



Biogen Idec Forms First-of-Its-Kind Research Consortium to Identify ALS Drug Targets

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WESTON, Mass.--(BUSINESS WIRE)--[Biogen Idec](#) (NASDAQ: BIIB) announced today that it has created a new research consortium in collaboration with several leading academic research centers that will leverage a range of scientific techniques and disciplines to identify new approaches to treating amyotrophic lateral sclerosis (ALS). It is anticipated that coordinating research and sharing results across a number of different disciplines will greatly accelerate the understanding of the mechanism of this disease and the development of new targets and approaches to treatment.

Through the Consortium, each participating lab will undertake a three-year research project. Researchers will meet on a regular basis to provide updates and share information and insights emerging from their research and to exchange results from ongoing efforts at Biogen Idec, which will help to guide ongoing research activities. This level of collaboration will enable researchers to apply others' learnings to their own efforts in near real time.

"ALS research is a primary area of focus for Biogen Idec, but has proven to be a very difficult disease to understand and treat," said Spyros Artavanis-Tsakonas, Ph.D., senior vice president, chief scientific officer, for Biogen Idec and Professor of Cell Biology, Harvard Medical School. "We believe that taking a holistic approach that explores the many variables involved in the development and progression of ALS will speed our ability to identify viable drug targets that can be moved into testing. We are hopeful that the assembly of this extraordinary group will allow unprecedented insights and define a new level of synergy between academic research and Biogen Idec."

Biogen Idec has committed more than \$10 million over three years to fund research projects by members of the Consortium. This initiative will complement and extend a collaboration the company announced earlier this year with Duke University and the Hudson Alpha Institute to sequence the genomes of 1,000 people living with ALS.

The members of the Consortium were identified based on their core scientific and technical skills. Expertise in ALS was not a primary requirement for participation, although the group includes some of the most renowned neuroscientists and ALS researchers in the world.

Members of the Consortium are:

Pietro De Camilli, M.D., Eugene Higgins Professor of Cell Biology and Professor of Neurobiology; Director, Yale Program in Cellular Neuroscience and Neurodegeneration and Repair; Investigator, Howard Hughes Medical Institute, Yale University School of Medicine. Dr. Camilli's research as part of this Consortium will investigate the general role of the VAP protein family in lipid regulation and endoplasmic reticulum stress response and to identify the molecular pathways that are affected by disease-causing mutations of the VAP-B protein.

J. Wade Harper, Ph.D., Bert and Natalie Vallee Professor of Molecular Pathology, Harvard Medical School. Dr. Harper's work as part of the Consortium will focus on profiling the organization of the mitochondrial interactome in response to ALS mutant proteins to determine whether expression of mutant proteins linked to ALS leads to activation or inhibition of the mitochondrial quality control system.

Christopher E. Henderson, Ph.D., Gurewitsch/Vidda Professor of Rehabilitation and Regenerative Medicine, Pathology, Neurology and Neuroscience; Director, Columbia Stem Cell Initiative; Co-Director, Center for Motor Neuron Biology and Disease (MNC); Co-director, Project A.L.S. Laboratory for Stem Cell Research; Columbia University. Dr. Henderson's work is focused on the mechanisms of motor neuron growth, survival and cell death and how these are regulated in human disease. His research as part of the Consortium is to explore the differences between motor neurons in the spinal cord, which are destroyed in ALS, and those which move the eyes, which are unaffected in ALS, with the goal of identifying genes that may confer protection against the disease.

Arthur L. Horwich, M.D., Sterling Professor of Genetics and Professor of Pediatrics, Yale School of Medicine; Investigator, Howard Hughes Medical Institute, Yale University School of Medicine. Dr. Horwich's research as part of the Consortium is focused on the role of the SOD1 gene, which has been associated with protein misfolding and has been shown to play a role in pathology of ALS. In particular, the lab will explore whether misfolded SOD1 protein can spread from affected tissue to normal tissue using mouse models of ALS.

Lee L. Rubin, Ph.D., Professor of Stem Cell and Regenerative Biology; Director of Translational Medicine, Harvard Stem Cell Institute, Harvard University. Dr. Rubin's work uses a stem cell based approach to understand neurodegenerative diseases. Dr. Rubin's work as part of the Consortium is to develop SOD mouse or human motor neuron cell lines that capture early pathological changes and use these cellular phenotypes to identify drug-like molecules/pathways that affect disease pathology to identify potential therapeutic leads.

Marc Tessier-Lavigne, Ph.D., President; Carson Family Professor; Head of the Laboratory of Brain Development and Repair, The Rockefeller University. Dr. Tessier-Lavigne's laboratory investigates how neural circuits in the brain form during embryonic development. As part of the Consortium, Dr. Tessier-Lavigne's lab will investigate cell death pathways and determine how they contribute to motoneuron degeneration and disease progression in ALS.

Spyros Artavanis-Tsakonas, Ph.D. will oversee the Consortium on behalf of Biogen Idec and will lead research efforts for the company and his lab at Harvard University. Dr. Artavanis-Tsakonas's Consortium research focus will employ high throughput screens in *Drosophila* (often called "fruit flies") to identify genes that effect ALS dependent phenotypes resulting from expression of disease causing forms of SOD1, TDP-43 and FUS, and to identify protein complexes associated with ALS-related proteins in humans and *Drosophila*.

Biogen Idec Commitment to ALS Research

Biogen Idec is committed to advancing the understanding and treatment of ALS. The company is working with researchers around the globe to explore ways to create better outcomes for people with ALS.

Biogen Idec is investigating multiple compounds in ALS, with two compounds in preclinical research. In 2012, the company entered into a research collaboration with Duke University and Hudson Alpha Institute to sequence the genomes of up to 1,000 people with ALS over the next five years in an effort to gain a deeper understanding about the fundamental genetic causes of the disease. Duke and Hudson Alpha will work with several world-class researchers who have deep expertise and experience with ALS and the genes associated with the disease.

In addition, Biogen Idec has sponsored research agreements with various academic institutions with the shared goal of understanding the common course of ALS from symptom onset to diagnosis, through treatment and life expectancy.

The company has also contributed \$500,000 to the University of Massachusetts Medical School ALS Champion Fund. The funding will increase awareness of ALS and support basic and clinical science research into potential treatments for ALS and other neurodegenerative diseases.

About ALS

Amyotrophic lateral sclerosis, also known as Lou Gehrig's disease and motor neuron disease, is a universally and rapidly fatal neurodegenerative disorder characterized by progressive muscle weakness and wasting. ALS affects adults in the prime of life and creates a substantial burden for caregivers. Worldwide incidence of ALS is approximately two people per 100,000. Only one drug has been approved for the treatment of ALS, and it typically extends survival by two to three months. Life expectancy after the onset of symptoms is usually three to five years. Though mutations in several genetic factors result in ALS, the precise mechanistic cause is not yet known.

About Biogen Idec

Through cutting-edge science and medicine, Biogen Idec discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hemophilia and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company. Patients worldwide benefit from its leading multiple sclerosis therapies, and the company generates more than \$5 billion in annual revenues. For product labeling, press releases and additional information about the company, please visit www.biogenidec.com.

Biogen Idec Safe Harbor Statement

This press release contains forward-looking statements, including statements about the development of potential therapies for ALS. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "expect," "forecast," "intend," "may," "plan," "will" and other words and terms of similar meaning. You should not place undue reliance on these statements. Drug development and commercialization involve a high degree of risk. Factors which could cause actual results to differ materially from current expectations include the risk that adverse safety events may occur, regulatory authorities may require additional information or may fail to approve any potential new therapy, product reimbursement may be limited or unavailable, there may be problems with manufacturing processes, intellectual property rights may not be adequately protected, and the other risks and uncertainties that are described in the Risk Factors section of Biogen Idec Inc.'s most recent annual or quarterly report and in other reports Biogen Idec Inc. has filed with the SEC. These statements are based on current beliefs and expectations and speak only as of the date of this press release. Biogen Idec Inc. does not undertake any obligation to publicly update any forward-looking statements.

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