

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

Authors: Frangiosa, T<sup>1</sup> | Peterson, I<sup>1</sup> | Chirayil, Priya<sup>1</sup> | Lavigne, G<sup>2</sup>  
Faegre Drinker Consulting<sup>1</sup> | Schizophrenia & Psychosis Action Alliance<sup>2</sup>



## 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.

### RESPONDENT CHARACTERISTICS

A total of N = 282 people with schizophrenia were represented in the survey, 96 by self-completed surveys and 186 via caregiver-completed surveys: their characteristics are presented in Tables 1-3.

| TABLE 1<br>Demographic characteristics of people with schizophrenia represented in survey sample (N = 282) |                       |                             |
|------------------------------------------------------------------------------------------------------------|-----------------------|-----------------------------|
| Demographic Characteristics                                                                                | Self-Completed (n=96) | Caregiver-Completed (n=186) |
| Mean Age (Range)                                                                                           | 56 (21, 80)           | 34 (18, 81)                 |
| Gender                                                                                                     |                       |                             |
| Male                                                                                                       | 45 (47%)              | 133 (72%)                   |
| Female                                                                                                     | 44 (46%)              | 36 (19%)                    |
| Another identity                                                                                           | 5 (5%)                | 3 (2%)                      |
| Prefer not to answer or unknown                                                                            | 2 (2%)                | 14 (8%)                     |
| Race                                                                                                       |                       |                             |
| American Indian or Alaska Native                                                                           | 1 (1%)                | 1 (1%)                      |
| Asian                                                                                                      | 8 (8%)                | 3 (2%)                      |
| Black or African American                                                                                  | 3 (3%)                | 5 (3%)                      |
| White                                                                                                      | 71 (74%)              | 150 (81%)                   |
| Another Identity (includes Multiple Races)                                                                 | 11 (12%)              | 14 (8%)                     |
| Unknown                                                                                                    | 2 (2%)                | 13 (7%)                     |
| Ethnicity                                                                                                  |                       |                             |
| Hispanic, Latino, or of Spanish Origin                                                                     | 7 (8%)                | 16 (9%)                     |

| TABLE 2<br>Employment/educational status and current living situation of people with schizophrenia represented in survey sample (N = 282) |                       |                             |
|-------------------------------------------------------------------------------------------------------------------------------------------|-----------------------|-----------------------------|
| Demographic Characteristics                                                                                                               | Self-Completed (n=96) | Caregiver-Completed (n=186) |
| Employment Status                                                                                                                         |                       |                             |
| Full-time                                                                                                                                 | 21 (22%)              | 9 (5%)                      |
| Part-time                                                                                                                                 | 19 (20%)              | 21 (11%)                    |
| Actively looking                                                                                                                          | 7 (7%)                | 2 (1%)                      |
| Not employed                                                                                                                              | 32 (33%)              | 151 (81%)                   |
| Retired                                                                                                                                   | 13 (14%)              | 1 (1%)                      |
| Prefer not to say                                                                                                                         | 4 (4%)                | 2 (1%)                      |
| Educational Status                                                                                                                        |                       |                             |
| Yes, full-time                                                                                                                            | 3 (3%)                | 8 (4%)                      |
| Yes, part-time                                                                                                                            | 11 (12%)              | 13 (7%)                     |
| No                                                                                                                                        | 82 (85%)              | 164 (88%)                   |
| Don't know                                                                                                                                | 0 (0%)                | 1 (1%)                      |
| Current Living Situation                                                                                                                  |                       |                             |
| Living independently, alone                                                                                                               | 30 (31%)              | 18 (10%)                    |
| A household with relatives or friends                                                                                                     | 56 (58%)              | 125 (67%)                   |
| Supervised group housing                                                                                                                  | 3 (3%)                | 14 (8%)                     |
| Partially supervised group housing                                                                                                        | 0 (0%)                | 6 (3%)                      |
| Residential substance use program                                                                                                         | 1 (1%)                | 1 (1%)                      |
| Supportive housing                                                                                                                        | 4 (4%)                | 7 (4%)                      |
| Long-term care facility                                                                                                                   | 0 (0%)                | 3 (2%)                      |
| Correctional facility                                                                                                                     | 0 (0%)                | 2 (1%)                      |
| Shelter                                                                                                                                   | 0 (0%)                | 1 (1%)                      |
| Currently without housing                                                                                                                 | 0 (0%)                | 5 (3%)                      |
| Unknown                                                                                                                                   | 2 (2%)                | 4 (2%)                      |

| TABLE 3<br>Insurance coverage of people with schizophrenia represented in survey sample (N = 282) |                       |                             |
|---------------------------------------------------------------------------------------------------|-----------------------|-----------------------------|
| Demographic Characteristics                                                                       | Self-Completed (n=96) | Caregiver-Completed (n=186) |
| Insurance Coverage                                                                                |                       |                             |
| Employer-sponsored health insurance                                                               | 25 (26%)              | 40 (22%)                    |
| Commercial health insurance obtained through the marketplace                                      | 9 (9%)                | 11 (6%)                     |
| Medicare                                                                                          | 44 (46%)              | 68 (37%)                    |
| Medicaid                                                                                          | 31 (32%)              | 92 (50%)                    |
| Short-term disability insurance                                                                   | 3 (3%)                | 0 (0%)                      |
| Long-term disability insurance                                                                    | 4 (4%)                | 7 (4%)                      |
| Other, please specify:                                                                            | 9 (9%)                | 24 (13%)                    |
| None                                                                                              | 3 (3%)                | 7 (4%)                      |
| Prefer not to answer or unknown                                                                   | 6 (6%)                | 14 (8%)                     |



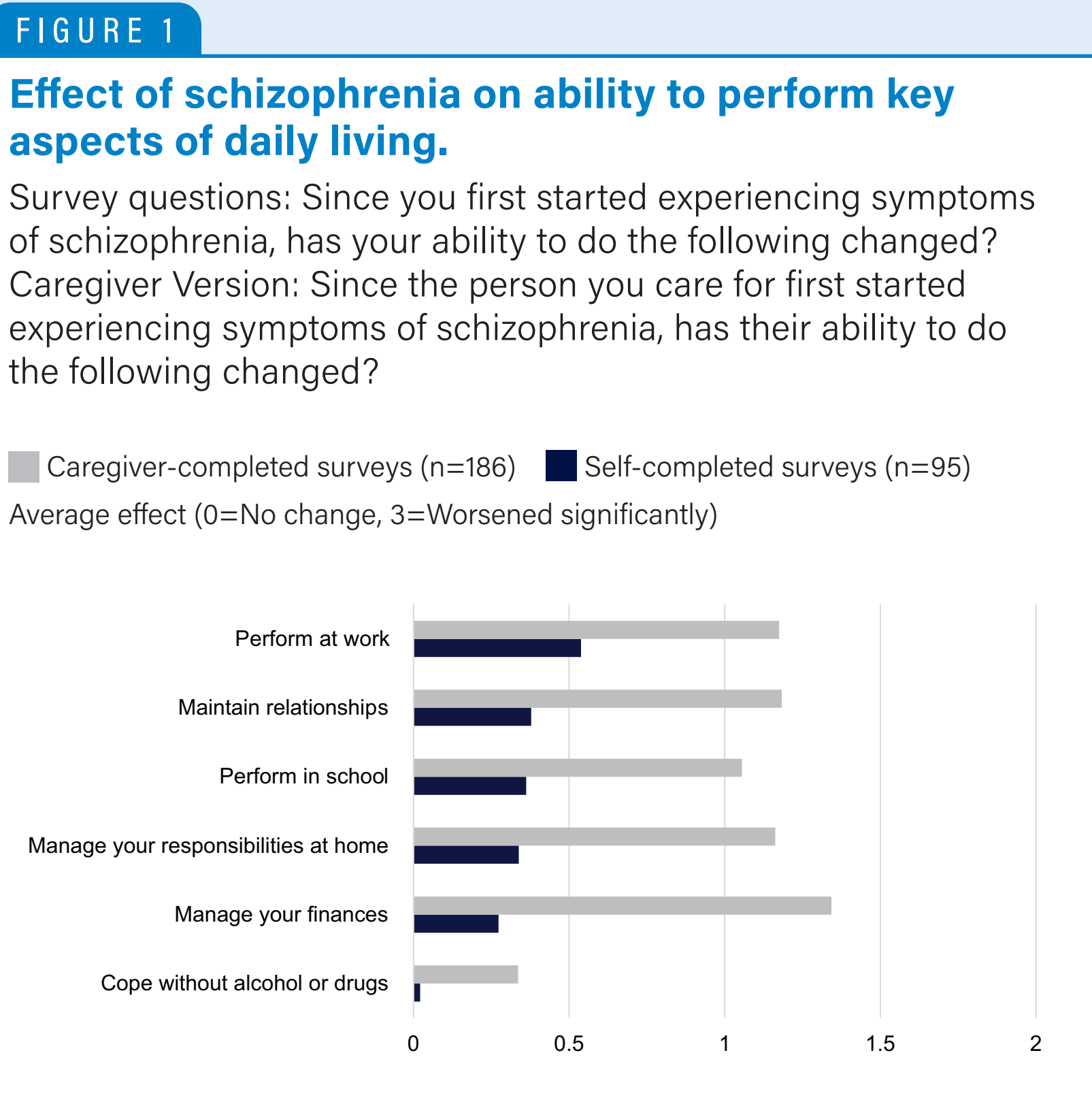
## Introduction

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 survey<sup>1</sup> 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 documents<sup>2</sup>, these findings can support patient-focused clinical trial development.

### FINDINGS

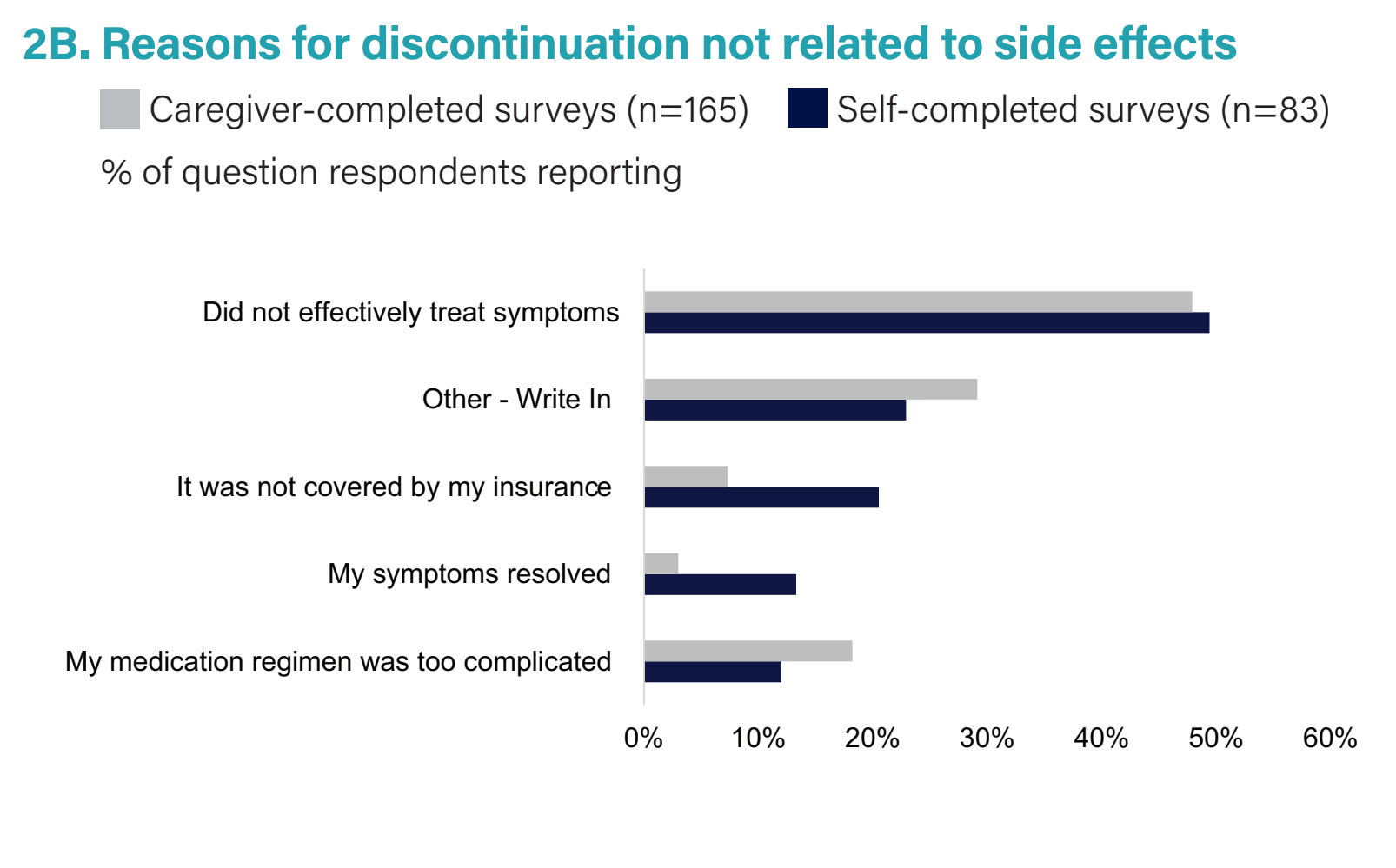
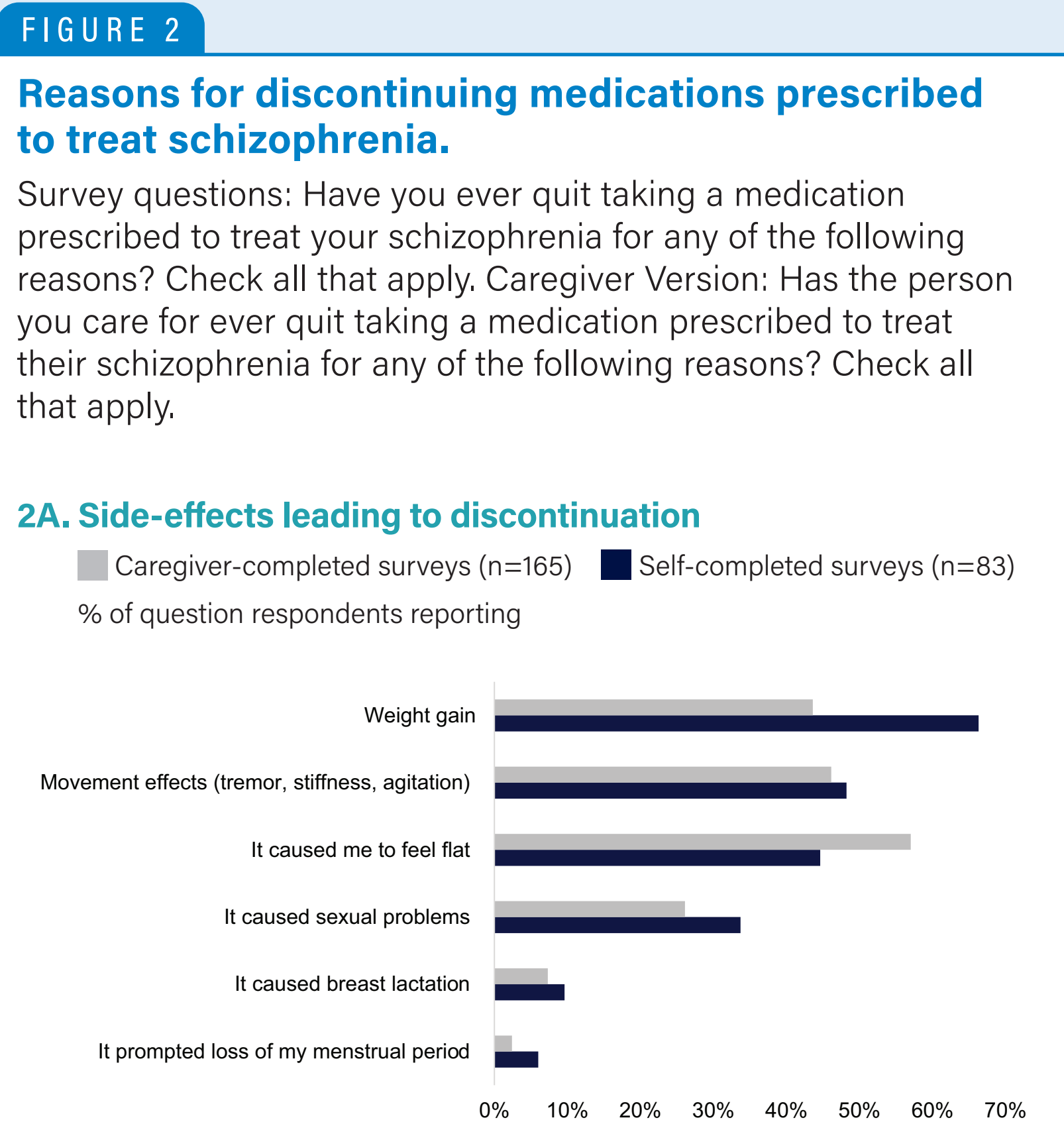
#### Impacts of Schizophrenia on Daily Life

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. (Figure 1) Caregivers consistently rated impacts as more significant than people with schizophrenia. Further, caregivers more frequently reported challenges associated with drugs and alcohol.



#### Medication Discontinuation

Weight gain was the most common side effect leading to medication discontinuation for people with schizophrenia (66.3%); other frequently identified side effects included movement effects (48.2%), “feeling flat” (44.6%), and sexual problems (33.7%). For caregivers, the most common side effect leading to medication discontinuation was “feeling flat” (57.0%); other frequent issues were movement effects (46.1%), weight gain (43.4%), and sexual problems (26.1%). Additional data is shown in Figure 2.

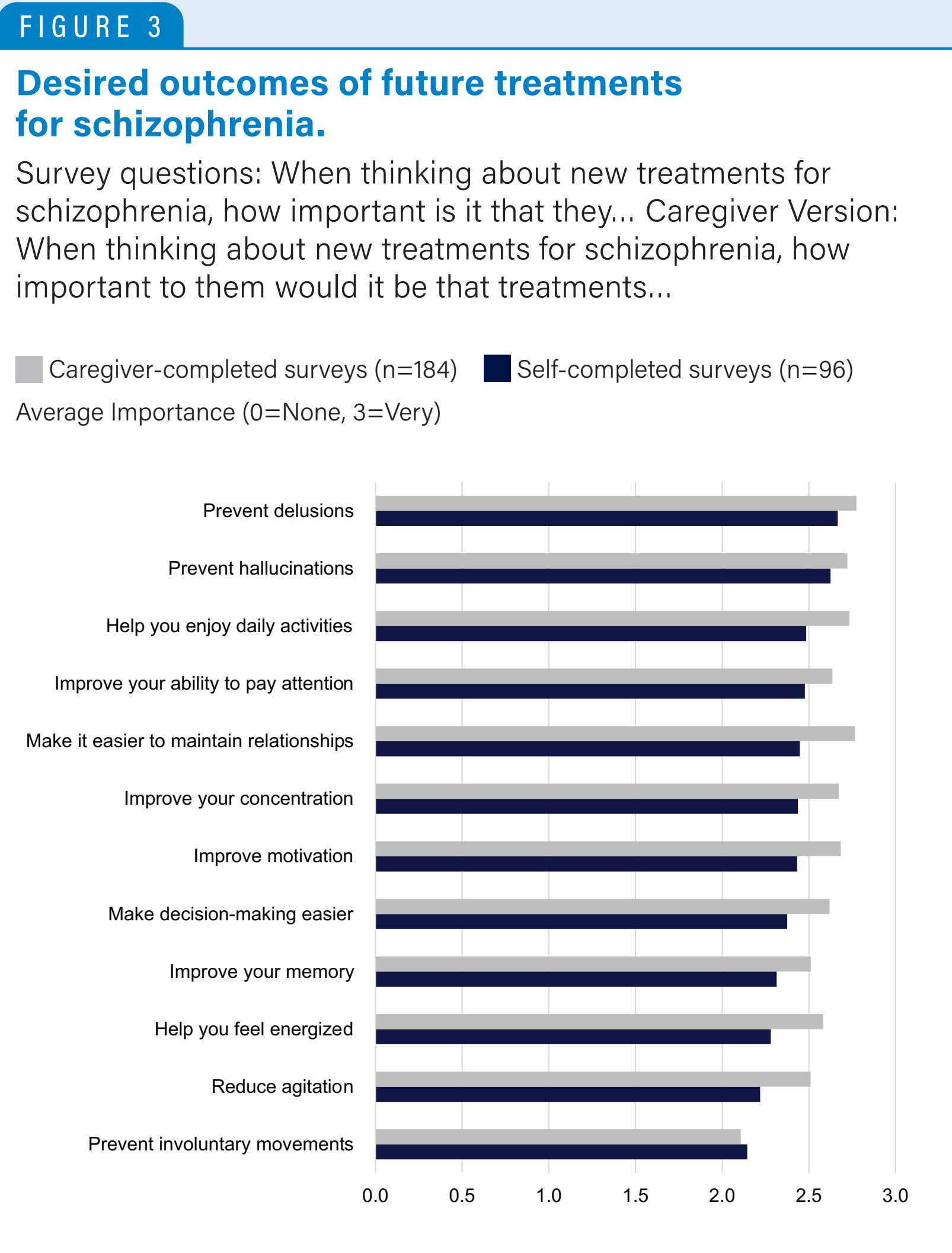


## Methods

Individuals eligible to complete the survey included adults with a diagnosis of schizophrenia diagnosed at least six months earlier and caregivers of adults with schizophrenia who could not complete the survey. Respondents must have lived in the United States and have reached the age of majority in their state or territory. Participants were recruited via outreach by Schizophrenia & Psychosis Action Alliance, American Foundation for Suicide Prevention, Mental Health America, National Alliance for Mental Illness and the National Council for Mental Wellbeing.

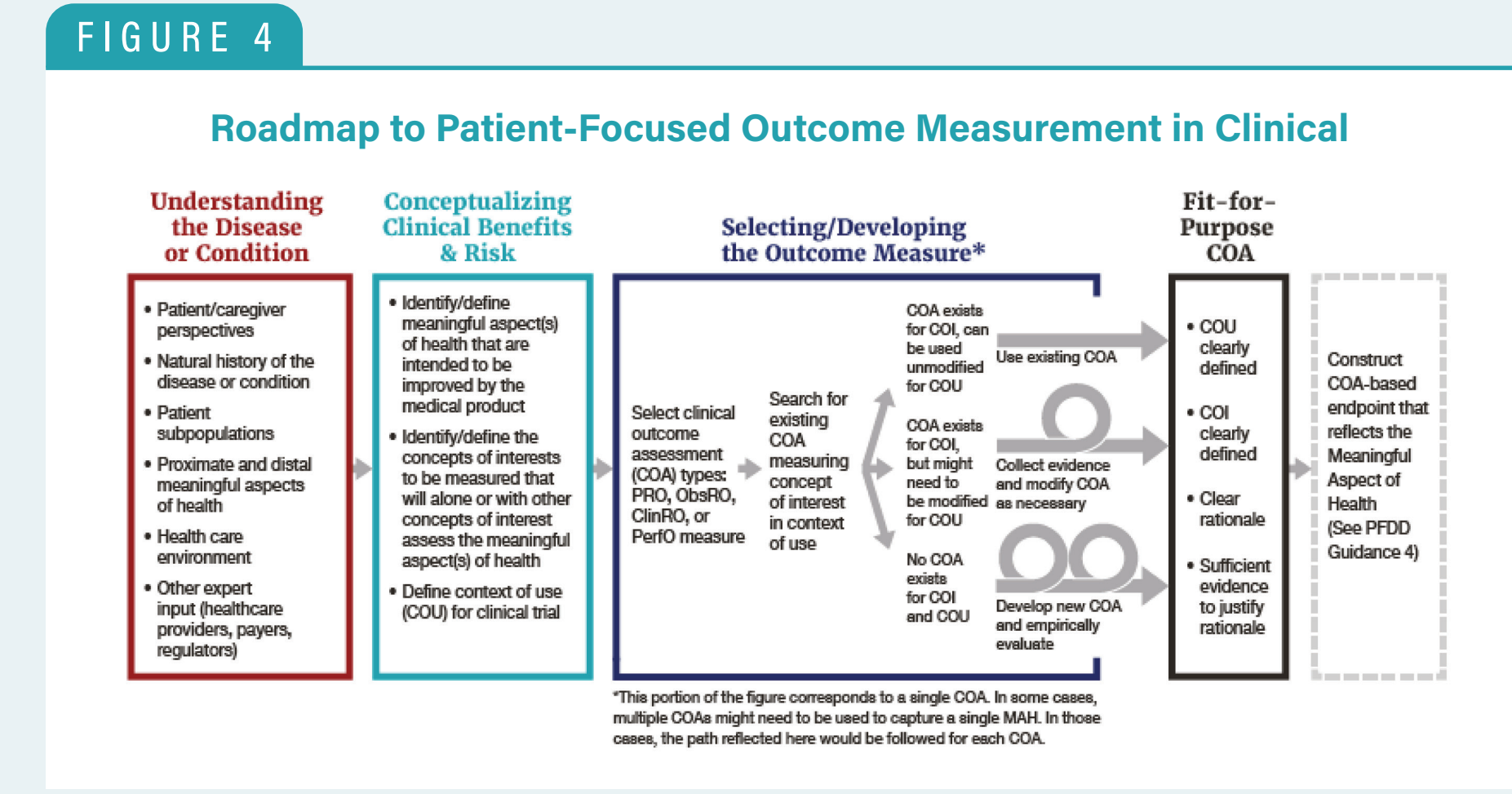
#### What Matters Most from New Treatments

There were a multitude of treatment needs communicated, including positive symptoms (i.e. preventing delusions and hallucinations), negative symptoms (i.e. improve motivation), cognition (i.e. improve concentration) and quality of life (i.e. enjoyment of daily activities). Both diagnosed individuals and caregivers assigned a high degree of importance to several specific traits (Figure 3).



## FDA's Roadmap to Patient-Focused Outcome Measurement in Clinical Trials

FDA's Roadmap to PFDD in Clinical Trials (Figure 4) identifies how patient and caregiver perspectives – such as those collected in this PFDD meeting survey – can inform outcome measurement and, in turn, support patient-focused clinical trials. This data specifically can support conceptualization of clinical benefits by enhancing understanding of schizophrenia impacts and meaningful aspect(s) of health that patients and caregivers want to see improved by medical products. This, in turn, can support patient-focused clinical outcome measure selection and development for patient-focused clinical trials. Interestingly, two products for schizophrenia were approved by FDA after this meeting was held. While neither manufacturer acknowledged submission of this data in their NDA, FDA's Integrated Review for Cobenfy indicates that the meeting report was considered in their review.



## CONCLUSION

Leveraging FDA's PFDD Guidance, PFDD meetings, and qualitative and quantitative research enable people with lived experience and the advocacy organizations that serve them to move from gathering anecdotal experience to quantifiable data that supports patient-focused product development. These survey findings provide context for drug developers, regulatory agencies, clinicians, and health plans who are interested in understanding what matters most to people with schizophrenia and their caregivers.

References  
1. Peterson IS, Chitale R, Frangiosa T, Krasa HB, VonEdWaerd-Benjamin R, Fisher A, et al. Schizophrenia Impacts and Treatment Priorities: Results of An Externally-Led Patient-Focused Drug Development Meeting Survey. Ther Innov Regul Sci [Internet]. 2026 [cited 2026 July 23]; https://doi.org/10.1007/s43441-026-01001-2  
2. US Food and Drug Administration. Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments [Internet]. 2025 Oct. https://www.fda.gov/media/159500/download. Accessed 2 Feb 2026

**Acknowledgement:**  
The authors wish to thank those whose dedicated efforts have advanced research designed to reflect and elevate the patient voice, including those with a diagnosis of schizophrenia and their caregivers.

**Contact Us**  
  
**Terry Frangiosa**  
Principal  
+1 202 230 5231  
theresa.frangiosa@faegredrinker.com



**Schizophrenia  
& Psychosis  
Action Alliance**

