# ALS: Changing the Outcome of a Debilitating and Fatal Disease



Arthur Horwich Professor, Genetics

# **CURRENT RESEARCH**

A multi-avenue approach including a black box one to identify causes and treatment for patients with ALS

At any one time, there are about 20.000 Americans affected with ALS, also known as Lou Gehrig's Disease. Patients with ALS often present with muscle weakness/cramping or slurred speech. Symptoms are progressive and debilitating, affecting a patient's ability to move, speak, and, eventually breathe. Despite the many patients, there is little treatment available to combat the disease. Dr. Arthur Horwich, Professor of Genetics at Yale School of Medicine, hopes to understand the basic mechanisms in ALS that lead to motor neuron dysfunction and cell death, with a view to finding ways to develop drugs that could treat this lethal disease. His work is motivated by the people that such breakthroughs will save. So challenging is ALS that Tony Judt, a famous British/American historian of the 20th century who developed and succumbed to the disease, described it as "imprisonment without the chance of parole." Dr. Horwich and his team are aiming to change that outcome.

Currently, the only drug available for patients with ALS is riluzole, which prolongs life by about 1-2 months. One approach Dr. Horwich and others have taken is to study mutant SOD1 transgenic mice, which develop ALS resembling the human disease; mutant forms of SOD1 cause about 20% of inherited ALS. Dr. Horwich finds that when these mice also express a "chaperone" protein called Hsp110, it appears to protect against motor neuron damage, and these animals survive much longer. Additionally, he and his team are carrying out electrophysiology studies, with Professor David McCormick at Yale, shedding light on the motor neurons that are most vulnerable in ALS; further studies of molecules involved may allow them to figure out how to protect these vulnerable...

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# **AFFILIATION**

Yale University

## **EDUCATION**

- A.B. & M.D., Medicine, 1975 , Brown University
- Pediatric Residency Training, 1975-1978 , Yale School of Medicine
- Postdoctoral Fellow, Molecular Biology and Virology Laboratory, 1978-1981 , Salk Institute
- Postdoctoral Fellow, Genetics, 1981-1984 , Yale School of Medicine

### **AWARDS**

- Gairdner International Award
- Rosenstiel Award for Distinguished Work in Basic Medical Science
- Louisa Gross Horwitz Prize
- Shaw Prize in Life Science and Medicine
- Lasker Award for Basic Medical Research
- and 1 more...

### **RESEARCH AREAS**

Life Science, Neurological / Cognitive, Neurological / Cognitive

# **FUNDING REQUEST**

Your contributions will support the continued research of Dr. Art Horwich, of Yale School of Medicine, as he seeks to understand the basic mechanisms of ALS. Donations will fund the necessary \$1M/year required for personnel, lab equipment, and therapeutic testing. Additional funding would allow Dr. Horwich and his team to broaden their scope and pursue their studies more rigorously. Join in research that will attack an unmet medical need impacting thousands of patients affected by ALS each year; fund Dr. Horwich's efforts.

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