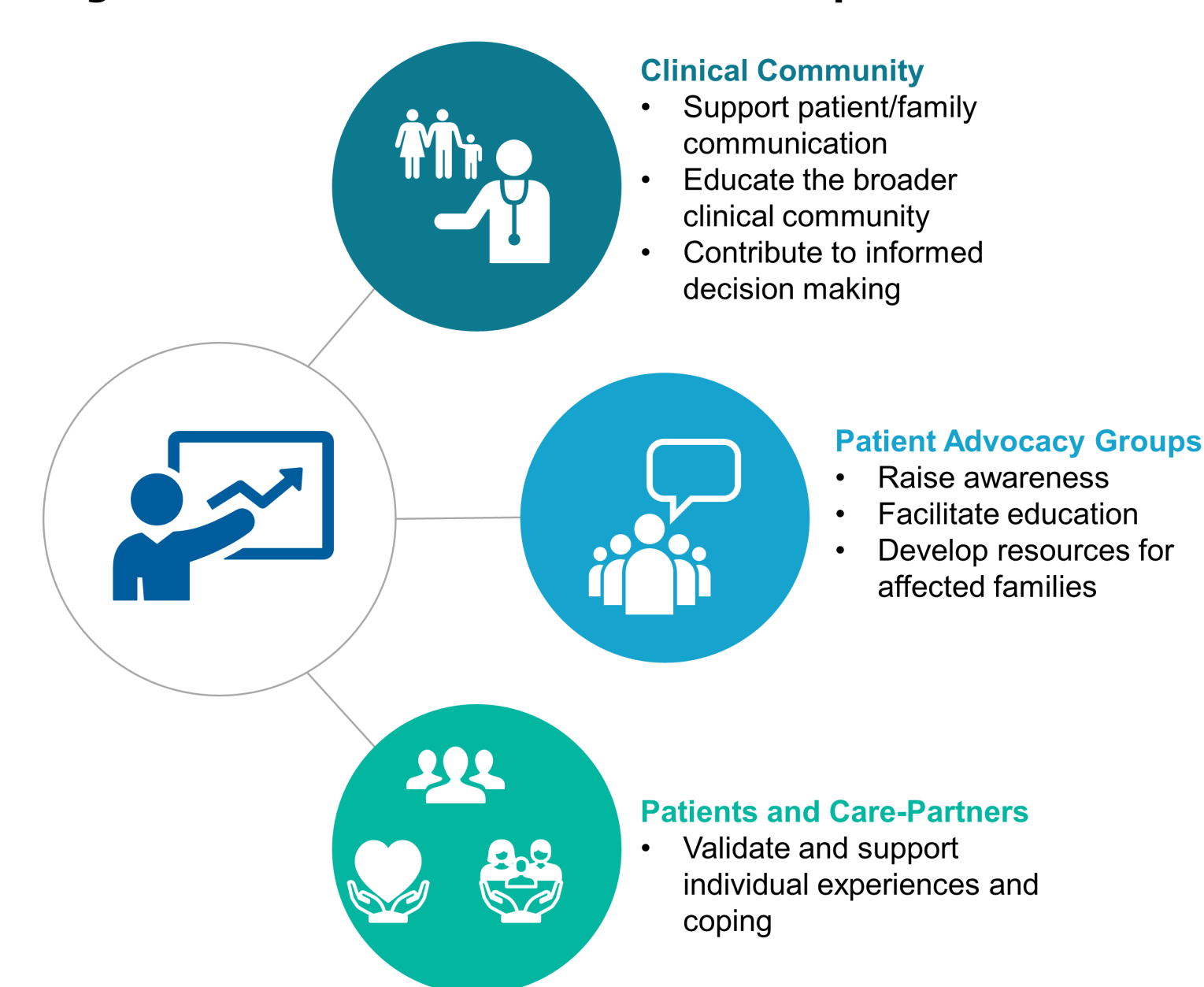
Figure 4. Data collection domains for patients and parents/care-partners



Disclosures & Funding

MB: Received consultancy fees from Traverse Therapeutics, Inc. and Amgen Inc. **KG**: Received consultancy fees from Traverse Therapeutics, Inc. **WT, ZZ, and MZ**: Are employees of Analysis Group, which received consultancy fees from Traverse Therapeutics, Inc. **DR and PS**: Have no competing interests to declare. This study is funded by Traverse Therapeutics, Inc. Writing support was provided by **Eve Hunter-Featherstone** of Genesis Research and was funded by Traverse Therapeutics, Inc.

Figure 5. Desired multi-stakeholder impact of HONUS



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