AHRQ and CMS Clinical Trial Meeting

On September 10, 2004, CMS met with representatives from AHRQ, NIA, FDA, academia, and industry to discuss the clinical trial proposed in our draft decision memorandum. The group was in general agreement on the outline of a large, community-based, practical clinical trial that would assess the additional benefit that the availability of PET scan would have on patient management, quality of life measures for beneficiaries and caregivers, resource utilization, adherence to care plans, and hospitalization or admission to nursing facilities.[1]

The group recommended that the patient population be comprised of beneficiaries presenting with cognitive impairment including those diagnosed with MCI or early dementia. Also, patients would be assigned to study groups with some receiving only a standard workup and others having access to a PET scan in addition to the standard workup. The group also suggested that the benefit should be separately assessed for primary care physicians evaluating and treating cognitive decline as well as for specialty providers with significant experience in the management of Alzheimer's disease. Patients would be followed for several years with data reporting at pre-specified time intervals. The final study protocol will be developed by an expert workgroup with similar composition to this group.

It is important to state that some NIA representatives and others expressed reservations about the value of the proposed trial at this time, given that studies have not shown the added value of PET scans and other imaging or biomarker modalities in the clinical diagnosis of AD. They noted that experienced clinicians could diagnose AD with a high degree of accuracy, although diagnosis is more difficult at earlier stages of the disease. Members of the group noted that the lack of disease-modifying treatments for cognitive impairment or AD at this time, and little evidence that early treatment with the drugs currently approved for the treatment of symptoms in AD is beneficial, will add to the difficulty in evaluating meaningful clinical outcomes to PET scanning or other imaging or biomarker modalities that show comparable potential for early diagnosis.

NIA staff appreciated efforts to collect data on meaningful clinical outcomes on which to make evidence-based reimbursement decisions, and underscored that having such data in hand prior to decisions will encourage the collection of quality data. There were concerns about the impact of any reimbursement decision before the analysis of data from a trial to assess outcomes, which had been discussed at the April meeting organized by the NIA. One of the concerns was that the availability of reimbursement would decrease incentives for research, as has been seen in other situations after CMS reimbursement decisions.

[1] S Tunis et al. Practical clinical trials, Increasing the value of clinical research for decision making in clinical and health policy *JAMA* September 2003.