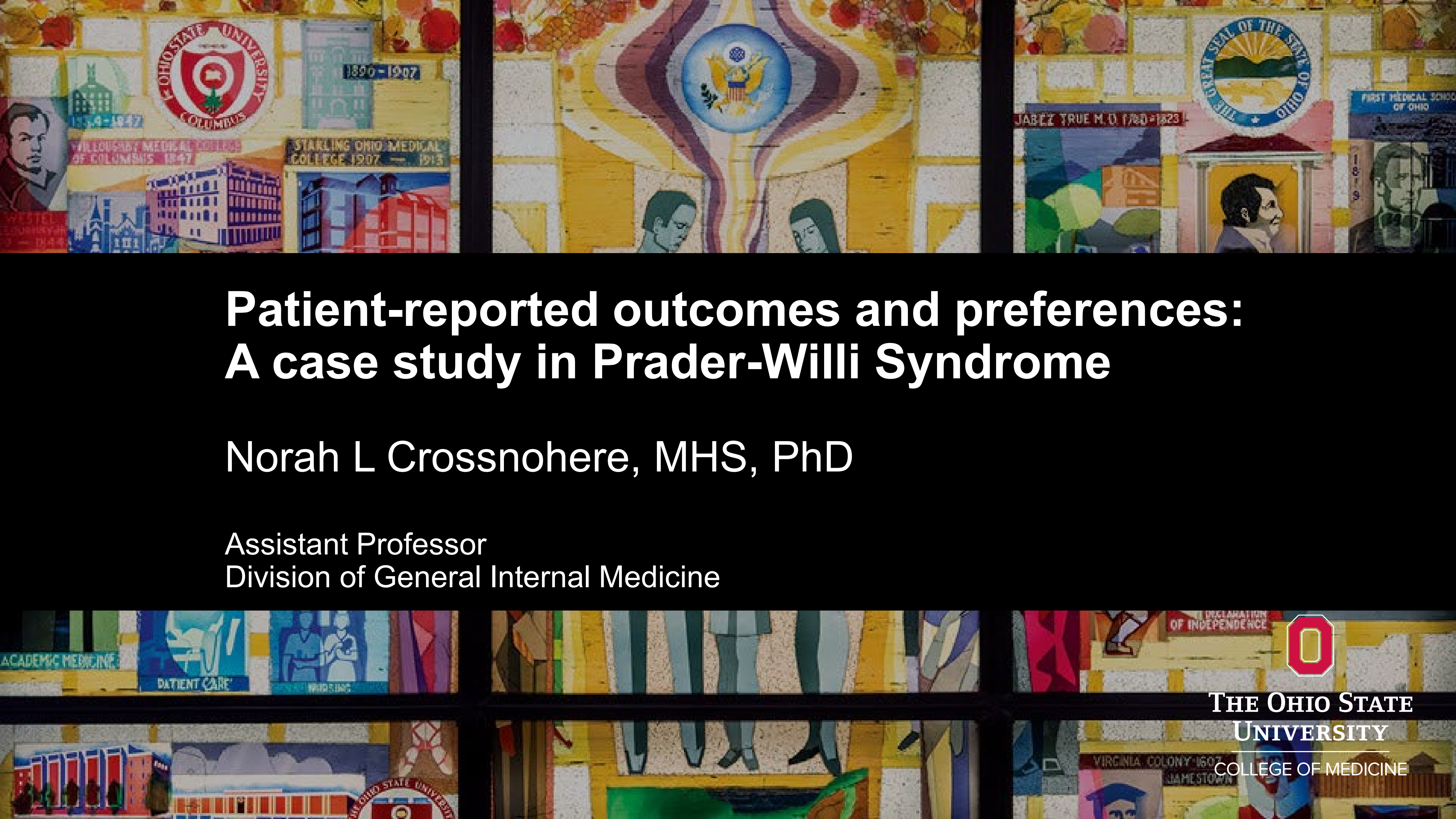

Patient-reported outcomes and preferences: A case study in Prader-Willi Syndrome

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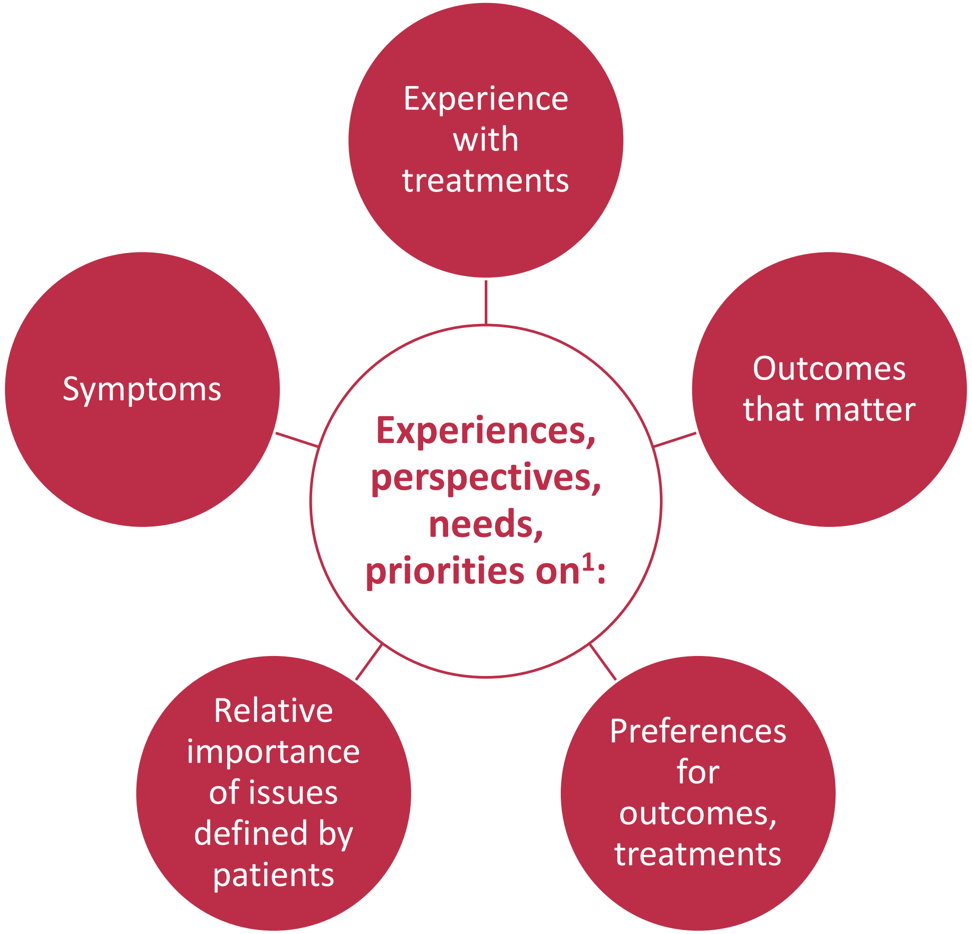
Patient experience data

Having data on patient experience information improves patient-centered outcomes in many medical contexts

- **Regulation:** patient-centered benefit-risk assessment informs how regulators consider new products[2]

- **Clinical care:** routine collection of symptoms has been demonstrated to improve quality of life[3] and extend survival[4]



Experiences, perspectives, needs, priorities on[1]:

- Experience with treatments
- Outcomes that matter
- Preferences for outcomes, treatments
- Relative importance of issues defined by patients
- Symptoms

[1]FDA 2018, [2]Ho et al. 2015, [3]Porter et al. 2021, [4]Basch et al 2017

Three concepts in patient experiences

	Description	Example collection approach	Example concepts measured
Patient-reported outcomes (PROs)	Describe how patients feel, function, live their lives[1]	Standardized, validated questionnaires	Patient status (such as in symptoms, functioning, HRQoL)
Priorities	Topics that are regarded with greater relative importance[2]	Ranking, best-worst scaling (BWS)	Relative importance (e.g., most important treatment outcomes)
Preferences	Relative desirability acceptability of specified alternatives[3]	Discrete-choice experiment (DCE), time-trade off (TTO)	Willingness to accept risk in exchange for given benefit

[1]FDA 2009, [2]Hollin et al. 2022, [3] FDA 2016

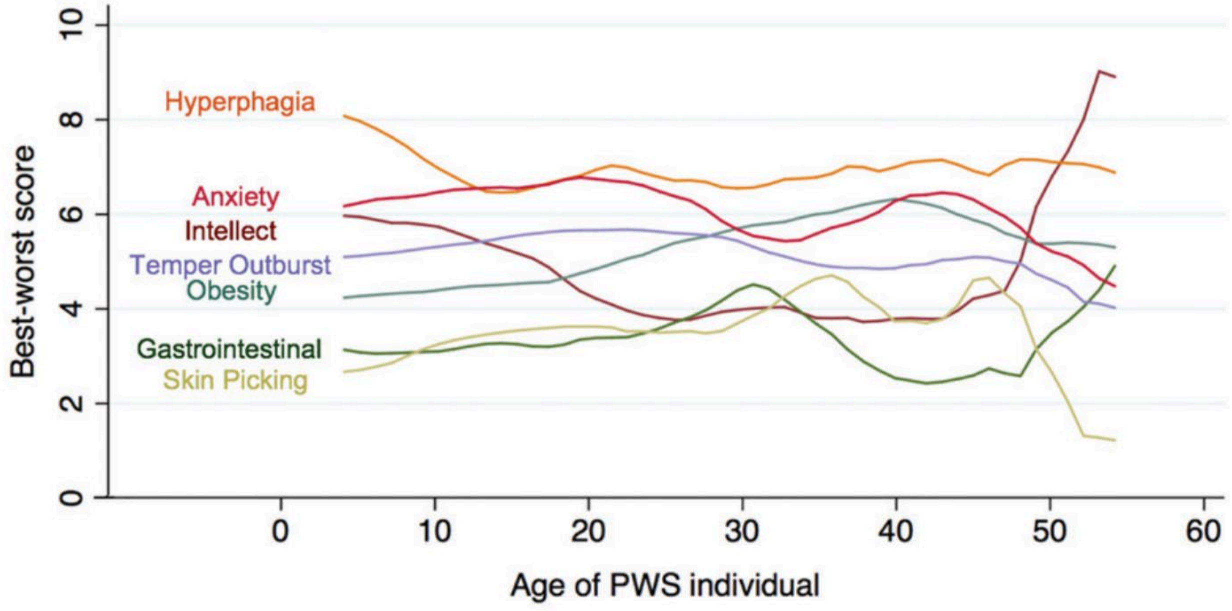
Case study: Prader-Willi syndrome (PWS)

- Rare condition characterized by hyperphagia (insatiable hunger), obesity, intellectual disability, and shortened life span[1,2]
- PWS treatments in development have modest, but potentially meaningful, impacts on hyperphagia and behavior[3]
- PWS patient groups are working to enhance patient-centeredness of regulatory review by measuring patient priorities and preferences[4-7]

[1]Butler et al 2002, [2]Angula et al 2015, [3]FPWR Blog July 2021, [4]Tsai et al. 2018, [5]Tsai et al 2021, [6]Lavelle et al 2021, [7]Patient Listening Session, CDER, US FDA, July 2021. Image courtesy of PWS Clinical Trials Consortium

Study 1: Prioritize treatment endpoints

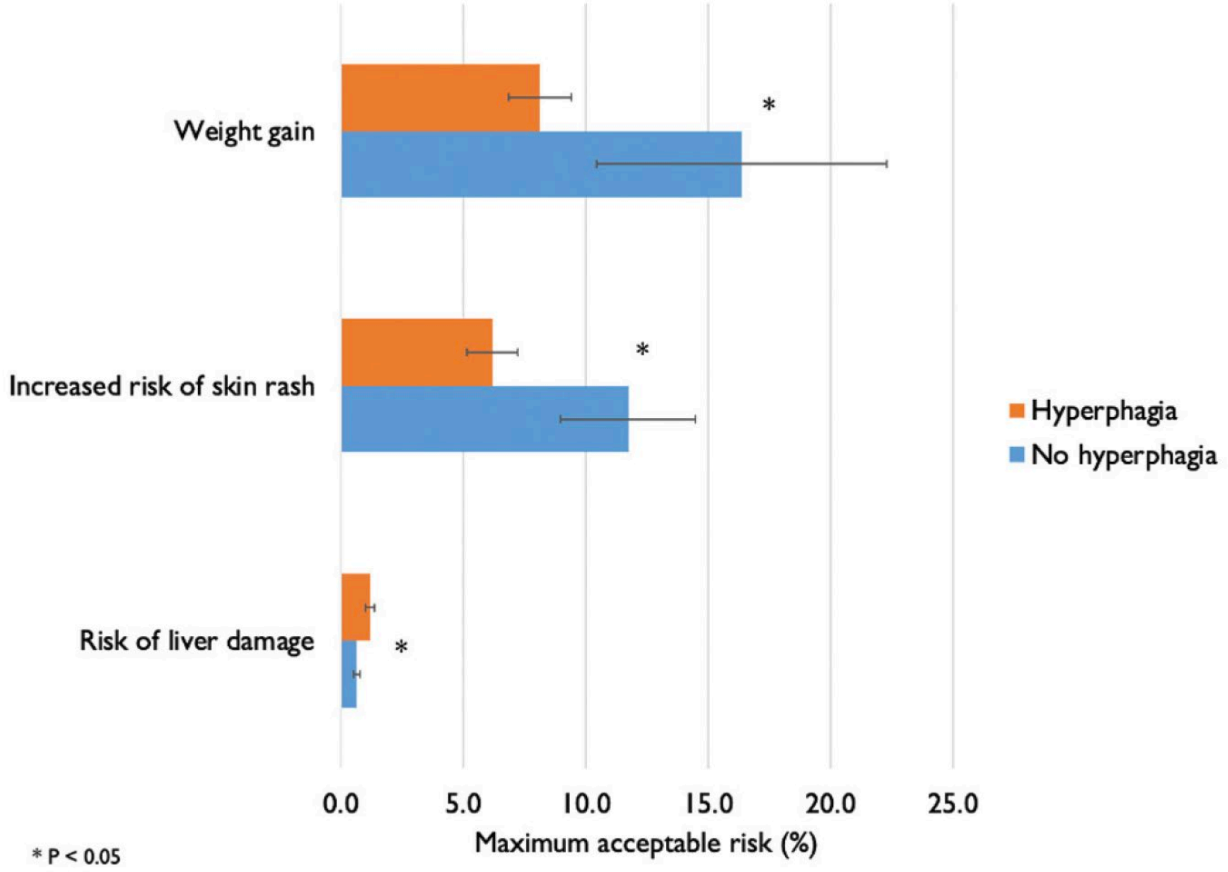
- Prioritized treatment outcomes using best-worst scaling
- Hyperphagia, not obesity, was most important endpoint to broad PWS community
- Demonstrated importance of having a hyperphagia, rather than obesity, endpoint



Tsai J, Scheimann A, McCandless S, Strong T, & Bridges J. (2018). Caregiver priorities for endpoints to evaluate treatments for Prader-Willi syndrome: a best–worst scaling. *Journal of Medical Economics*, *21*(12), 1230-1237.

Study 2: Identify acceptable benefit-risk tradeoffs

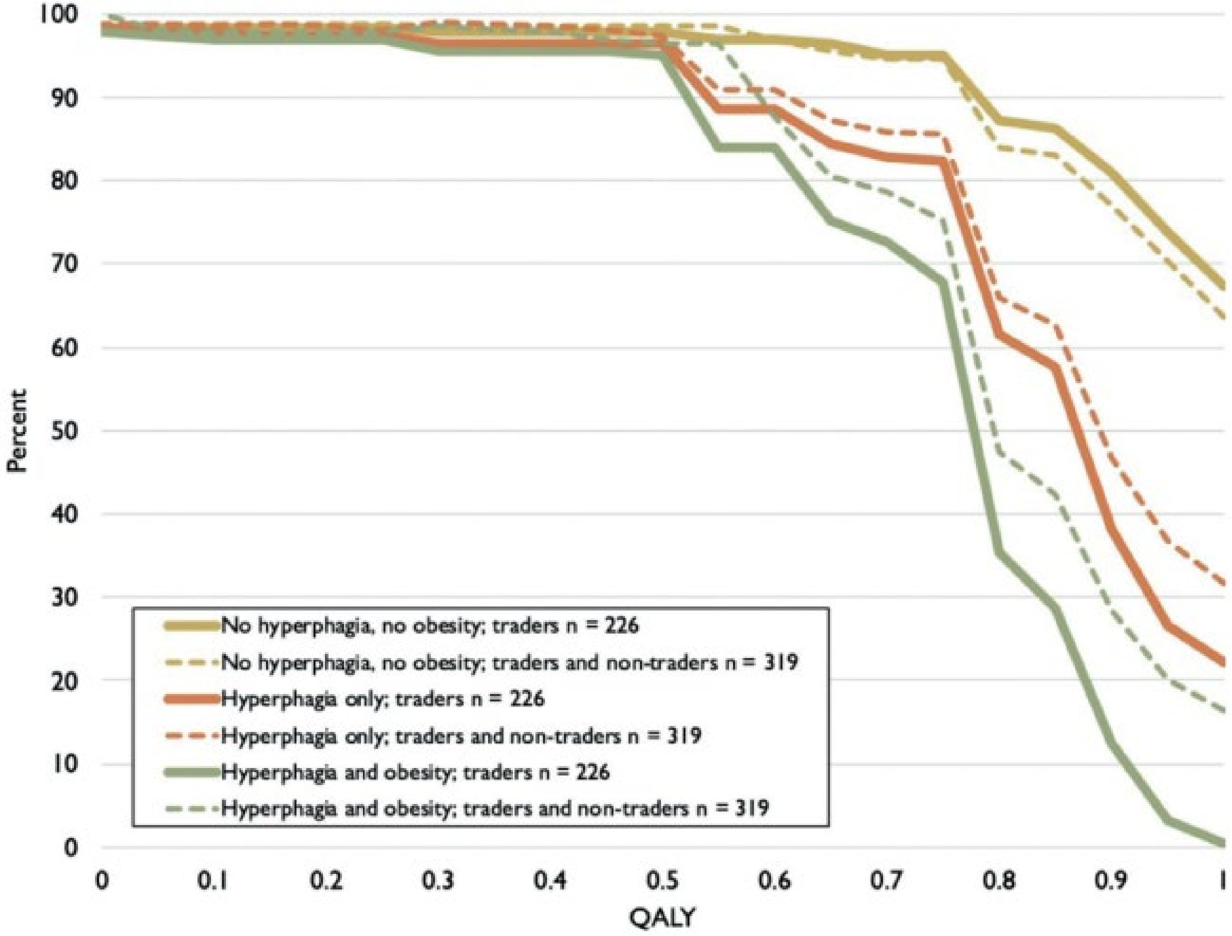
- DCE assessed risk tolerance in exchange for improvement on the HQ-CT, an ObsRO measure of hyperphagia
- Tolerated risk in exchange for improvement in hyperphagia, but how much risk varied by experience with hyperphagia
- Quantifies a “meaningful benefit” on the HQ-CT



Tsai, J, Crossnohere N, Strong T, Bridges, J. (2021). Measuring meaningful benefit-risk tradeoffs to promote patient-focused drug development in Prader-Willi syndrome: A discrete-choice experiment. *MDM Policy & Practice.* doi.10.1177/23814683211039457

Study 3: Quantify burden of meaningful symptoms

- Estimated burden of PWS health states with/without hyperphagia and obesity using TTO
- Caregivers were willing to trade years of life to avoid hyperphagia
- QALYs have emerging value for pre-market decision makers



Lavelle T, Crossnohere N, Bridges, J. (2021). Quantifying burden of hyperphagia in Prader-Willi syndrome using quality-adjusted life years. *Clinical Therapeutics. 43*(7)1164-1178.

Take aways

1. Measuring priorities and preferences of patient communities can inform patient-centered agenda setting for research and care
    - Example activity: community-based surveys including preference and priority assessment[1,2]

2. Measuring PROs at the point-of-care can improve clinical and patient-centered outcomes
    - Example activity: establish PRO systems for use in routine care[3]

[1]Brizzi et al. 2020, [2]Crossnohere et al. 2021, [3] The PROTEUS Practice Guide 2023

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