

**Media Contacts:** Robert Goldstein, ALS TDI, 617.441.7295, [rgoldstein@als.net](mailto:rgoldstein@als.net)  
Luke Roberts, Asklepios BioPharmaceutical Inc, 919.968.2727, [lroberts@askbio.com](mailto:lroberts@askbio.com)

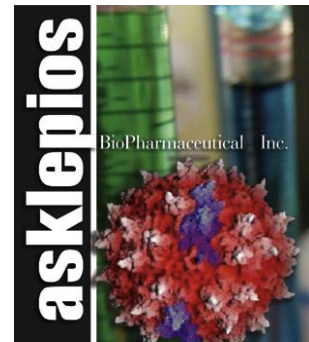
**FOR IMMEDIATE RELEASE**

## ALS TDI and Asklepios to Develop New Therapeutic Delivery Options for Treating Lou Gehrig's Disease

*Collaboration to evaluate gene delivery vectors for ALS*

CAMBRIDGE, Mass. & CHAPEL HILL, N. C. – January 20, 2009 – ALS Therapy Development Institute (ALS TDI) and Asklepios BioPharmaceutical, Inc. announced today their collaboration aimed at developing a panel of viral vectors that could be used to deliver therapeutics based on targets identified by ALS TDI that slow or stop amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease). The collaboration between the two research centers will focus on screening and characterization of optimal delivery vectors.

“This project is an aggressive and proactive effort to develop the safest alternative therapeutic delivery options available today,” reported Dr. Steve Perrin, Chief Scientific Officer at ALS TDI after a recent conference call with more than 140 members of the ALS community, “By focusing on developing therapeutic strategies while operating the largest discovery biology program for this disease, we are preparing to rapidly move potential therapeutics through the drug development process with today's patients in mind.”



The custom constructed Biological Nano Particle (BNP) vectors are designed using proprietary technology developed by Asklepios. ALS TDI will evaluate the BNP vectors in a tightly-managed mouse colony that is based on mutations in a specific mutated protein, which is a cause of a genetic form of disease. BNP vectors are of interest to drug development scientists because they generally do not elicit as aggressive an immune response often associated with other viruses, and have been clinically tested. Samples from animals treated with BNP vectors will be analyzed by a variety of molecular and immunohistochemistry analysis techniques at ALS TDI to determine in which cell types each vector becomes localized in various tissue samples. This project is seen as bolstering tools available as part of a key therapeutic strategy used by ALS TDI to develop effective therapeutics for ALS: viral gene therapy.

“There is a consensus that gene therapeutics are ideally suited to target the largely unmet medical needs of a variety of orphan diseases including ALS. I'm excited that AskBio has formed a partnership with ALS TDI, as it combines our experience in developing gene delivery technology with the expertise and capabilities that ALS TDI have in screening ALS therapeutic leads. Our main objective is to resolve issues that have limited successful gene delivery approaches to ALS in the past, and I look forward with a

great deal of optimism to offering this initial step toward progress for the ALS community,” said Jude Samulski, Ph.D., President and Founder of Asklepios BioPharmaceutical, Inc.

Funding for this project is made possible as part of a major, three-year, \$18 million funding and scientific collaboration between ALS TDI and with the Muscular Dystrophy Association (MDA) and its Augie’s Quest Initiative, entered into at the beginning of 2007. Asklepios BioPharmaceutical, Inc. also receives funding from MDA.

### **About the ALS Therapy Development Institute**

The ALS Therapy Development Institute (ALS TDI) ([www.als.net](http://www.als.net)), based in Cambridge, Massachusetts, operates the world’s largest research and development program focused exclusively on ALS. The Institute has a staff of more than 30 scientists and research technicians, working on behalf of ALS patients to discover and advance therapeutics for treating, and ultimately curing, ALS. The nonprofit biotechnology institute excels in identifying novel disease targets, discovering compounds that may act against these targets, and screening potential treatments for clinical development.

### **About Asklepios BioPharmaceutical, Inc.**

AskBio is a private clinical-stage biotechnology company engaged in the development of novel, gene therapy mediated protein therapies using a proprietary Biological Nano Particle (“BNP<sup>tm</sup>”) technology platform. BNPs may be used to deliver a broad variety of biological material to a cell, including therapeutic genes, monoclonal antibodies, RNAi, and vaccines, among others. The company’s mature intellectual property portfolio is the result of over \$35 million in grants and other funding and is based on the scientific work of Professor R. Jude Samulski & colleagues at the University of North Carolina at Chapel Hill. The company is currently engaged in multiple gene therapy based therapeutic programs. For additional information, visit AskBio’s web site at <http://www.askbio.com>

### **About Augie's Quest**

Fitness pioneer Augie Nieto started Augie's Quest ([www.augiesquest.org](http://www.augiesquest.org)) in conjunction with MDA's ALS Division. Nieto is co-founder and former president of Life Fitness of Chicago, and chairman of Octane Fitness. He and his wife, Lynne, serve as co-chairpersons of MDA's ALS Division. Nieto received a diagnosis of ALS in March 2005.

### **About MDA**

MDA ([www.mda.org](http://www.mda.org)) is the world’s largest provider of ALS services and funder of ALS research. Over the years, it has expended \$230 million in this effort. It operated 225 clinics across the county, 38 of which are ALS-specific research and care centers.

###