Leveraging Externally Led Patient-Focused Drug Development (PFDD) Meeting and Survey Data to Enable Patient-Focused Clinical Trial Design in Schizophrenia

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SUBMISSION DETAILS

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What is the Methodological Issue Being Addressed? This poster applies FDA Patient-Focused Drug Development (PFDD) best practices, demonstrating how survey data from an Externally-Led PFDD (EL-PFDD) meeting that characterizes disease impacts and identifies priority outcomes for drug development in schizophrenia can be applied to support patient-focused clinical trial design.

Introduction PFDD is a systematic process for incorporating what matters to patients into drug development and evaluation. In 2022, the Schizophrenia & Psychosis Action Alliance and several partners convened the schizophrenia EL-PFDD meeting. A pre-meeting survey1,2 of diagnosed individuals and caregivers gathered insight into: (1) symptom burden/severity, (2) functional impacts, (3) reasons for treatment discontinuation, and (4) desired outcomes of future treatments. This abstract reports key survey findings and describes how, with the FDA's PFDD guidance documents3, these findings can support patient-focused clinical trial development.

Methods 282 survey responses from 96 individuals with schizophrenia and 186 caregivers were reflected in the peer-reviewed survey publication, which is the primary focus of this poster. Survey data was descriptively analyzed and to identify burdensome symptoms and functional impacts as well as reasons for treatment discontinuation and desired treatment outcomes. This data was placed in the context of the FDA's Roadmap to PFDD in Clinical Trials, which provides a practical algorithm for applying patient and caregiver perspectives to outcome measurement and, in turn, to support patient-focused clinical trials.

Results People with schizophrenia and caregivers who responded to the meeting survey reported significant impacts on daily living covering work and education, relationships, responsibilities at home, and finances. Caregivers consistently rated impacts as more significant than people with schizophrenia. Weight gain was most common reason for discontinuing medications reported by people with schizophrenia; for caregivers, it was "feeling flat." Preventing delusions, preventing hallucinations, and enjoying daily activities received the highest average ratings as desired outcomes of future treatments from people with schizophrenia, whereas preventing delusions and making it easier to maintain relationships had the highest average ratings from caregivers.
In accordance with FDA's Roadmap, this data enhances understanding of schizophrenia impacts and meaningful aspect(s) of health that patients and caregivers want to see improved by medical products, which can support patient-focused clinical outcome measure selection and development for patient-focused clinical trials.

Conclusion FDA's PFDD Guidance define best practices for researching what matters to people with lived experience of disease and translating this into product development and evaluation. This case study illustrates how exploratory survey research conducted in conjunction with a PFDD meeting can support PFDD.

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Keywords

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PFDD
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Guidelines I have read and understand the Poster Guidelines

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