PRADER-WILLI SYNDROME
FDA PATIENT LISTENING SESSION
HIGHLIGHTS FROM JUNE 17, 2021

Prader-Willi SYNDROME ASSOCIATION | USA
SAVING AND TRANSFORMING LIVES

FOUNDATION FOR PRADER-WILLI RESEARCH
Working Toward an Independent Future

On June 17, 2021, FPWR and PWSA | USA held a **Patient Listening Session** with the FDA to share our community's experiences related to Prader-Willi syndrome (PWS). The purpose of this meeting was to promote dialogue between the FDA and members of the PWS patient community to ensure the FDA has a full understanding of the unique challenges of PWS and issues related to PWS clinical trials.

More than 60 FDA representatives attended the 1-hour meeting along with representatives from FPWR, PWSA | USA and advocates from the PWS research field:

- Dr. Theresa Strong*, Director Research Programs, FPWR, parent of an adult with PWS
- Rob Lutz*, Member Board of Directors, PWSA | USA, parent of an adult with PWS
- Elizabeth Dykens*, Professor, Vanderbilt University
- Paige Rivard, CEO, PWSA | USA, parent of a child with PWS
- John Walter, CEO, FPWR
- Susan Hedstrom, Executive Director, FPWR, parent of a child with PWS
- Lauren Schwartz-Roth, Clinical Psychologist, FPWR, parent of an adult with PWS
- Elizabeth Roof, Senior Research Specialist, Vanderbilt University
- Caroline Vrana-Diaz, FPWR, note taker

What is a Patient Listening Session?

Patient Listening Sessions allow patients, caregivers, and their advocates to share their experiences and perspectives by talking directly with FDA staff. The sessions can play an important role in drug development as they help the FDA better understand what is most important to our specific community as medical products are being developed. Listening sessions cannot be used to discuss specific medical products but are an opportunity to share disease related experiences and perspectives.

What Was Covered During the PWS Listening Session?

Throughout the PWS Listening Session, we provided insight and perspective on the unique challenges of PWS, with particular focus on issues related to clinical trials. We discussed how moderate changes in PWS-associated symptoms could lead to meaningful improvements in daily living and the ability of the person with PWS to achieve more independence. We also community's tolerance for risk as it relates to new treatments, and the impact of the COVID-19 pandemic on PWS clinical trials. We provided valuable information about the unmet medical needs of our community to the lead review division and FDA colleagues.

During the meeting, we provided information and resources on the following areas of awareness:

- Review of patient experience data from the PWS community: severity of disease, unmet medical need, treatment preferences, and risk tolerance
- Summary of the perspective of individuals with PWS

- Impact and meaningfulness of modest improvements in PWS-associated behaviors
- Impact of COVID-19 on PWS families and implications for clinical trials
- Discussion of how PWS patient experience informs clinical trial conduct and interpretation (benefit: risk profile); feasibility challenges for future PWS trials

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FDA Patient Listening Session

Highlights from June 17, 2021

About PWS

- PWS is a complex neurodevelopmental and metabolic disorder
- PWS is life threatening, even in food secure environments
- Despite earlier diagnosis and family education, the risk of death remains high
- PWS has a profound impact on functioning and quality of life, and ability to achieve independence

Impact of PWS

- PWS impacts the entire family with higher burden than, for example, caring for a person with Alzheimer disease
- The impact of PWS changes over time (e.g., 0-4 years is hypotonia and feeding difficulties, 19+ is hyperphagia and behavior)
- Our loved ones with PWS live with risk every day, and as such caregivers are willing to accept significant risk for a new treatment.
- Families are struggling to manage behavior with more than 65% of individuals age 18+ taking one or more psychiatric medications **–** these medications have never been evaluated for safety and efficacy in the PWS population.

Treatment Priorities

- Caregivers prioritize treating hyperphagia, but other behaviors like anxiety and temper outbursts are also of high priority
- People with PWS prioritize treatments for hunger, anxiety, and outbursts
- Even a modest improvement could have a meaningful impact on people with PWS and their families
- A diverse portfolio of treatments will be needed

Considerations for PWS Clinical Trials:

- What makes a meaningful trial endpoint?
- What do families consider meaningful change?
- What are the feasibility challenges of additional trials?
- How can existing/additional data (e.g., natural history studies) support PWS drug development?