

BRAVE-PWS: Burden and Real-World Assessment of Caregiver Voice and Experiences in Prader-Willi Syndrome—Study Design of an Observational, App-Based Health-Tracking Cohort Study

Christopher Joshi, MD, MPH¹, Robert Dufour, PhD², Samantha McStocker, BS¹, Sarah Leuzzi, DPT², Mae Kwong, PharmD²

¹Folia Health
²Soleno Therapeutics, a Neurocrine Biosciences Co.

Poster #8
FPWR Research Symposium
October 7, 2026
Philadelphia, PA

INTRODUCTION

- Caregivers of individuals with PWS experience high levels of burden
- Prior studies show negative effects on mental health, sleep, employment, social functioning, and family relationships
- Hyperphagia and associated behavioral symptoms serve as key drivers of burden, including the constant vigilance, anticipatory anxiety, and planning required to prevent access to food and manage behavioral escalations.^{1,2,3}
- Despite growing recognition of the caregiver and family impact of PWS, important gaps remain in the literature, especially in
 - quantifying the impact of situational and environmental contexts, such as holidays, school breaks, or disruptions to routine, and
 - linking fluctuations in hyperphagia and downstream behavioral symptoms with contemporaneous changes in caregiver quality of life.

Home-reported outcomes (HROs) are uniquely equipped to address this gap. HROs are structured, patient-generated observations tracked in the Folia Health app. Because patients and caregivers determine what they track, HROs can capture symptoms often missed by other RWE methods, particularly in rare diseases, as well as non-symptom observations such as the situational and environmental contexts described above.

OBJECTIVES

Design an observational study using HROs to quantitatively characterize the real-world caregiver and family burden associated with hyperphagia in individuals with PWS.

Additional secondary objectives:

- Clarify the relationship between hyperphagia and downstream behavioral symptoms (e.g., aggression, emotional outbursts), and explore how these behaviors contribute to caregiver burden and quality of life.
- Identify contextual and situational triggers (e.g., social gatherings, schedule changes) that exacerbate hyperphagia and related behaviors, and assess how these periods may affect caregiver stress and family impact.
- Identify, categorize, and quantify any positive experience/events (e.g., enjoyable experience at a family gathering) that may be captured during the observation period.

METHODS

In order to inform study design, qualitative, semi-structured interviews were conducted with caregivers of individuals with PWS and clinician experts. Caregivers were asked about their child’s typical symptoms and behaviors, current and past treatments, the impact on their own life as caregivers, and their experience with and preferences for health tracking. Clinicians were asked open-ended questions about how they monitor PWS in their patients, what information they use to make management recommendations, and what information is missing in the current literature.

Interviews were conducted with three clinicians specializing in PWS: a pediatric psychiatrist, pediatric endocrinologist, and pediatric geneticist.

Demographic		Value
Caregiver relation to individual with PWS	Mother, n (%)	6 (100%)
Age of individual with PWS	Median (range)	6.5 (4–27) years
Location	USA, n (%)	5 (83%)
	Canada, n (%)	1 (17%)
Pharmacotherapy	Current VYKAT XR use, n (%)	1 (17%)

Table 1. Demographics from caregiver interviews

RESULTS

Informed by semi-structured qualitative interviews with caregivers and KOLs, a remote observational study was designed to contemporaneously measure PWS symptoms and behaviors alongside indicators of caregiver burden. Caregiver participants use the Folia Health app to track HROs for themselves and the individuals for whom they care and complete monthly standardized surveys over a 6-month data collection period.

Participants record changes in HROs as they observe them, at frequencies ranging from daily to weekly, over the course of the study. Additionally, participants can add additional HROs of interest at any time throughout the study. Figure 1 shows an example screenshot from the app after a caregiver has tracked their HROs for the day.

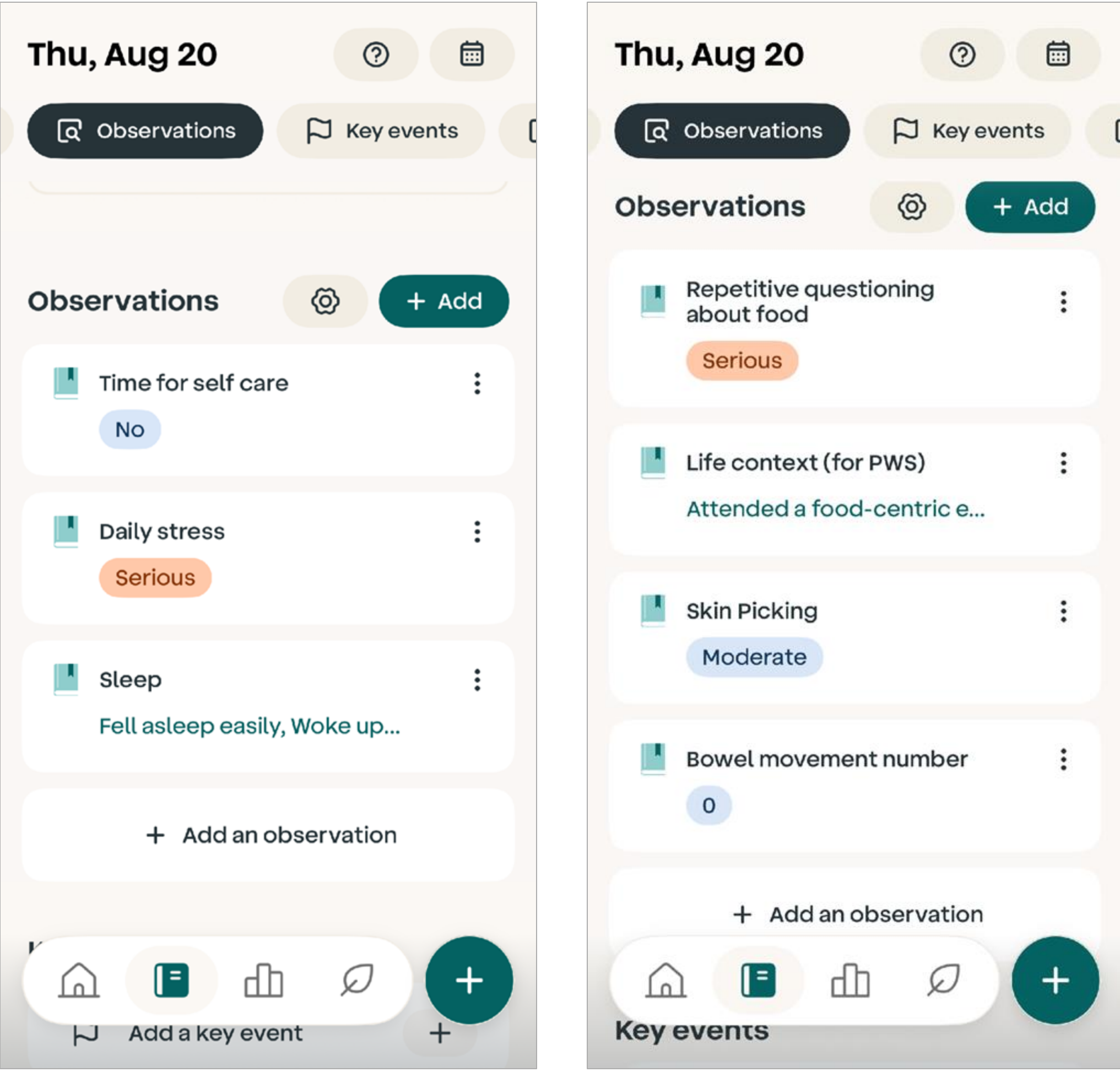


Figure 1. Screenshots from the Folia Health app of an example PWS caregiver who tracked HROs for (a) themselves and (b) their child with PWS

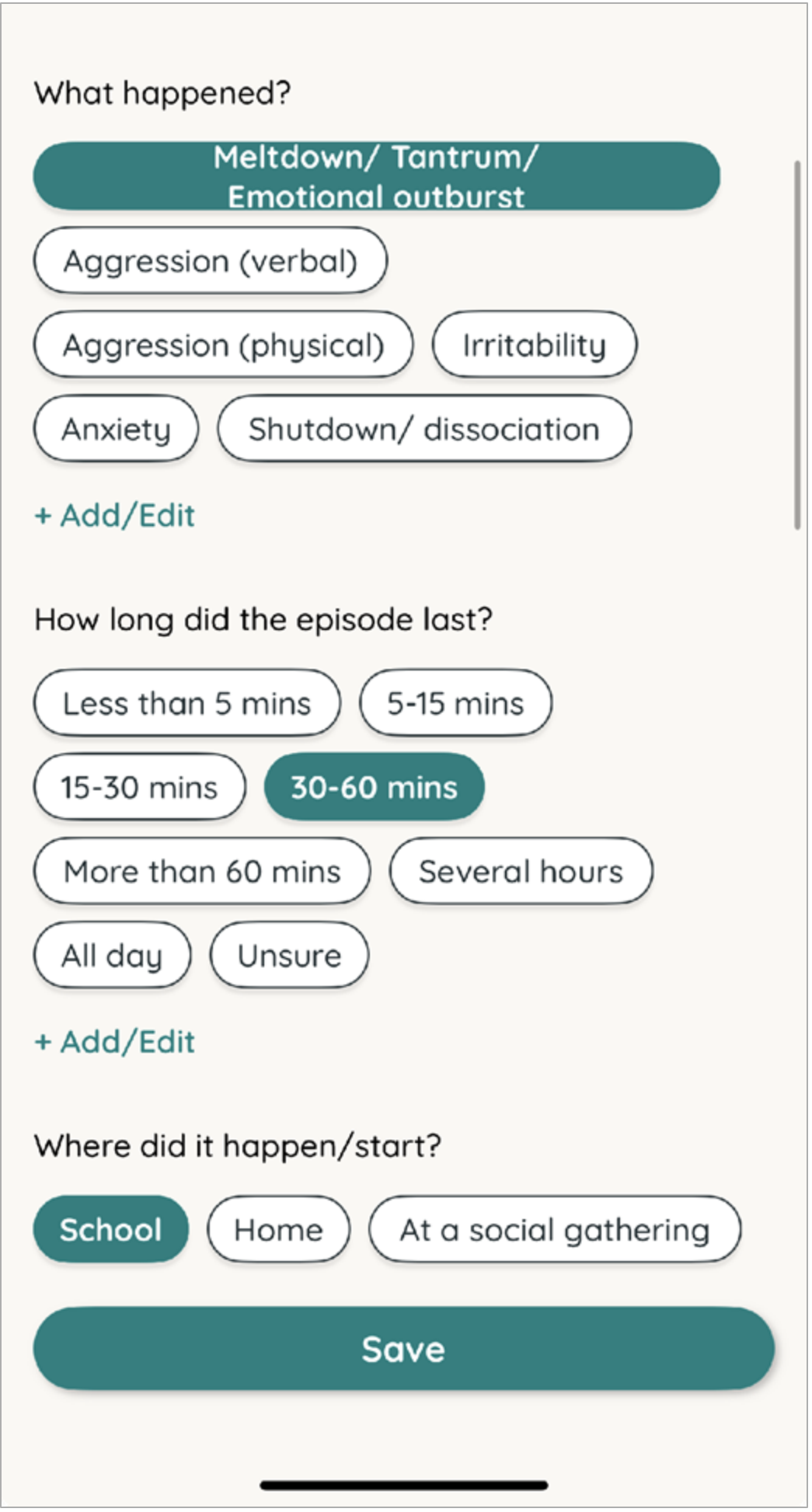


Figure 2. Screenshot of the first three questions of the “behavioral episode” form

Caregiver HROs

Suggested caregiver HROs, offered at enrollment as tracking options in the participants’ Folia profiles, include sleep, anxiety, daily stress, depression, fatigue, difficulty concentrating, irritability, and time for self care.

PWS Patient HROs

A larger set of observations are suggested for the individual with PWS, spanning hyperphagia, behavior and mood, sleep, and GI symptoms (Figure 3). A “behavioral episode” form lets caregivers log episodes of heightened behaviors, such as meltdowns or aggressive outbursts, including triggers, de-escalation strategies, and caregiver impact (Figure 2).

Life Context Observation

A “life event” observation captures departures from routine. It includes twelve suggested options (e.g., attended a social gathering, went on vacation, change in usual caregiver), plus the ability to add custom events.

Anecdotally, these events often cause acute increases in caregiver burden, providing context for symptom fluctuations. They can also be markers of burden themselves, as worsening symptoms often lead families to limit social activities.

Patient–Reported Outcome Measures

In addition to HROs, three standardized PROMs were included in the study to provide additional insight into the experience of caregivers:

- PROMIS Global Health-10*: overall physical, mental, and social health
- Zarit Burden Interview (ZBI)*: caregiver burden
- Food Safe Zone (FSZ) Questionnaire*: food safety strategies used by caregivers

Study start survey (collected at baseline):

Caregiver: demographics, employment status, healthcare navigation
Individual with PWS: genetic subtype, concurrent diagnoses, current treatment plan, school & living setting

Ongoing HRO & monthly survey data collection:

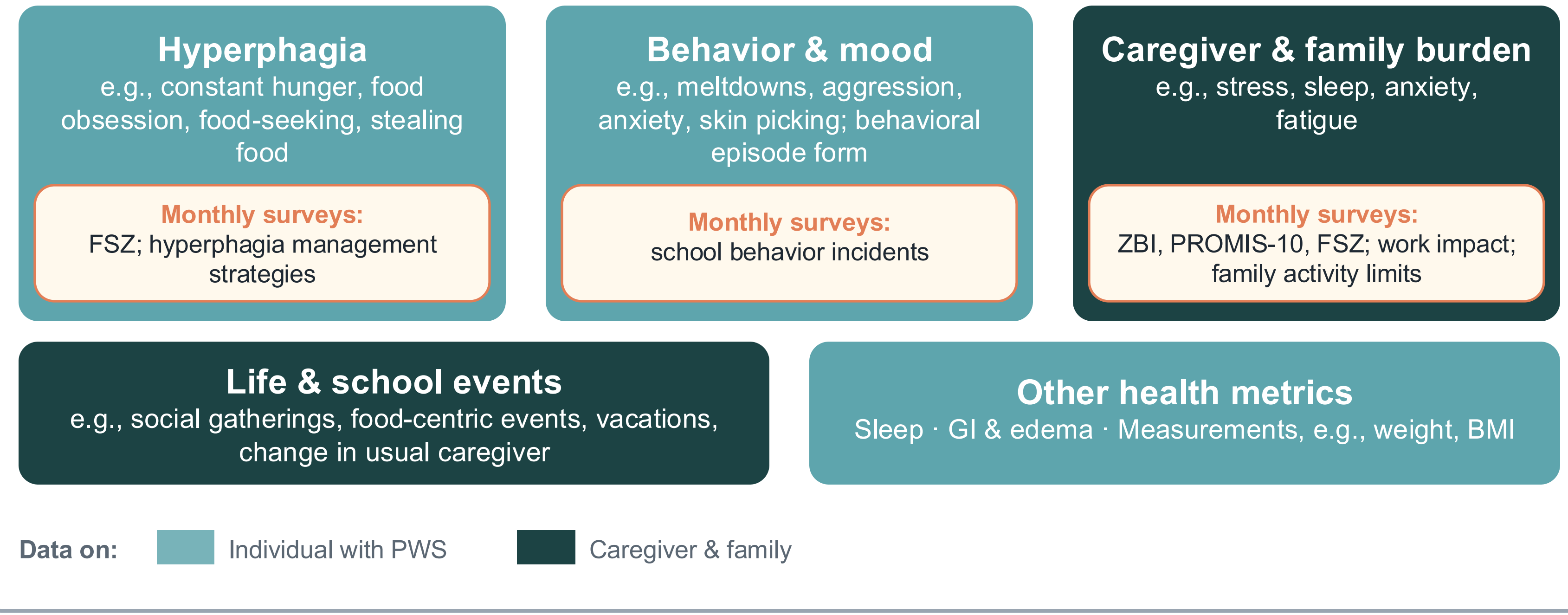


Figure 3. Elements of BRAVE-PWS data collection

NEXT STEPS

The BRAVE-PWS Caregiver Study has launched and enrollment is ongoing. Caregivers of individuals with PWS aged 4+ years, living in the U.S., are eligible to participate.

Interested caregivers can learn more at <https://www.foliahealth.com/pws-caregiver-study>

Clinicians interested in referring their patients should email research@foliahealth.com



REFERENCES

- Currie G, Estefan A, Caine V. Mothering a child with complexity and rarity: a narrative inquiry exploring Prader-Willi syndrome. *Qual Health Res.* 2024;34(8-9):742-755. doi:10.1177/10497323231225412
- Kayadjanian N, Schwartz L, Farrar E, Comtois KA, Strong TV. High levels of caregiver burden in Prader-Willi syndrome. *PLoS One.* 2018;13(3):e0194655. doi:10.1371/journal.pone.0194655
- Usubini AG, Bondesan A, Caroli D, et al. Psychological conditions of caregivers of adult subjects with Prader-Willi syndrome. *Orphanet J Rare Dis.* 2024;19:392. doi:10.1186/s13023-024-03385-6

ACKNOWLEDGMENTS

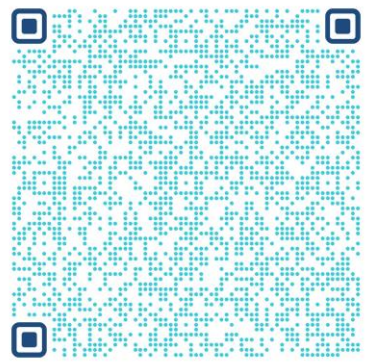
The authors would like to thank the PWS caregivers and clinicians interviewed for their time and invaluable insights. Thank you to Cortlynn Danby for support with graphic design. This study is funded by Soleno Therapeutics, a Neurocrine Biosciences company.

ABBREVIATIONS

HRO = home-reported outcome; KOL = key opinion leader; PROM = patient-reported outcome measure; PROMIS = Patient Reported Outcomes Measure Information System; ZBI = Zarit Burden Interview

CONTACT INFORMATION

For more information, contact christopher.joshi@foliahealth.com



ELECTRONIC POSTER
Copies of this poster obtained through the QR code are for personal use only.