The Humanistic Burden of Rare Kidney Diseases: Understanding the Impact of Focal Segmental Glomerulosclerosis (FSGS) and Immunoglobulin A Nephropathy (IgAN) on Patients and Caregivers Study (HONUS): Results for FSGS in the United States (US)

Justyna Szklarzewicz,¹ Mark Bensink,² Ute Floege,³ Daniel Gallego,⁴ Kelly Helm,⁵ Keisha Gibson,⁶ Kamyar Kalantar-Zadeh,ˀ Ali Poyan-Mehr,ጾ Dale Robinson,⁶ Bonnie Schneider,¹⁰ Philip Smith,⁰ Kjell Tullus,¹¹ Value Robinson,⁰ Bonnie Schneider,² University Bonnie Robinson,⁰ Bonnie Schneider,² University Bonnie Robinson,⁰ Bonn Bruce Hendry,² Nisha C Hazra,¹² Chunyi Xu,¹² Jingyi Liu,¹² Zheng-Yi Zhou¹²

 Travere Therapeutics, Inc., San Diego, CA, USA; ³University Hospitals of Leicester, UK; ²Travere Therapeutics, Inc., San Diego, CA, USA; ³University Hospital of the RWTH Aachen University Hospital of the RWTH Aachen, Germany; ⁴European Kidney Patients Federation, FEDERACION ALCER Spanish k of North Carolina at Chapel Hill School of Medicine, Chapel Hill, NC, USA; ¹²Analysis Group, Boston, MA, USA; ¹⁴Great Ormond Street Hospital, London, UK; ¹²Analysis Group, Boston, MA, USA; ¹⁴Great Ormond Street Hospital, London, UK; ¹⁴Analysis Group, Boston, MA, USA; ¹⁵Chool of Medicine University of California, Irvine, CA, USA; ¹⁶Chool of Medicine University of California, Irvine, CA, USA; ¹⁸Chool of Medicine University of California, Irvine, CA, USA; ¹⁸Chool of Medicine University of California, Irvine, CA, USA; ¹⁸Chool of Medicine University of California, Irvine, CA, USA; ¹⁸Chool of Medicine University of California, Irvine, CA, USA; ¹⁸Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine, CA, USA; ¹⁹Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine University of California, Irvine, CA, USA; ¹⁹Chool of Medicine, CA, U

- 77 adult patient and care partner pairs, 1 adult patient (without care partner), and 29 pediatric caregivers were included in this analysis (**Table 1**)
- The average age of adult patients (N=78) was 43.4 (SD, 15.0) years; 74.4% were female, 68.0% were Caucasian, and one-third (35.9%) were employed full time
- Among pediatric patients (N=29), mean age was 12.0 (2.5) years; 58.6% were female, 69.0% were Caucasian, and 79.3% were full-time students
- Most care partners of adult patients were partners (63.6%) or parents (24.7%); most caregivers of pediatric patients were parents (96.6%)

Table 1. Sociodemographic Characteristics

	Adult patients (N=78)	adult patients (N=77)	(via caregiver proxy) (N=29)	pediatric patients (N=29)
Age, y				
Mean ± SD	43.4 ± 15.0	48.0 ± 14.5	12.0 ± 2.5	43.2 ± 5.3
Median	44.5	47.0	12.0	42.0
Sex, n (%)				
Male	18 (23.1)	35 (52.0)	12 (41.4)	0 (0.0)
Female	58 (74.4)	40 (45.5)	17 (58.6)	29 (100.0)
Other/unknown	2 (2.6)	2 (2.6)	0 (0.0)	0 (0.0)
Race and ethnicity, n (%)				
Caucasian	53 (68.0)	53 (68.8)	20 (69.0)	22 (75.9)
African American	16 (20.6)	16 (20.8)	4 (13.8)	2 (6.9)
Hispanic	9 (11.5)	10 (13.0)	3 (10.3)	2 (6.9)
Asian and Pacific Islander	1 (1.3)	0 (0.0)	3 (10.3)	4 (13.8)
Native American	4 (5.1)	1 (1.3)	2 (6.9)	0 (0.0)
Prefer not to answer	1 (1.3)	1 (1.3)	0 (0.0)	0 (0.0)
Employment status, n (%)				
Full time	28 (35.9)	41 (53.3)	_	14 (48.3)
Part time	7 (9.0)	8 (10.4)	_	2 (6.9)
Self-employed	4 (5.1)	8 (10.4)	_	0 (0.0)
Looking for work	4 (5.1)	3 (3.9)	_	1 (3.5)
Not looking for work	6 (7.7)	5 (5.2)	_	0 (0.0)
Retired	6 (7.7)	6 (7.8)	_	1 (3.5)
Disability	15 (19.2)	1 (1.3)	_	1 (3.5)
Student	2 (2.6)	0 (0.0)	_	0 (0.0)
Homemaker	2 (2.6)	5 (6.5)	_	9 (31.0)
Other	4 (5.1)	1 (1.3)	_	1 (3.5)
School status, n (%)				
Full time	_	_	23 (79.3)	_
Homeschool	_	_	4 (13.8)	_
Not attending school	_	_	1 (3.5)	_
Part time	_	_	1 (3.5)	_

Patient Disease Characteristics

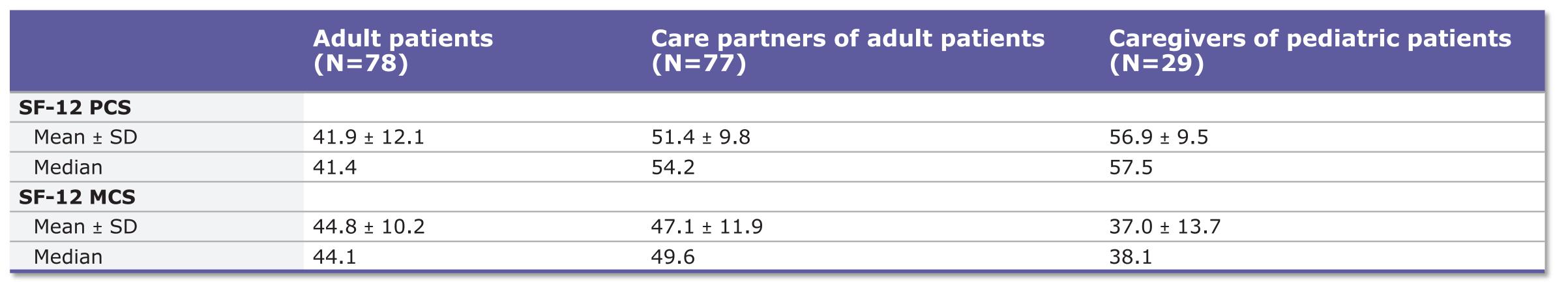
SD, standard deviation.

- Patients had been living with FSGS for an average of 11.1 years (adults) and 5.4 years (pediatric patients)
- Approximately one-third of adult patients were in chronic kidney disease (CKD) stages 1 or 2 (30.8%), one-quarter in CKD stage 3 (25.6%), 3.9% in CKD stage 4, and 1.3% in CKD stage 5. Eighteen (23.1%) adult patients were receiving dialysis, 10 (12.8%) had experienced kidney failure and received a kidney transplant; unknown CKD stage was 2.6%. CKĎ stage in the pediatric population was CKD stage 1 or 2, 34.5%; CKĎ stage 3, 10.3%; CKĎ stage 4, 6.9%; receiving dialysis, 10.3%, kidney transplant, 13.8%; unknown 24.1%
- The most common comorbidities among adult patients included hypertension (76.9%), anemia (56.4%), and depression (30.8%); among pediatric patients, hypertension (72.4%), anemia (41.4%), and heart failure (34.5%) were most common
- Focal segmental glomerulosclerosis (FSGS) is a rare kidney disease shown to cause significant clinical and economic burden; however, less is known about the humanistic burden associated with the disease¹
- HONUS (<u>H</u>umanistic Burden <u>o</u>f Rare Kid<u>N</u>ey Diseases: Understanding the impact of FSGS and IgAN on Patients and Caregivers **S**tudy) is a multinational, sectional survey designed in consultation with ents with immunoglobulin A nephropathy (IgAN) or and clinical community members. HONUS aims to the humanistic burden of rare kidney diseases from both patient and caregiver (care partner)
- The current analysis focused on patients with FSGS and their caregivers or care partners in the US who participated in HONUS

Health-Related Quality of Life

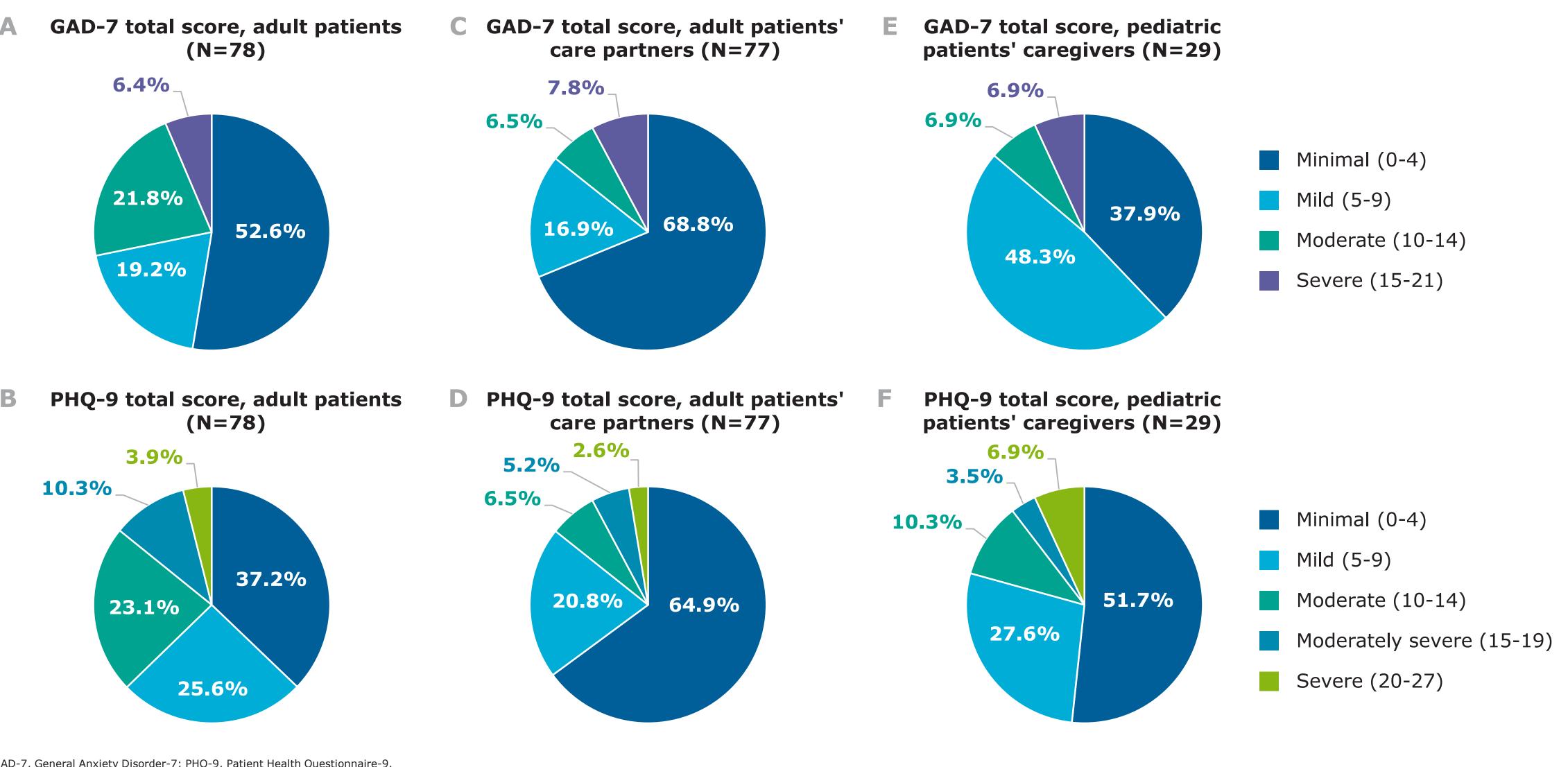
- For adult patients, mean 12-Item Short Form Survey (SF-12) scores for both mental and physical components reflected worse health-related quality of life (HRQOL) (lower score) than previously published US general population scores (physical component summary and mental component summary of 50 [SD, 10]).9 For care partners/caregivers, SF-12 mental components were lower than general population estimates (Table 2)
- Kidney Disease Quality of Life Instrument-36 (KDQoL-36™) scores (higher score = better HRQOL) in adult patients were: - Mean (SD) burden of kidney disease, 45.9 (29.3); symptom/problems, 67.9 (16.0); effects of kidney disease, 65.3
- For pediatric patients, mean (SD) Pediatric Quality of Life Inventory (PedsQL™) total score (reported via parent/caregiver proxy) was 65.8 (20.6), reflecting worse HRQOL (lower score) than previously published US proxy-report scores in a general pediatric population (total score of 81.3 [15.9])
- Individual item scores were also lower across all domains compared with general population estimates, mean (SD): physical, 69.8 (23.8); psychosocial, 61.7 (19.4) including emotional, 60.5 (21.5); social, 67.9 (19.3); and school, 56.7 (26.1) functioning
- One-quarter of adult patients reported at least moderate anxiety (Figure 1A), and approximately one-third had at least moderate depression (Figure 1B). Approximately 15% to 20% of adult care partners and pediatric caregivers experienced moderate to severe anxiety or depression (Figure 1C-1F)

Table 2. HRQOL for Adult Patients With FSGS, Their Care Partners, and Pediatric Caregivers



GGS, focal segmental glomerulosclerosis; HRQOL, health-related quality of life; MCS, Mental Component Summary; PCS, Physical Component Summary; SF-12, 12-Item Short Form Survey.

Figure 1. GAD-7 and PHQ-9 Total Scores*,†



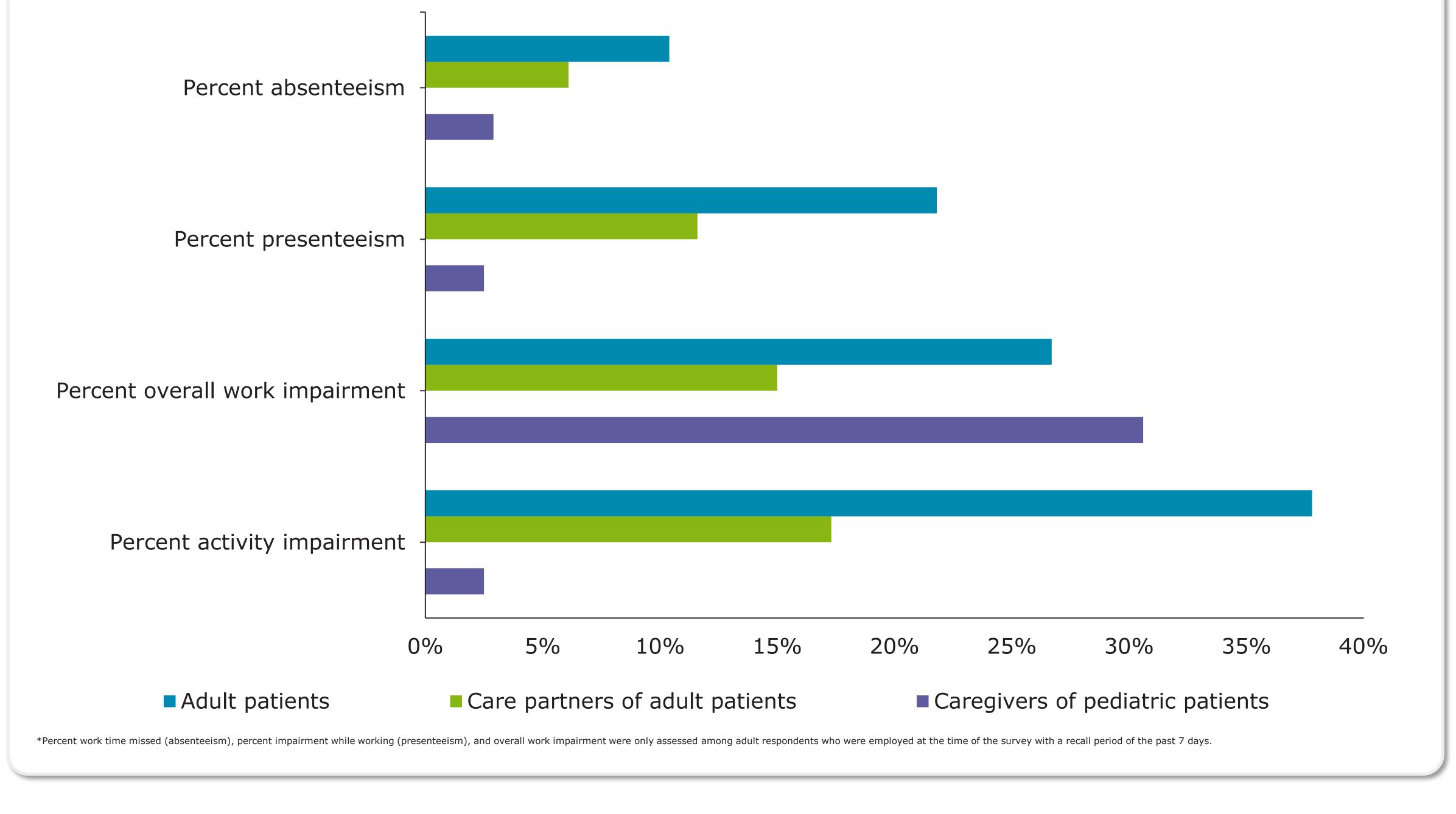
Most Burdensome Symptoms and Fear of the Future

- The top 3 most burdensome symptoms reported by adult patients were bone or joint pain (68.0%), headache (60.3%), and facial swelling (56.4%); for pediatric patients, the most burdensome symptoms were headache (62.1%), bone or joint pain (55.2%), and embarrassment (34.5%)
- Over one-third of adult patients (35.9%) and 1 in 6 pediatric patients (17.2%) experienced feelings of fear and uncertainty for the future due to their disease "often" or "always"
- Feelings of fear and uncertainty for the future were reported as "often" or "always" by 40.3% of adult patient care partners and 58.6% of pediatric patient caregivers

Work Productivity

- About half of adult patients (51.3%), most adult care partners (74.0%), and more than half of pediatric caregivers (55.2%) had worked in the past 7 days
- A significant proportion of employed individuals reported various levels of absenteeism, presenteeism, and overall work impairment due to FSGS-related reasons (higher score = greater impairment and less productivity), with highest impairment among adult patients. Overall activity impairment among all participants was also substantial and highest among adult patients due to FSGS-related reasons (Figure 2)

Figure 2. WPAI:SHP of Adult Patients With FSGS, Their Care Partners, and Pediatric Patients' Caregivers



LIMITATIONS

- Selection bias may exist as participants who voluntarily participated in the survey may differ from those who did not; in addition, the sample size of the pediatric population is small
- The study relied on self-reported survey responses and proxy responses (for pediatric patients) and therefore could be subject to biases. Patients' self-reported diagnosis and disease history of FSGS may also differ from a clinician's
- Participant responses may be confounded by the COVID-19 pandemic. This may impact socioeconomic status, accessibility of care, health outcomes, and reported HRQOL

Adult patients with FSGS,

To understand the burden and impact of and pediatric patient's caregiver perspective

their care partners, and

advocacy group (NephCure Kidney International) and 2 medical centers (University of North Carolina Kidney Center and Kaiser Permanente) in the US. The survey was approved by the Pearl Institutional Review Board (Indianapolis, IN) and is Health Insurance

Portability and Accountability

Act compliant.

Study Population

Inclusion criteria

 Adult patients (≥18 years old), their paired adult care partners or caregivers of pediatric patients (8-17 years old) in the US who had a physician-provided diagnosis of FSGS and were able to provide informed consent

Exclusion criteria

- Patients (and their care partner/caregiver) were excluded if the patient had FSGS secondary to another condition
 - History of malignancy other than adequately treated basal cell or squamous cell skin cancer

Participated in a kidney disease clinical trial and potentially received

active treatment as part of the trial at the time of recruitment

 Coexisting glomerular disease (e.g., membranous nephropathy or lupus nephritis

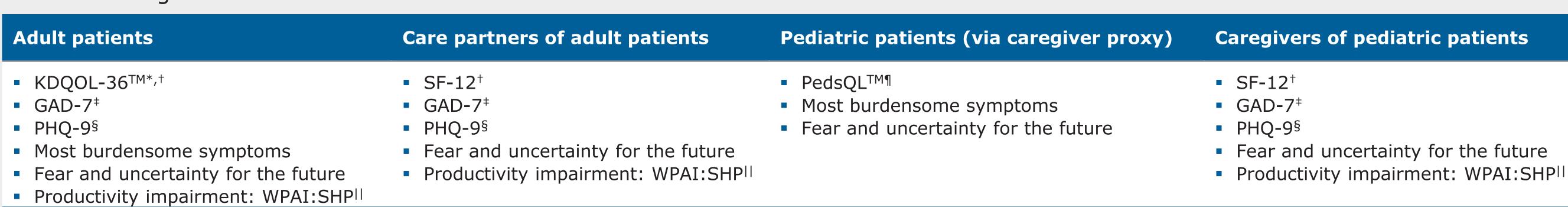
KDQOL-36™ is a short form that includes the SF-12 as a generic core plus burden of kidney disease, symptoms/problems of kidney disease scales from the KDQOL-SF™ v1.3.3 †SF-12 is a general health questionnaire that assesses the impact of health on everyday life.4 ‡GAD-7 is a 7-item instrument that is used to neasure or assess the severity of generalized anxiety disorder.5 §PHQ-9 is the 9-item depression module from the full PHQ and measures the effect of specific health problems on worker productivity.7 ¶PedsQL™ assesses the health-related quality of life of children and adolescents, with responses

Analysis All outcomes were summarized descriptively. Continuous variables were summarized as mean, median, and standard deviation (SD);

categorical variables were summarized as count and proportion

Study Outcomes

The following data were collected among adult patients with FSGS, their care partners, pediatric patients with FSGS (via parent/caregiver proxy), and their caregivers:



Adult patients with FSGS in the US experienced impaired mental and physical health compared with the US general population; the disease also resulted in negative impacts on work productivity

CONCLUSIONS

Poster TH-P0597

Pediatric patients with FSGS also experienced impaired health-related quality of life across all domains (physical and psychosocial, including emotional, social, and school functioning) compared with US general pediatric population estimates

Both care partners of adult patients with FSGS and caregivers of pediatric patients with FSGS had considerably worse mental health compared with the US general population; care partner and caregiver work productivity was also negatively impaired

DISCLOSURES

JS, UF, DG, KG, KK-Z, KH, DR, BS, PS, KT, AP-M: Received consultancy fees from Travere Therapeutics, Inc. **BH:** Employee, Travere Therapeutics, Inc. MB: Managing director of Benofit Consulting, which received consulting fees from Travere Therapeutics, Inc. NCH, CX, JL, Z-YZ: Employees of Analysis Group, which received consultancy fees from Travere Therapeutics, Inc.

ACKNOWLEDGMENTS

This study was funded by Travere Therapeutics, Inc. Editorial support was provided by Astika Bhugeloo, PhD, of Articulate Science (US), a part of Nucleus Global, an Inizio Company and was funded by Travere Therapeutics,

REFERENCES

1. Kalantar-Zadeh K, et al. Kidney Int Rep. 2021;6(10): 2679-2688. **2.** Szklarzewicz J, et al. *J Am Soc Nephrol*. 2021;32:466. Abstract PO1479. **3.** Peipert JD, et al. *J Am* Soc Nephrol. 2019;30(4):654-663. 4. Kosinski M, et al. User's Manual for the SF-12v2 Health Survey. Quality Metric Incorporated; 2007. **5.** Spitzer RL, et al. *Arch Int Med*. 2006;166(10);1092-1097. **6.** Kroenke K, et al. *J* Gen Intern Med. 2001;16(9):606-613. 7. Reilly MC, et al. Pharmacoeconomics. 1993;4(5):353-365. 8. Varni JW, et al. *Ambul Pediatr*. 2003;3(6):329-341. **9.** Ware JE. Spine. 2000;25(24):3130-3139.

To obtain a PDF of this poster:



Please scan the Quick Response (QR) code.

No personal information is stored.

