



ALS Research Forum e-Newsletter Vol. 150

April 29, 2016

Visit the [ALS Research Forum](#) to read the complete stories featured in this e-newsletter. Friends and colleagues can sign up for the newsletter [here](#). Follow us on [Facebook](#) and [Twitter](#) for the latest updates.

Conference News

[Paving a Path for FTD Drug Development at the Frontotemporal Dementia Study Group Workshop](#)

At the Frontotemporal Dementia Study Group Workshop, which took place March 31 - April 1 in Washington, DC, scientists, regulators, funders and the FTD patient community convened to collaboratively strategize on a path for development of FTD therapies. Discussions covered design of drug trials, biomarkers and the regulatory pathway. Here we bring you in depth news on the Workshop through our collaboration with Alzforum.

[Part I: Drug Trials in Frontotemporal Dementia: Can Field Push Forward Together?](#)

Part I provides an overview of FTD-related initiatives and of the current state of knowledge on the complex biology of FTD.

[Part II: WANTED: Biomarkers for Drug Trials in Frontotemporal Dementia](#)

Part II discusses the quest for biomarkers for patient selection, as well as for assessing target engagement and drug effects. Promising candidate fluid and imaging biomarkers are presented.

[Part III: Regulators Tell Frontotemporal Dementia Community: We Play on Your Team](#)

Part III lays out the discussion between representatives of US and European regulatory agencies and drug developers to better define the regulatory pathway for therapies under development for FTD.

AAN 2016: Three Researchers Awarded Prizes for Contributions to ALS/FTD Genetics

At the American Academy of Neurology meeting, held April 15-21 in Vancouver, British Columbia, three ALS researchers were recognized for their significant contributions to the field of ALS and FTD genetics. Rosa Rademakers of the Mayo Clinic in Jacksonville, FL, and Bryan Traynor of the National Institute on Aging were awarded the [2016 Potamkin Prize](#) for their studies in the genetics of neurodegenerative diseases, in particular the discovery of expansions in the C9orf72 gene, which are the

most common genetic cause of ALS and FTD. Ammar Al-Chalabi of King's College London in the UK was awarded the [2016 Sheila Essey Award](#) for key discoveries in ALS genetics, and for playing an important leadership role in large scale genetic studies in ALS and FTD. Read more about the awardees [here](#) and [here](#).

Research News

[Mutations in CCNF Strengthen Link Between ALS and FTD](#)

A new ALS gene has been identified that strengthens the genetic link between ALS and Frontotemporal dementia (FTD). Mutations in CCNF, which encodes the cyclin F protein, were identified by an international team of researchers by combining genetic linkage analysis and exome sequencing on families with a history of ALS, FTD or both. As reported in the April 15 *Nature Communications*, these mutations are found in sporadic ALS as well, and occur with a frequency similar to TDP-43 and FUS mutations. The Cyclin F protein encoded by CCNF is a component of the ubiquitin protease system, and mutations in the protein cause build up of ubiquitinated TDP-43. This new discovery provides additional evidence pointing to abnormal proteostasis in ALS and FTD.

[To Regenerate an Injured Spinal Cord, Add Spinal Cord \(and Ignore the Astrocytic Scar\)](#)

Strategies for repairing the injured spinal cord have leveraged diverse approaches aimed ranging from restoring developmental cues to remove physical barriers to axon growth. Two new papers highlight breakthroughs on both these fronts. In the March 28 *Nature Medicine*, researchers show that grafting caudalized neural stem cells into the site of spinal cord injury (SCI) in rats promotes robust growth of transected axons, regeneration beyond the site of injury, and improvements in motor function. A second study, published in the April 14 *Nature*, shows that the astrocytic scar, long thought to inhibit regeneration after SCI, instead is necessary for repair, a finding that is likely to reorder priorities for development of SCI repair in humans.

Deals and Partnerships

[AC Immune and Biogen Partner to Develop TDP-43 Imaging Biomarkers](#)

Swiss biopharmaceutical company [AC Immune](#) has entered into an R&D collaboration with biotechnology company [Biogen](#) to develop PET radioligands for TDP-43, a protein present in pathological aggregates in the majority of cases of ALS and FTD. AC immune will leverage its chemistry technology platform to design small molecules selective for TDP-43, and jointly with Biogen, these will be further developed and clinically validated for use as PET tracers for TDP-43 proteinopathies (see [April 2016 news](#)).

[Molecular Stethoscope Explores Cell-Free RNA Diagnostics in ALS and Others](#)

Startup company [Molecular Stethoscope](#) has announced a partnership with 23 academic and medical institutions to test and validate their cell-free RNA diagnostics technology in a wide range of neurodegenerative and liver diseases. The company has received over 700 plasma and serum patient samples, including ALS patient

samples. The technology uses rapid analysis of the the cell-free transcriptome in blood samples to detect organ damage. Under this new initiative, the company will apply this approach to detect disease-specific signatures that could be leveraged for diagnosis and drug development. The original technology was developed in the laboratories of Stephen Quake at Stanford University and Eric Topol at Scripps Health ([Koh et al., 2014](#)).

Funding Opportunities:

NEW! [CReATe Biomarker Development/Validation Project](#). Letter of Intent due May 13, 2016.

[NINDS Program Project Grant \(P01\)](#). Applications due May 25, 2016.

NEW! [Treeway Graduate Reward Program](#). Applications due May 31, 2016.

[ALS Canada Discovery Grants](#). Full application due June 3, 2016.

[CIRM Partnering Opportunity for Translational Research Projects](#). Applications due July 15, 2016.

[Full List of Funding Opportunities >>](#)

Upcoming Meetings:

May 2016

May 19-21, 2016: Milan, Italy: [Annual ENCALS Meeting](#).

NEW! May 28-31, 2016: Copenhagen, Denmark: [Congress of the European Academy of Neurology](#).

June 2016

June 5-9, 2016: Whistler, British Columbia: [Keystone Symposia, Autophagy, Molecular & Physiological Mechanisms](#).

NEW! June 12-16, 2016: Keystone, Colorado: [Keystone Symposia, Microglia in the Brain](#).

[Full List of Upcoming Meetings>>](#)

Resources:

[ALS Drugs in Development Database](#)

[ALSGene](#)

[Alzforum ALS Mouse Model Database](#)

[The PRO-ACT Database](#)

[NEALS Biofluid Repository Available to Researchers](#)

[VABBB ALS CNS Tissue Request Information Site](#)

[TargetALS Core Facilities](#)