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Point of View

What Is Reasonable and Necessary for People Living with AD after the FDA Approves a Treatment?

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The recent accelerated approvals for two anti-Abeta monoclonal antibodies (MABs), Aducanemab and Lecanemab, have important implications for people living with AD, physicians, and researchers, and they have raised controversy (1). Putting aside the issues of whether or not the drugs should have been approved by the US Food and Drug Administration (FDA), and whether or not the use of the accelerated approval process was the best approach to these approvals, it was surprising to many stakeholders that the Center for Medicare and Medicaid Services (CMS) decided that these drugs would not be reimbursed for regular clinical use, and that coverage would only be provided for people affected by AD if they enrolled in trials to generate evidence of clinical efficacy, specifically trials answering several questions proposed by CMS, and utilizing a process specified in their final National Coverage Determination (2).

There is no doubt that CMS, the FDA, and all participants in this discussion recognize the enormous challenge that Alzheimer's disease poses to society and to individuals, and that all wish to be part of the solution. Yet, in a public letter to the National Alzheimer's Association responding to the Association's final and formal request for reconsideration of the NCD, CMS explained that they would not consider revising the NCD after the second anti-Abeta MAB was approved. CMS cited their statute, and emphasized that to provide coverage nationally, CMS is required to examine whether a medication is reasonable and necessary (3). They went on to say that even if a drug of this type receives full approval from FDA, they will still only grant Coverage with Evidence Determination (CED), through registry studies or other qualified approaches, as spelled out in the NCD (2).

The main problem with this statement is that what medications are deemed "reasonable and necessary" for people living with Alzheimer's disease will vary among people. To some officials responsible for budgetary decisions, reasonable may mean an optimized investment/return ratio. Necessary may mean only that which is required by law or statute. To people living with AD and their advocates, reasonable may mean any drug that could possibly work, and necessary may mean that the best treatment that exists anywhere for their condition must be tried. Mediating between the public payor view and the desperate people affected by AD there is usually a physician or other healthcare provider who tries to educate herself to all of the perspectives, examines medically the benefit/risk ratio, filters that knowledge through his experience, considers the constraints of their local practice situation, and also has to inform the affected person of what she/he/they can actually do with respect to prescribing a treatment in the setting in which the healthcare provider works. Finally, most clinicians also feel the obligation to tell the people they are treating for AD what treatments might be available elsewhere, which requires further research and monitoring of the field.

This process by which physicians and other healthcare providers treat patients has always been a weighty responsibility, even when the first FDA-approved AD therapies quickly entered medical practice post-approval. The NCD decision adds significant burdens to the medical decision-making and the responsibility of health care providers to advocate for their patients. Now they must create or enroll in a CED study if they want to prescribe the new drugs, or research where such studies are being done and how their patients might access such studies (or explain why they cannot), and they must assist them in navigating a clinical registry or other clinical trial environment (eligibility, consent, study visits, etc.) in addition to providing routine care. Physician burnout is a serious problem (4), and what is reasonable and necessary to ask of them and their staff should figure more prominently into this discussion.

The burden of managing AD extends beyond health care providers to the societies in which they live with their patients, and this burden stretches toward a long-term horizon. So a seemingly responsible budgetary decision that saves costs in the short-term may miss the potential for reducing costs in the mid (2030) and long-term (2040/50) which are otherwise estimated to reach 1.1 trillion in the USA by 2050, (5). Timely and accurate diagnosis, timely treatment, and delayed disease progression will save money for care in the most advanced disease stages, while improving well-being of people living with AD and alleviating the urgent need for an estimated 40 million new health care workers to

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support people with dementia globally (6); a need which is compounded in the United States by well-documented weaknesses in the current U.S. dementia care workforce (7)

All things considered, a reasonable approach would simply be to say that when the FDA approves a treatment because it is safe and efficacious, or because it is reasonably likely to be safe and efficacious but has some post-marketing commitments that must be met to support that prediction, patients and clinicians can discuss the pros and cons and decide whether to use the treatment. Pharma companies should work with payors to responsibly define the patients who should be treated, and patients should then be able to count on their payors to cover the medication. Such an approach will reassure affected families, unburden clinicians, and demonstrate to all societal stakeholders that efforts are being made today to avoid a tsunami in the future.

Conflict of interest: RS Doody and S Skerjanec are employees of F. Hoffman-Laroche Ltd, and hold stock and stock options in the company.

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