

Meaningful ALS Research Does Not Always Involve Treatment

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The ultimate goal of research into ALS is to find a cure. Not surprisingly, the research that individuals with ALS are usually most interested in concerns new treatments. However, research sometimes is done to improve our ability to manage symptoms and maximize quality of life (QOL). Or, research may be done to develop a tool to help in diagnosis. These types of research typically receive less attention than treatment trials, but are very important nonetheless.

Research to improve management of symptoms and QOL: It is probably accurate to say that on a day-to-day basis, most individuals with ALS, their families, and their caregivers spend most of their time dealing with management issues. But, how does one measure the effect of such management on QOL, and what management measures are of value? Research can play a role in helping us sort this out.

We recently designed a questionnaire to measure QOL in ALS, and administered it to 342 individuals with ALS at 7 university centers. That study demonstrated that QOL depended on emotions, interaction with people and the environment, intimacy, and religion, in addition to physical symptoms and bulbar function. We are now entering into the final phase of testing this instrument at a number of university-based ALS centers. This has improved our perception of what is important to our patients, and has permitted us to better assess the effect on QOL of medications and other means of managing and treating ALS.

Research has also been a key factor in sorting out the role of religion and spirituality in ALS. A recent publication by our group demonstrated that there is an increasingly strong relationship between religiousness and QOL over time in individuals with ALS. In view of this, we undertook a study whose goal was to assess the usefulness of the services provided by our pastoral care counselor in ALS clinic. After all, if we don't ask, how do we know whether this service is useful? We are now reviewing all the data, and I anticipate we'll be announcing our results soon.

One of our other interests has been the study of thinking and concentration (cognitive function) in ALS. We found that some individuals with ALS have difficulty recognizing emotions in people's faces. We now conduct tests of thinking and concentration regularly in ALS clinic, and have found that some individuals with ALS develop changes in their ability to process complex ideas or perform complex tasks, while others develop some changes in personality or judgment. By understanding this better, we hope

to be able to work more effectively with these individuals and their families during the course of their disease.

Many individuals with ALS receive specific dietary recommendations or are prescribed non-invasive positive pressure ventilation (NIPPV, or BiPAP). However, the guidelines for using these treatments are still being worked out. We will soon be one of several ALS centers participating in a study sponsored by the National Institutes of Health to evaluate nutrition and NIPPV in individuals with ALS. This study will not involve any new treatments. However, individuals who volunteer for the study will undergo a number of tests of their energy intake, energy consumption, and breathing, so that physicians and nutritionists caring for these patients can provide better recommendations. I'll provide more details in a future newsletter.

Research to aid in diagnosis: Obtaining a diagnosis of ALS can be a very long process involving many tests to rule out other disorders. For example, virtually every individual with ALS undergoes an MRI scan at some point. This is done to rule out other disorders, but is not used to make a diagnosis of ALS. We are making plans to begin a study in which we will ask individuals to have some special MRI scans of their brains, in an attempt to better understand the regions of the brain that are involved in ALS, and to determine whether these scans may be helpful in diagnosing ALS. A spinal tap is another example of a test usually used to rule out other diseases. But, recent studies at the University of Pittsburgh have looked at certain compounds in spinal fluid which may aid in the diagnosis of ALS. These compounds, known as biomarkers, are also being studied in Alzheimer's disease. We hope to undertake a study here in the near future to look at spinal fluid biomarkers as well. A test which produces a "positive" result in ALS, and thereby helps to confirm the diagnosis, could greatly decrease the time and effort involved in testing.

Closing thoughts: It is likely that some of you have been asked, or will be asked, if you would like to participate in research studies which involve no direct benefit to you, but which may increase knowledge of ALS in general. I have been repeatedly impressed by the willingness of many of our patients to participate in such research, despite the inconvenience and time involved. I offer my sincere thanks and gratitude for the contributions so many of you have made to understanding and managing this disease.