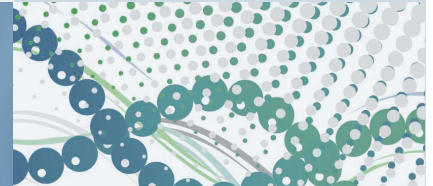




Summary of the ALS Therapy Alliance's recent accomplishments

- Funded 44 different ALS investigations to date
- Funded primary investigators in 6 countries (US, Australia, Chile, India, Mexico, UK)
- Sponsored 9 ALS conferences worldwide
- Awarded \$16 million in grants
- Generated data and funding for 5 NIH research grants, amounting to > \$10M

ALS Therapy Alliance: New Research, New Treatments, New Hope.



“New News” in ALS Research

New Treatments

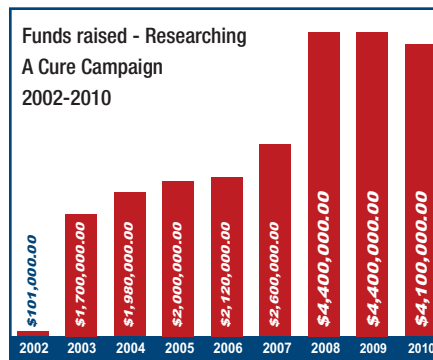
Pramipexole, a novel oral neuroprotective therapy, had potentially beneficial effects in recent pilot studies, showing that it may extend survival in ALS patients. The drug is now entering a phase three study. In addition to traditional drugs, researchers are also investigating therapies that silence expression of toxic genes, such as SOD1G, a mutation that can cause ALS. This innovative new approach represents tremendous progress.

The role of DNA

Scientists believe that susceptibility to ALS is strongly influenced by genetic makeup and there has been substantial progress in identifying new genetic variants that make people susceptible to the disease. A large consortium study has recently demonstrated that about 10% of sporadic ALS cases are associated with variants in a genetic marker that may also be related to familial ALS and a type of dementia (fronto-temporal dementia). This underscores the importance of finding this ALS-FTD gene and also highlights the important potential overlap between inherited and non-inherited forms of ALS. ATA-funded studies use the latest gene identification technology in the search for new ALS genes.

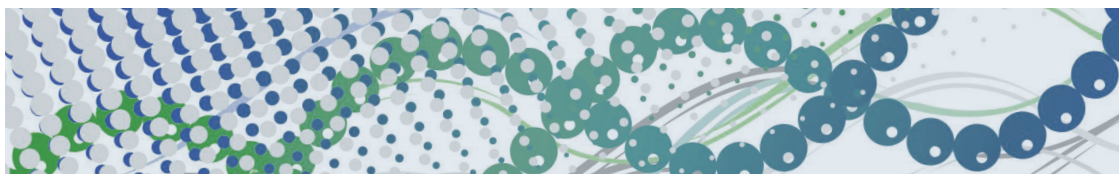
Cross-cultural Collaboration

The ATA has galvanized experts from around the globe in the fight against ALS. ATA has helped establish new ALS networks in Mexico and India, and funds research worldwide, including Australia, China, England, Belgium and Germany. Additionally, the ATA recently co-funded two international symposia on ALS, as attendees from around the world strive to find a cure.



Highlights of Research by ATA-funded Investigators

- Accelerated identification of at least six new ALS genes
- Accelerated application of new technologies for gene identification
- Permitted investigations of novel ALS aspects of RNA biology in ALS
- Enhanced therapy development
- Funding studies of stem cells to explore the biology of ALS and ALS-dementia





“ By bringing together the very best minds in the field of ALS research, the ATA has focused funds and resources to make great strides in the battle against this disease. ”

Jonathan Roberts

Executive Vice President, Rx Purchasing, Pricing and Network Relations, CVS Caremark
ATA Board of Directors

Animals Aid Research

Animals are powerful tools in the study of ALS, and the diversity of animal models has expanded substantially. These animal models have involved mice and rats and, more recently, fruit flies and fish. Each animal offers its own particular advantages as an experimental model. By studying animals with ALS, scientists can learn more about the process of degeneration of motor nerves in the brain and spinal cord, efficiently testing new discoveries and treatments.

Advances in Stem Cell Therapy

There's been extraordinary progress in the application of stem cell biology to studying neurodegeneration in ALS, which has significant implications for processes such as drug screening. Stem cell biology has made huge advances in the last five years, accelerating ALS research and the quest for therapy. Stem cells may offer a variety of benefits for ALS, and may eventually allow individuals to replace lost neurons, helping to restore lost neurological function and may form new support cells that make existing neurons healthier. And, stem cells may be useful as vehicles for delivering drugs to the nervous system. It's now possible to generate stem cells from skin biopsies of living patients, which is significant

because scientists can study motor neurons that have the exact genetic makeup and constituents of the patients under assessment. This huge advance also bypasses ethical issues surrounding fetal-derived stem cell research.

Drug delivery to the brain

Ground-breaking methodologies exist for silencing genes that make toxic poisons or toxic proteins that lead to neurodegeneration. At least two new technologies are being tried in humans to silence these offending genes, including antisense oligonucleotides and SIRNA, small inhibitory RNA. Both are currently showing promising results.

Axonal Transport

Axonal transport has become an important new area of ALS research. Genetics, and some mutant ALS genes, have incriminated the process of axonal transport, which is pathological in both sporadic and familial ALS. New findings indicate that in some cases of sporadic ALS (with no mutations in the SOD1 gene or protein), the SOD1 protein gets misfolded and can directly inhibit axonal transport.

“ The ALS Therapy Alliance board members are profoundly grateful to CVS/pharmacy for its ALS fundraising campaign, which has been invaluable in the effort to develop an international consortium of ALS researchers. ”

Robert H. Brown, Jr.

D.Phil., M.D, Chair of the Dept. of Neurology, University of Massachusetts Medical School

ALS
THERAPY ALLIANCE



Alan Abraham, President of Granite State Development



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Jonathan C. Roberts, Executive Vice President, Rx Purchasing, Pricing & Network Relations for CVS Caremark



H. Robert Horvitz, Ph.D., Massachusetts Institute of Technology, Nobel Prize in Physiology or Medicine in 2002



Craig C. Mello, Ph.D., University of Massachusetts Medical School, Nobel Prize In Physiology or Medicine in 2006



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