Quantiﬁng What Matters Most to Patients and Care Partners in Alzheimer’s Disease

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Background & Objectives

Relevance from the US Food and Drug Administration (FDA). Increased attention to what matters most to patients and their care partners is being driven by a variety of factors, including increased awareness of the importance of patient-centered outcomes and the FDA’s focus on patient-reported outcomes (PROs) in drug development. FDA and sponsors are increasingly recognizing the value of measuring PROs in regulatory submissions, including studies seeking approval of new indications for existing therapies, with the goal of establishing evidence of therapeutic benefit. This rapid growth in the use of PROs has not been without criticism, however, and there is increasing recognition of the challenges involved in capturing these outcomes, including the development of patient-friendly measures of PROs. The WMM, a measure of what is important to patients with Alzheimer’s disease (AD), has been widely used by investigators in the industry and the academic community to generate PROs in AD, and recently was included in the FDA’s Guidance for Industry (2017, 2020). The Guidance recommends use of evidence from the WMM to support development of innovative PROs for AD clinical trials, and to inform the design of post-marketing studies.

The purpose of this study was to compare patient and care partner ratings of the importance of 42 items of importance to AD and compare the importance ratings assigned by each to patients and care partners. The study assessed what is important to patients and care partners through the use of the WMM, a measure of what patients and care partners rated important in their care. The results may have implications for the development of PROs for AD and the design of other clinical trial endpoints.

Methods

The survey instrument was developed after first applying a series of content validation and face validity to the results of phase 1 of the study. The Delphi round was then undertaken following a series of methods for capturing preferences and priorities. Finally, the draft survey instrument was provided with convenience samples of people with AD and care partners and was pretested with people with moderate AD and AD dementia before being deployed to the general AD and caregiving populations.

Respondents were recruited through multiple convenient channels: Global Research Market Group (GRMG), the Medicare Institute, and UsAgainstAlzheimer’s (UAA).

The survey was administered in 12 states and was evaluated using the same format and scale. The administration was conducted online for AD and care partners, and in person at a site in AD and care partner clinics, at the Alzheimer’s Association (AA) of the four participating states (in Alabama, Arizona, Florida, and Georgia), department of health clinics, and through home visits, as appropriate. The survey was administered to care partners of patients with at least one year of AD and was completed online or via phone. The survey was administered to care partners of patients with moderate AD and AD dementia.

The primary objectives of phase 2 of the WMM study was to quantify the importance of symptoms, impairments, and related outcomes to AD and caregivers. The survey was administered to patients with AD and care partners.

Results

The 42 items in the WMM were rated by patients and their care partners using a Likert scale ranging from 1 (‘Very unimportant’) to 5 (‘Extremely important’). The results were analyzed using appropriate descriptive and inferential statistics.

A total of 3,125 respondents completed the survey between June 17, 2019 and September 25, 2019. Mean respondents in 2019 were recruited by GRMG and assigned to a respondent group by pharmacies. All respondents were assigned to a group: 175 respondents were assigned to the GRMG, 173 respondents were assigned to the Medicare Institute, and 1,257 respondents were assigned to UsAgainstAlzheimer’s. Additional respondents were recruited through UsAgainstAlzheimer’s in 2019 and 2020. Respondents were recruited through the Medicare Institute, UsAgainstAlzheimer’s, and GRMG in 2019 and 2020. The distribution of ratings over all items for each respondent group is presented in Figure 1. These results indicate that the WMM survey is useful to capture what matters most to patients and care partners of AD patients who are at risk for AD.

The proportion of respondents choosing each rating for each item is presented graphically for each respondent group in Figure 1 and for each care partner respondent group in Figure 2. The distribution of each rating among the patient respondent groups appears similar across the three groups in Figure 1 with each group having roughly equal numbers of respondents in each category. In contrast, the distribution of each rating among the care partner respondent groups appears different from the other groups in Figure 2 with each group having a different number of respondents in a particular category. The distribution of ratings appears to be skewed towards the higher end of the Likert scale among the patient respondent groups and more evenly distributed among the care partner respondent groups. The results were consistent with those reported by Paulsen et al. (2020) in their analysis of the results from the WMM survey.

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Conclusion & Future Directions

For more information, please contact Allison.Martin@faegredrinker.com.

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Supporting Information

The Supporting Information is available under the Article Tab.