THE ALS RESEARCH FORUM
Discovering a Common Goal, Discovering a Cure
PRIZE4LIFE ALS ASSOCIATION

ALS Research Forum e-Newsletter Vol. 187 **December 20, 2017**

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Research News

CRISPR: In the Nick of Time for ALS?

The CRISPR/Cas editing system is emerging as a potential approach to correct mutations that cause ALS. But with at least four approaches now being developed, which of these strategies could be the most promising approach for which form of the disease? Experts weigh in.

Check out this feature to learn about potential CRISPR/Cas editing strategies being developed for ALS, including one unveiled at SfN 2017.

An Emerging Test May Help Expedite ALS Diagnosis

Biomarkers may help neurologists diagnose ALS shortly after the first signs of the disease according to a new study led by University of Ulm's **Markus Otto** in Germany (Feneberg et al., 2017) . The study found that key components of neurofilaments in the blood and CSF could help differentially diagnose ALS, even early in the course of the disease (≤ 6 months). The findings add to a growing evidence that this approach may help increase the accuracy of diagnosis, by helping to discriminate ALS from conditions that outwardly resemble the disease (see May 2017 news; Poesen et al., 2017). The findings appeared on December 6 in *Neurology*.

To learn more about these biomarkers for ALS including an emerging test for ALS being developed, check out *Could Neurofilament Proteins Help Clinicians Diagnose ALS?*

Before 40, Brain Atrophy in ALS Mutation Carriers?

The most common form of ALS may be detectable years before the first symptoms of the disease. The study, led by Centre de Référence des Démences Rares ou Précoces' **Isabelle Le Ber** in Paris, France, found, using MRI, that key structural changes could be detected in the brain of people harboring repeat expansions in the C9orf72 gene under 40 years of age. The results open up the possibility that this approach could help diagnose ALS, before the first signs of the disease. The study adds to growing evidence that ALS may be one of many neurodegenerative diseases that may begin decades before the onset of the disease. The study is

published on December 2 in *JAMA Neurology*.

Conference News

A New Knock-In TDP-43 Mouse Enters the ALS Ring

A new mouse model may help researchers identify new targets for ALS according to a preliminary analysis presented at the 47th Annual Meeting of the Society of Neuroscience on November 15 in Washington, D.C. The knock-in mouse, developed by University of Cambridge's **Jemeen Sreedharan** in England in collaboration with University of Massachusetts Medical Center's **Robert Brown**, harbors an ALS-linked Q331K mutation in the RNA-binding protein TDP-43. TDP-43 pathology can be detected in the CNS in more than 95% of cases of ALS. The mouse is the first knock-in model of TDP43-associated disease.

To find out more of the latest advances in ALS unveiled at SfN 2017, check out our wrap-up of potential therapies approaching the pipeline.

Clinical Trial News

Antisense Oligonucleotide Squelches Huntingtin Protein in Phase 1/2a Trial

An antisense oligonucleotide lowers levels of a key disease-linked toxic protein in the nervous system in people with the repeat expansion disorder, Huntington's Disease according to its developer Ionis Pharmaceuticals. A clinical trial is now planned to determine whether this drop in huntingtin could slow progression of the disease. The potential treatment appeared to safe and well-tolerated according to phase 1/2A results according to Ionis Pharmaceuticals. This is the first antisense oligonucleotide to lower levels of a disease-linked protein in the nervous system in people with a neurological disease. The results will be presented at a scientific conference in the first half of 2018.

Washington University School of Medicine's **Timothy Miller**, who is developing a similar strategy for SOD1 ALS (see May 2013 news), shares his perspective.

Check out our website to read more of the latest research advances in ALS.

Funding Opportunities:

Applications are now being accepted by Fight MND for funding for the development of ALS therapies at the preclinical and Phase 1 stage. Researchers globally are invited to apply! Check out their website for details! Deadline: April 1, 2018.

January 2018

ALS ACT: Biomarker Discovery of ALS . ALS Association and ALS Finding A Cure. Letter of Intent due by **January 8, 2018.**

NEW! Rapid Isolation and Characterization of Extracellular Vesicles of CNS Origin. NIH Blueprint Neurotherapeutics Network. Application due by January 22, 2018.

ALS Research Grants. Thierry-Latran Foundation. Abstracts due by January 29, 2018.

Altman Award in Developmental Neuroscience. Japan Neuroscience Society. Application due by January 31, 2017. Scientists worldwide are invited to apply.

February 2018

NEW! Joint Call for European Research Projects on Rare Diseases. E-Rare. Pre-Proposal due by February 6, 2018.

High Impact Neuroscience Research Resource Grants. NINDS. Application due by February 9, 2018.

Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases. Focus: Clinical Outcome Measures and Biomarkers. NINDS. Application due by February 15, 2018.

Klingenstein-Simons Fellowship in Neuroscience. (For Early-Career PIs.) Applications due by February 15, 2018.

Transformational Research: Canada 2018. Weston Brain Institute. Deadline extended! LOI now due by February 15, 2018.

Check out our updated list of grants and awards.

Job Opportunities:

Chair, Neurodegenerative Disorders. University of Sydney. Sydney, Australia.
Professor, Biomedical Sciences. University of Texas - Rio Grande. Edinburg, TX.
Asst. or Assoc. Professor, Computational & Systems Biology. University of PA. Philadelphia, PA.
Assistant or Associate Professor, Neuroscience. University of Florida. Gainesville, FL.
Postdoctoral Fellow. Shirley Ryan AbilityLab. Chicago, IL.
Research Specialist, Southworth Lab. University of California. San Francisco, CA.

Principal Scientist, Neuroscience. ALS. Amgen. Cambridge, MA.

Hiring someone onto your team? Contact us to add your listing to our updated job board: ALSjobs@prize4life.org.

Full List of Job Opportunities >>

Upcoming Meetings:

March 2018

March 11-14, 2018. Arlington, VA. MDA Clinical Conference.

March 18-21, 2018. EMBL. Heidelberg, Germany. Microglia 2018. Abstracts due: February 15, 2018.

March 22-24, 2018. Melbourne, Australia. Australasian Motor Neurone Disease Symposium.

April 2018

April 17-21, 2018. Cold Spring Harbor Laboratory. Cold Spring Harbor, NY. Protein Homeostasis in Health and Disease . Abstracts due: February 2, 2018.

April 21-27, 2018. Los Angeles, CA. Annual Meeting of the American Academy of Neurology.

Organizing an ALS meeting? Contact us to add your conference to our updated calendar: ALSmeetings@prize4life.org.

Full List of Upcoming Meetings>>

Download the Working with ALS Mice Manual Here

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