



## MeiraGTx Awarded Target ALS Grant For Its NMD-Based Therapy for ALS

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NEW YORK, Feb. 1, 2017 /PRNewswire/ — MeiraGTx, a New York and UK based gene therapy company, announced today that it has been awarded a grant from Target ALS, a privately funded foundation bringing together a consortia of academic and biotech/pharmaceutical researchers focused on finding treatments for ALS patients. Target ALS is jointly funding this grant with the research organization ALS Finding A Cure®. The grant will fund testing of a novel gene therapy approach to treat this devastating neurodegenerative disease that targets TDP-43, a protein that is misregulated in over 95% of all ALS patients. The approach would control misregulated proteins in the cell. Out of 43 applicants for the industry-led consortium funding, MeiraGTx was one of just five awardees. The grant was reviewed by an independent review committee comprised of senior investigators in academia and in the biotech/pharmaceutical industry.

The ALS-associated protein TDP-43 has been emerging as a possible Achilles' Heel in ALS treatment. Research conducted by Dr. Greg Petsko and colleagues at Weill Cornell Medicine and Brandeis University has revealed that one of the cell's own quality control mechanisms, the nonsense-mediated decay (NMD) pathway, appears to be able to alleviate TDP-43 mediated toxicity in cell and animal based disease models of ALS. The research found that increased expression of UPF1, the master regulator of NMD, can significantly protect mammalian motor neurons from TDP-43 mediated toxicity.

The grant was received to confirm and extend initial findings on the efficacy of this therapeutic approach. MeiraGTx will test the approach in well-characterized mouse models of ALS derived in the laboratory of Dr. Neil Shneider at the Columbia College of Physicians and Surgeons, and through the use of biochemical methods defined in the Petsko and MeiraGTx lab will generate candidate biologics that are even more potent in generating the same response.

"Target ALS is pleased to support the MeiraGTx led collaborative consortium," said Manish Raisinghani, President of the Target ALS Foundation. "This award is part of our goal to seed and support ALS drug discovery programs in the pharma and biotech industries to realize new treatments for this devastating disease."

"Joining with Target ALS to increase the number of researchers developing novel treatments for ALS is a great opportunity to advance our ultimate mission to cure this deadly disease," said Peter Foss, President of ALS Finding A Cure®. "We are proud to be a part of this innovative effort."

"We are honored to receive this prestigious grant to help us pursue our promising findings about NMD-based therapy," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx. "We believe this is a key development in the treatment of ALS and that our novel approach may help the thousands of people affected by this devastating neurodegenerative disease."

### About MeiraGTx

MeiraGTx is committed to the development of novel gene therapies to transform the lives of patients suffering from acquired and inherited disorders. The company is developing treatments for ocular diseases, including rare inherited blindness and age-related macular degeneration (AMD). MeiraGTx is also establishing treatments for xerostomia, a frequent and debilitating side effect of radiation treatment used in head and neck cancers, as well as certain neurodegenerative diseases. In addition, MeiraGTx is developing novel gene regulation platforms that promise to transform the way gene therapy can be applied and create new paradigms for biologic therapeutics.

### About Target ALS

Target ALS Foundation ([www.targetals.org](http://www.targetals.org)) is a non-profit organization with the overall goal of accelerating development of new treatments for ALS. We drive emergence of novel ALS drug discovery programs in industry by funding collaborative consortia focused on development of novel therapeutic targets. To ensure that all new ideas get tested, we make essential tools and resources openly available to all – especially young investigators – with no embargo or strings attached.

### About ALS Finding A Cure

ALS Finding A Cure® is dedicated to being a game changer in discovering a cure for this fatal disease. Founded in 2014 by noted philanthropist and Conair Founder and Chairman, Leandro (Lee) Rizzuto, as a tribute to Christie Rizzuto, Lee's daughter-in-law who was diagnosed with ALS in 2009 at the age of 41, and under the leadership of Peter N. Foss and a team of renowned ALS researchers, ALS Finding A Cure® is focused on identifying the gaps in the scientific understanding of ALS that are preventing the development of a cure. The organization, a program of The Leandro P. Rizzuto Foundation, collaborates with a wide range of companies, ALS organizations, other disease research organizations, and ALS patients and families to ensure research efforts are non-duplicative, synergistic, and focused on the ultimate goal: finding a cure. For more information about ALS Finding A Cure®, visit <http://www.alsfindingacure.org>.

### Forward-Looking Statements

*This press release contains forward-looking statements. These forward-looking statements are based on management's expectations and are subject to certain factors, risks and uncertainties that may cause actual results, outcome of events, timing and performance to differ materially from those expressed or implied by such statements. The information contained in this press release is believed to be current as of the date of original issue. MeiraGTx expressly disclaims any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations with regard thereto or any change in events, conditions or circumstances on which any such statements are based.*

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