Beyond The Bucket: Q&A with John Ravits, MD

By Scott LaFee | January 05, 2016

The Ice Bucket Challenge has come and gone, but efforts to find new treatments and perhaps a cure for amyotrophic lateral sclerosis (ALS or "Lou Gehrig's disease") go on. The progressive neurodegenerative disease affects more than 20,000 Americans, with 6,400 new diagnoses each year.

We asked John Ravits, MD, professor of clinical neurosciences at the Center for ALS Research and Therapy, for an update.

Question: The first estimate of ALS prevalence in the United States was published by the Centers for Disease Control in 2014. It reported 3.9 cases per 100,000 persons in the general population. How does this number jibe with your work and understanding?

Answer: ALS is reported in incidence (number of new cases per 100,000 population) and prevalence (number of existing or total cases per 100,000 population). Since ALS patients live about three to five years (after diagnosis), the incidence is about 2 to 3 per 100,000 and the prevalence is about 8 to 10 per 100,000. These seem right for San Diego County.

Q: Since the Ice Bucket Challenge, have you seen any identifiable or measurable effects, such as more funding available for research or a sustained, higher public awareness of the disease?

A: Yes, the Ice Bucket Challenge has allowed the ALS Association to do many things that they previously wanted to do but could not afford. We are seeing more consistent and solid support than before for research efforts and patient care support.

Public awareness for this disease is also higher, which I think is in part because of the Ice Bucket Challenge, but I also think the success of the Ice Bucket Challenge shows that the public awareness was already there and the time was right for an outpouring of support.

Q: The current standard of care for ALS emphasizes treatment of symptoms. There is one approved drug – riluzole – that appears to slow motor neuron damage in some patients. How would you describe prospects of new drugs or therapies that go beyond riluzole?

A: There is a large effort now not only by the National Institutes of Health and lay organizations but also by industry, which sees opportunity. Many drugs targeting different molecular pathways and trying different approaches are in the pipeline. I am very encouraged.

Q: Which areas of ALS research specifically interest or excite you?

A: The three most exciting areas for my research are: developing therapies that bring down gene products in genetic forms of the disease—these therapies have been pioneered by Isis Pharmaceuticals in nearby Carlsbad, which is partnering with Biogen in Cambridge, Mass. to start clinical trials for some gene mutations in early 2016. Don Cleveland in the Department of Cellular and Molecular Medicine at UC San Diego has played a major role in this development; developing an understanding of the spread of disease though the central nervous system; and studying molecular pathways neuropathologically.

Also, there has just been a report finding a possible role for an endogenous retrovirus that has been inserted into the genome through evolution. That's a story that scientist will move very fast to validate and pursue if validated.

Q: What do you tell patients or others about how things stand in terms of understanding ALS and, ultimately, curing or reversing it? Are we on the cusp of major advances or still early days?

A: I tell patients that there has never ever been such rapid, real and exciting progress in the field. This is a worldwide effort by investigators, particularly in the U.S., Canada, Europe and Japan. Investigators in other parts of the world are also joining in. At the same time, research takes years.

For example, mutations in a very important gene called C9orf72 were identified in late-2011 as the most common genetic cause of ALS, and now four years later, there has been astounding progress in understanding this and, probably in one or two more years, drugs that I discussed above targeting this gene will go into clinical trials.

While incredibly rapid, this is a very long time in the life of an ALS patient. I try my best to encourage them. I also try to remind them that participation in research will benefit the next generation of patients.

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