



CHALLENGE SPECS

CHALLENGE
ALS Biomarker

DISCIPLINES
Medicine, Biology, Genetics,
Drug Discovery

SOLVERS ENGAGED
2,969

SOLUTIONS
108 Solutions Received, 12
Finalists

CHALLENGE AWARD
\$1,175,000

CHALLENGE IMPACT

- The biomarker has the potential to reduce the cost of Phase II clinical trials by more than 50%.
- ALS treatments can now move more quickly through the development pipeline, providing a greater incentive for pharmaceutical and biotech companies to invest in ALS drug development.
- The prize has brought new research funding and raised the visibility of the disease in the eyes of the larger public.
- The solution may also be applicable to Parkinson's and other neuromuscular disease research.

A Deadly Disease With No Cure

Amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, is a progressive and fatal neurodegenerative disease. In ALS, the nerve cells that control voluntary muscle movement gradually stop functioning, and patients become paralyzed, though their minds typically remain intact. Most people with ALS die from respiratory failure, usually within two to five years. There is no known cure for ALS, and only one treatment which prolongs survival for a few months at best and is often prohibitively expensive.

A major obstacle in finding a cure for ALS has been the lack of a way to measure the progression of the disease and monitor the effectiveness of treatments. Clinical trials require large numbers of patients over a long period of time, and in the minds of many companies, ALS patients die too quickly to be effectively studied. This has made ALS drug development difficult and expensive, thus pharmaceutical and biotech firms have steered away, leaving ALS patients with little hope for the future.

Challenge: Find a Biomarker for ALS

In 2006, Avi Kremer, a 29 year old Harvard Business Student and former Captain in the Israeli army was diagnosed with ALS. After learning that there was no cure, and in fact, no hope for a cure, he decided to apply his training and brainpower and the resources available to him at Harvard to make the biggest impact he could on ALS research. He formed Prize4Life, Inc. along with several classmates with the mission "to accelerate the discovery of treatments and a cure for ALS by using powerful incentives to attract new people and drive innovation."

After studying various options, Avi and the other founders of Prize4Life decided to use incentive prizes as a way to attract new minds and new money to the fight against ALS and focus their attention and resources on very specific research outcomes. They would award \$1 million to the first person or team to identify an ALS biomarker.

The prize approach had several advantages. First, it would bring attention to the need for an ALS biomarker and would give researchers clear metrics for effectiveness. Second, the prize presented a novel way to get funding for the disease – donors would only pay if the Challenge was solved. This appealed to donors who want to see results from their donations. Third, the publicity around the prize – the largest that InnoCentive had ever posted – would help generate awareness for the disease.

To launch their Challenge, Prize4Life worked closely with InnoCentive to articulate the problem in a way that would provide the highest likelihood of success. The Grand Challenge was posted on InnoCentive's cloud-based open innovation and crowdsourcing platform and made available to InnoCentive's 250,000+ registered Solvers and millions of potential Solvers via InnoCentive's partnerships with organizations including Nature Publishing Group. Nearly 3,000 Solvers from dozens of countries participated in the Challenge. In the end, 12 Solvers submitted solutions for the \$1 Million prize.

“InnoCentive’s Solver network is unparalleled.”

“Working with InnoCentive expanded our reach to new minds around the world, which would have been inaccessible without this partnership. When we launched what we thought was a neuroscience Challenge we certainly didn’t expect some of the most exciting ideas to come from the fields of dermatology, chemistry, and plant biology and yet that’s what happened. InnoCentive’s Solver network is unparalleled.”

Melanie Leitner, Ph.D.,
Chief Scientific Officer,
Prize4Life

Solution: New Applications for Electrical Current

In 2007, Prize4Life awarded \$75,000 in Thought Prizes to encourage promising concepts. The 5 winners of these prizes included a plant biologist and a dermatologist with no prior ALS experience, demonstrating the importance of diversity for solving problems (and in fact, two-thirds of the teams competing for the prize came from outside the traditional ALS field). Another \$100,000 in Progress Prizes was awarded in 2009 to two groups: “We thought that the findings of these two Solver teams were so significant that we had to broadcast them to the larger ALS community as well as honor the accomplishments of the research teams in this vital area of biomarker discovery,” said Melanie Leitner, Chief Scientific Officer for Prize4Life. “For that reason, even though neither Solver team met 100% of our very rigorous prize criteria, the Scientific Advisory Board wanted to honor them with these Progress Prizes and alert the rest of the ALS community to their impressive work.”

In early 2011, the full \$1 million was awarded to Dr. Seward Rutkove, a neurologist at Beth Israel Deaconess Medical Center in Boston, for his work with electrical impedance myography (EIM), which sensitively measures the flow of a small electrical current through muscle tissue. In his solution, Rutkove demonstrated that as the disease progresses, ALS patients’ muscles atrophy, and the more their muscles weaken and shrink, the greater the change detected as current moves through it. By comparing the size and speed of electrical current as it passes through healthy and diseased tissue, EIM can be used to accurately measure the progression of the disease.

Impact: Hope for ALS Patients

The current cost of a Phase II ALS clinical trial is roughly \$10 million. Using this biomarker, the time required to determine the therapeutic benefit of a given drug in a clinical trial will be shorter and fewer patients will be needed. This will reduce the cost to \$5 million or less. Faster and cheaper clinical trials mean that potential therapies will move more quickly through the development pipeline, accelerating progress toward a treatment or cure for ALS, and the pharmaceutical industry will have a greater incentive to invest resources in ALS drug development. In addition, the ALS Biomarker Prize competition has already brought new research funding into ALS and raised the visibility of the disease in the eyes of the larger public.

Conclusion: Marching Toward a Cure

Since the Challenge was awarded in 2011, Prize4Life has been actively working with biotechnology firms to integrate the new biomarker into ongoing clinical trials. Neuralstem, a biotherapeutics company, is using the biomarker in an ALS clinical trial. Two additional biotechnology companies, Biogen Idec and Genzyme, are actively considering incorporating the biomarker into trials as well. Dr. Doug Kerr, associate director of experimental neurology at Biogen Idec, which is working on an ALS drug, said more sensitive testing methods “will allow us to test more drugs, more patients, and get an answer earlier.” He called Dr. Rutkove’s method “a powerful new part of the armament to study ALS.”