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Prize4Life Announces $1 Million ALS Treatment Prize Winner

Recognizes French scientists for ALS gene therapy that has demonstrated dramatic increase in survival rate in preclinical development

Berkeley, CA – January 31, 2017 – Prize4Life, a nonprofit organization whose mission is to accelerate the discovery of treatments and a cure for ALS (Amyotrophic Lateral Sclerosis, also known as Lou Gehrig's disease), today announced the winner of its $1M Avi Kremer ALS Treatment Prize. The winners are the team of Drs. Martine Barkats and Maria-Grazia Biferi, from the Institute of Myology U974 INSERM UMPC in Paris, France. They have developed a gene therapy approach targeted at one of the most common known causes of inherited ALS. This approach significantly extends survival and slows disease progression beyond any results reported to date in the most frequently used ALS mouse model.

ALS is a terminal neurodegenerative disease caused by the death of neurons in the brain and spinal cord that control voluntary movement. People with the disease gradually lose the ability to walk, speak and eventually, breathe. In fact, 80 percent die within five years of diagnosis, with the average patient surviving only three years from diagnosis. Although the disease was first clinically described more than 150 years ago, there is only one FDA-approved therapy for ALS, which prolongs patient survival by merely a few months.

The ALS Treatment Prize was launched with the aim to accelerate discovery of a treatment for ALS by encouraging researchers from around the globe to test new candidate therapies. The prize criteria sought a treatment candidate that increased lifespan of SOD1 ALS mouse models by at least 25 percent (under specific experimental conditions) – significantly more than had ever been done before. SOD1, or superoxide dismutase, was the first ALS-causing gene to be identified, more than 20 years ago, and is mutated in approximately two percent of all ALS cases. The winning team's approach to shutting down expression of SOD1 not only extended survival of treated adult mice by more than 50 percent (far above the 25 percent prize requirement), but also significantly improved the motor function of the mice even after they began to show symptoms. While SOD1 gene silencing therapies have been the focus of many research efforts in the past and present, the winning team's approach is unique in its design, the mode of administration and its improved efficacy. The ALS Treatment prize was also designed to increase the likelihood of generating reproducible results between labs, a known challenge in biomedical research. At the close of the challenge, a repeated independent study conducted by the ALS Therapy Development Institute in Cambridge, Mass. confirmed the winning team's results.

"We launched this prize with the goal of creating momentum around testing of new treatments for ALS, and are enthusiastic about the potential of the winning team's solution. It far exceeds the survival threshold we laid out, and is beyond anything that's been done before in this mouse model. We are hopeful these results will ultimately translate to humans," said Dr. Nicole Szlezak, Chair of Prize4Life's Board of Directors.

"We are thrilled to have been recognized for the work we've done and awarded the ALS Treatment Prize. It has been extremely rewarding to dedicate our research efforts to such a devastating disease that still puzzles experts in the industry," commented Drs. Martine Barkats and Maria-Grazia Biferi. Adding, "We're proud to have helped accelerate the road to a viable treatment through our new approach to SOD1 therapy and look forward to continuing work to advance this gene therapy to human trials."

The ALS Treatment Prize is the third challenge of the original triptych of Prize4Life Challenges aimed at identifying biomarkers, new drug targets and therapies, and computational methods for improving ALS clinical trial design. In 2011, Prize4Life awarded a $1M ALS Biomarker Prize to Dr. Seward Rutkove of Beth Israel Deaconess Medical Center in Boston for his development of a technology that sensitively measures disease progression of ALS patients and can help reduce the costs of Phase II clinical trials by 50 percent. In 2012, the DREAM-Phil Bowen ALS Prediction Prize was awarded to two teams that developed algorithms to predict disease progression in patients and improve clinical trial design. Sentrana, a market research company that employed one of the winning teams, spun off the company Origent Data Sciences following the success of the ALS Prediction Prize. Origent Data Sciences is currently working with Cytokinetics and other ALS companies on clinical trial design and interpretation.

"It's exciting to see the progress being made by our prize participants, including winners Drs. Barkats and Biferi, as well as the broader industry, when it comes to finding therapies that can quickly advance to human trials," said Prize4Life Founder and Board of Director Avi Kremer, who was diagnosed with ALS in 2004. "We have made huge strides in ALS awareness and research and are proud our prizes are helping increase focus on the disease and march us toward an eventual cure."

About Prize4Life
Prize4Life is a 501(c)(3) nonprofit organization whose mission is to accelerate the discovery of treatments and a cure for ALS (Amyotrophic Lateral Sclerosis, also known as Lou Gehrig's disease) by using powerful incentives to attract new people and drive innovation. Prize4Life believes that solutions to some of the biggest challenges in ALS research will require out-of-the-box thinking, and that some of the most critical discoveries may come from unlikely places. Founded in 2006 by Avi Kremer, who was diagnosed with ALS at the age of 29, Prize4Life encourages and rewards creative approaches that will yield real results for ALS patients. For more information, visit www.prize4life.org.

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