

# Utility of Patient Experience Data (PED) & Patient Preference Data in Patient Focused Drug Development (PFDD)

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## SUBMISSION DETAILS

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**Methodological Issue Being Addressed** Patient experience data (PED) can be used to inform medical product development, yet it can be challenging for sponsors to determine whether, when, and how to obtain meaningful and fit-for-purpose PED and how to integrate it into product stage gates / regulatory communications. The authors provide two case studies of disorders with CNS impairment; one rare, pediatric-onset genetic condition (Angelman syndrome) and one common adult-onset degenerative disorder (Alzheimer's disease) to demonstrate ways to leverage PED.

**Introduction** In accordance with FDA patient-focused drug development (PFDD) guidance to outline collection and submission of PED, the authors highlight best practices, and benefits of PED collection at different stages of product development in two case examples.

Case study 1: The Alzheimer's Disease Patient and Caregiver Engagement (AD PACE) Initiative leveraged a public/private consortium to conduct research to understand What Matters Most (WMM) to at-risk and diagnosed individuals as well as their caregivers to inform meaningful treatment goals.

Case study 2: Angelman syndrome is a rare genetic disorder causing intellectual disability, seizures, and developmental delays. Gene-targeted products are in development, yet patient-focused product development requires data about meaningful change, product acceptability, and tolerance for risk and burden.

**Methods** Using two case studies, the presenters will describe the use of qualitative and quantitative approaches to obtain data that informs pre-clinical decision making, clinical trial design, regulatory engagement, product launch, and access/reimbursement.

**Results** Case 1. Alzheimer's disease qualitative data supported a revised conceptual disease model. A survey (n=640, diverse individuals) tested the hypothesis of differences in priorities by disease stage, respondent type, and ethnicity, using Best-Worst Scaling (BWS). These results show a clear prioritization by cohort across the disease spectrum and by race/ethnicity. Patients and caregivers prioritized (different) concepts as most important and descriptive analysis highlighted differences by race. Although some gold standard measures map to some important domains, there are gaps in measurement of overall priorities that can be addressed with new measures. Additionally, results can guide clinical care regarding impact of disease and support determination

of meaningful benefit of treatments for patients, regardless of race/ethnicity.

Case 2. For Angelman syndrome, an exploratory survey assessed symptom impact, meaningful change, willingness to participate, and tolerance of risk and burden (AS). Among 342 caregiver respondents, symptoms that impacted patient communication, daily living skills, cognition, and gross motor were most impactful. Verbal communication was a treatment priority for 69%. Most (72%) reported willingness (“definite” or “probable”) to enroll the patient in a non-curative clinical trial with repeated lumbar puncture and risk of serious adverse events including spinal injury. Results support the meaningfulness of non-curative therapy development and patient-centeredness of existing outcome measures. Strong willingness to participate is informative to sponsor planning. These data also supported an externally-led PFDD meeting with the FDA.

Presenters will conclude with a reflection on lessons learned for meaningful PFDD.

**Conclusion** PFDD can be actualized through integration of PED throughout product development. Our case studies reflect a range of approaches used to collect and report impactful patient data and lessons learned.

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### Keywords

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**Guidelines** I have read and understand the Poster Guidelines

**Disclosures** Theresa Frangiosa, Holly Peay and Ilse Peterson are all consultants working for Faegre Drinker Consulting

Theresa Frangiosa has held positions in the pharmaceutical industry from 1988-2013