Patient Focused Drug Development (PFDD): The Utility of Patient Experience and Preference Data Across the Product Development Lifecycle

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Methodological Issue Being Addressed

Patient experience data (PED) define patient perspectives, experiences, and needs. Patient preference data are a subset of PED and include treatment priorities and preferences. These data are increasingly used to inform the medical product lifecycle and are critical to patient-focused drug development (PFDD).

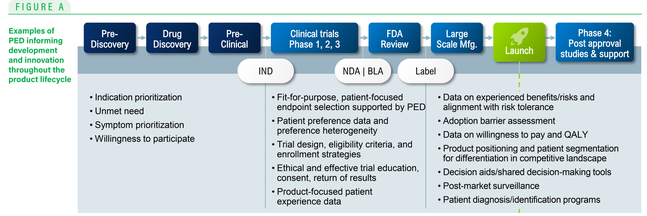
The FDA and other regulatory agencies promote the use of PED. Yet it can be challenging to determine whether, when, and how to obtain meaningful and fit-for-purpose PED, what type of PED will be most informative, and how to integrate PED into product stage gates and regulatory communications.

Introduction

PFDD can be actualized through integration of PED throughout product development. Figure A illustrates the use of PED throughout the product lifecycle.

To demonstrate PED methodology and outputs, we provide two case studies of disorders with CNS impairment and strong new medical product pipelines; one rare, pediatric-onset genetic condition (Angelman syndrome) and one common adult-onset degenerative disorder (Alzheimer's disease).

FIGURE A



Methods

Methods to generate PED should align with research objectives and questions/aims. Factors including condition and product characteristics, trial design, market dynamics, and resource constraints influence fit-for-purpose objectives and method selection. Methods range from qualitative for exploratory data collection, to mixed-methods qualitative and quantitative assessments, to complex health economic modeling.

Facilitators include FDA PFDD guidance documents, other methodological guidance and best practice resources, increased dissemination through professional forums, and growing expertise within the research and advocacy community.

Barriers include availability of experts who can conduct regulatory-grade PED research, methods that are often complex and resource-intensive (e.g., time, cost, sample size, participant burden), and other complexities described in the cases below.

Using two case studies, we describe qualitative and quantitative approaches to obtain data with implications across the product lifecycle.

Conclusions

PFDD is encouraged by regulators and valued by patients. Our case studies reflect a range of approaches used to collect and report impactful patient data that follow FDA and other methodologic guidance. The examples illustrate common challenges in PED, including complexities of heterogenous and/or progressive conditions, rare disorders, and achieving representativeness in the study cohort. Further, for AD, the study successfully addressed an important regulatory question regarding the ability to self-report in early disease. For AS, the study nuances caregiver reporting when the patient cannot self-report through careful differentiation of experienced caregiver impacts and anticipated patient impacts.

These studies are informing sponsors' efforts to define meaningful benefit, select and justify patient-centeredness of COAs, and estimate willingness to use a new product. In addition, study outputs are used to inform priority setting, regulatory engagement and decision making, patient care, and justify drug value proposition with payers.

CASE 1 RESULTS

The Alzheimer's Disease Patient and Caregiver Engagement (AD PACE) Initiative , a public/private consortium

Objective

Identify and measure treatment-related needs, preferences, and priorities of people living with or at risk of AD and their care partners. Results inform an updated disease conceptual model and examination of clinical outcome assessment measures.

Methods

The What Matters Most research consisted of two rounds of mixed methods research (Figure 1). The second round is reported here.
Round 2: Patient and caregiver focus groups (n=64) followed by survey (n=640) employing Best-Worst Scaling object-case (a health preference prioritization method) to assess priorities by disease stage, respondent type, and ethnicity.

Areas of complexity in the AD research

1. To inform priorities for AD, PED should include perspectives of at-risk and diagnosed individuals (including individuals with cognitive impairment), and caregivers.
2. As a progressive disorder where symptom impacts evolve, PED is needed over the AD disease course.
3. As a common disorder, it is critical to obtain data that is as representative of AD patients as possible.

Selected results

In the qualitative research (n=64), 50 concepts across 6 domains were endorsed as important and the AD conceptual model was refined (Figure 2).

The large observational survey (n=640) quantified prioritization and impact of disease concepts. At-risk and diagnosed individuals with MCI/in earlier clinical stages of disease were able to engage in the prioritization activities. Patients and caregivers prioritized (different) concepts as most important. Descriptive analysis highlighted differences by race.

The consortium then mapped frequently used clinical outcome assessments to priority traits (Figure 3). Although the COAs map to important domains, identified gaps can be addressed with adapted or new measures. Additionally, results guide clinical care and support determination of meaningful benefits.

FIGURE 1

AD What Matters Most™ (WMM) Research Program spanned multiple rounds, years, and cohort diversity

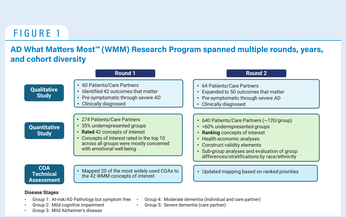


FIGURE 2

Round 2 Disease Conceptual Model for AD

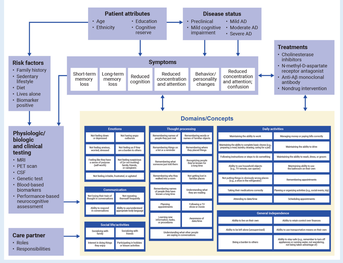


FIGURE 3

Mapping Patient and Caregiver Priorities to Legacy Measures for AD

		CDR/ CDR-SB	ADCS-ADL-MCI	FAQ	ADCOMS	iADRS	PACC	NPI/ NPI-Q	QoL-AD
Emotions (7 concepts)	Direct	0	0	0	0	0	0	5	2
	Indirect	1	0	0	1	0	0	1	1
Communication (4 concepts)	Direct	0	0	0	0	1	0	0	0
	Indirect	0	1	1	0	0	1	0	0
Social Life/Activities (4 concepts)	Direct	4	1	1	4	1	0	4	4
	Indirect	0	0	0	0	0	0	0	0
Thought Processing (15 concepts)	Direct	2	4	3	3	8	7	0	0
	Indirect	8	2	2	7	4	3	0	9
Daily Activities (14 concepts)	Direct	7	9	5	8	4	1	0	1
	Indirect	3	0	2	3	3	0	2	3
General Independence (6 concepts)	Direct	0	2	2	0	2	0	1	0
	Indirect	3	0	1	3	0	0	1	0

CASE 2 RESULTS

Angelman Syndrome (AS) Hope in Action Survey

Objective

Assess symptom impact, treatment priorities, meaningful change, willingness to participate, and tolerance of risk and burden. The results inform product development and supported an Externally Led PFDD meeting.

Methods

An online survey with closed- and open-ended questions was developed by a transdisciplinary team based on literature and advocate, parent, clinician, sponsor, and regulator input. Willingness to enroll was assessed using a hypothetical clinical trial vignette that described three benefit levels (referencing each respondents' selected treatment priorities) with set burden (i.e., one year trial, lumbar puncture) and SAE (i.e., pain, bleeding, infection, nerve injury) profiles.

Areas of complexity in the AS research

1. AS is a rare disorder, which impacts feasible methods, power, and ability to attain representativeness in sociodemographic variables.
2. AS is a multi-system and heterogeneous condition causing intellectual disability, seizures, and developmental delays. This heterogeneity complicates survey length and complexity.
3. Cognitive impacts prevent self-reporting. Priorities of caregivers and patients may be different. To help offset this challenge, investigators included question blocks about perceived impact on the person with AS and experienced impact on the caregiver.
4. Preferences about willingness to participate require a vignette to describe a future trial. Results must be interpreted in context of the trial scenario.

Selected results

Among 342 caregiver respondents, the top 10 treatment priorities are shown in Figure 4.

Communication impacts were most prioritized as treatment targets. When participants described small but meaningful changes (through open-ended questions), commonly-reported communication benefits (Figure 5) centered on basic skills (ability to answer yes or no; communicate with adaptive equipment) associated with improved health, caregiver responsiveness, and quality of life.

FIGURE 4

Symptoms selected most frequently by Angelman syndrome caregivers as a "top 5" most important to improve

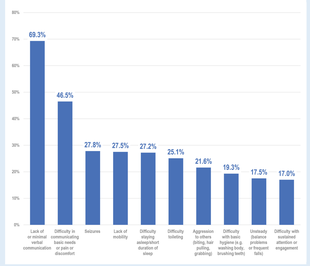
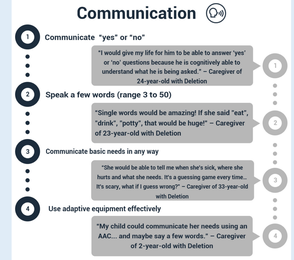


FIGURE 5

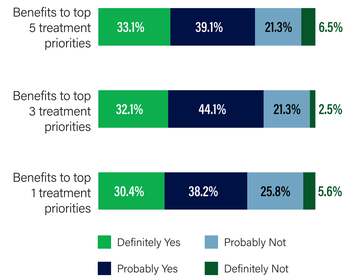
Commonly-Reported Small but Meaningful Changes in Communication for AS



Willingness to participate

Most caregivers reported willingness (probably or definitely yes) to enroll the patient in a hypothetical clinical trial at all three benefit levels.

FIGURE 6



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