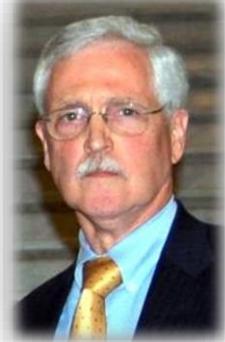


A Research Update - – Towards New Treatments for ALS

By Dr. Robert H. Brown (July 10, 2018)



I am pleased to provide an update on our ALS clinical trial activity, which has been so generously supported through by the Angel Fund. The focus has been on a new and innovative program to use gene therapy in treating a particular form of hereditary ALS associated with the mutant SOD1 gene. More recently, we now have developed a different – but similar – modality for treating ALS associated with the mutant C9 gene. With over 40 identified genes associated with ALS, the SOD1 and C9 forms together represent approximately 65% of all genetic ALS.

As you may know, last summer we treated a single ALS patient on a compassionate use basis using a new gene therapy approach aimed at silencing the SOD1 gene, from which we have learned a great deal. The results from that first patient have now embolden us to treat a second patient, also on a compassionate use basis, which we hope will happen soon.

The first patient was treated at UMass Memorial Medical Center in Worcester; this second patient will be treated in Boston at Mass General Hospital under the care of my longtime collaborator, Dr. Merit Cudkowicz. We have evolved the protocol for the second patient based on what we learned from the first, providing us with high hopes and expectations for further success. If we do see strong progress with the second patient, that hopefully will trigger a plan to launch an expanded access, Phase I trial that will treat approximately 10 additional patients.

I also am pleased to announce a new partnership with a biotech start-up, called Apic Bio, a novel gene therapy company that was spun-out of UMass Medical School. Together with your generous support, Apic will help finance this compassionate use trial and help us evolve this work into the expanded access, Phase I trial. Having an industry partner like Apic allows us to leverage your generosity and accelerate our work with high expectations for success at the clinical-trial-stage.

I also am pleased to report that in addition to our promising SOD1 work, we continue our efforts to bring new therapies to clinical trial targeting the C9 gene as well. Our work on C9 has been of particular interest and focus for the Angel Fund, for which we are so grateful. We are moving forward with our C9 initiative on a dual pronged approach. First, we are using our own, in-house antisense technology to silence the mutant C9 gene. Second, we are fortunate to have partnered with a Cambridge based biotech company, called Wave Life Sciences, to help them develop a novel type of product that also silences the C9 gene.

The past twelve months have proven to be an inflection point in my more than 30 years of studying ALS. Entering into these human clinical trials with innovative and promising new therapies represents the culmination of a lot of hard work by a talented and dedicated team at UMass Medical School and the Mass General Hospital. While we still have so much more work to do and many more miles to travel, I never have been more optimistic about the possibility of effectively treating genetic ALS – and eventually all forms of ALS.