



ALS Research Forum e-Newsletter Vol. 175

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Drug News

[The Phase III Results for Masitinib Are Now In. But Experts Remain Divided.](#)

Masitinib may be of benefit to some people with ALS according to phase 3 clinical trial results presented on May 18 at the 2017 European Network to Cure ALS (ENCALS) meeting in Slovenia. But experts remain divided on the interpretations of these results and their implications for people with the disease.

Check out [our feature](#) to learn more about masitinib, including the upcoming phase 3 clinical trial which is scheduled for Q3 2017.

Research News

[To Evaluate Stem Cell Therapies, Think Unilaterally?](#)

A growing number of scientists are turning to stem cells in hopes to promote motor neuron survival in people with ALS and/or reduce their toxicity. But how to evaluate these strategies in the clinic remains hotly debated. Now, Cedar Sinai Medical Center's **Clive Svendsen** and **Robert Baloh** in California report that an emerging outcome measure may facilitate the evaluation of stem cell therapies for the disease. The unilateral approach, which involves the side-by-side comparison of the strength of muscles of key upper and lower muscle groups, may enable clinicians to evaluate stem cell transplantation-based strategies in a smaller sample size without the need for sham surgeries and/or placebo injections. The study is published on June 9 in *Neurology*.

[Familial ALS Linked to Both Neuron and Astrocyte Pathology](#)

Motor neurons degenerate in ALS. But why these cells are destroyed in ALS remains unclear. Now, a research team, led by University College London's **Rickie Patani** and **Sonia Gandhi** in England, use live imaging of patient-derived motor neurons to develop a timeline of molecular events that lead to neurodegeneration in VCP-linked ALS. The results may help scientists identify treatment strategies that protect motor neurons in ALS by pinpointing the earliest events that contribute to their destruction. The study is published on May 30 in *Cell Reports*.

[What Makes a Microglia? Tales from the Transcriptome](#)

Microglia are emerging as a critical player in ALS. To elucidate the role of these immune cells in the disease, a growing number of researchers are studying them in culture. But according to a new study led by University of California's **Christopher Glass** in San Diego, these microglia may not be the same cells that contribute to the disease. The study, which compared the transcription profiles of resected human brain microglia before and after culturing, found that more than 1000 genes were either upregulated or downregulated more than 10-fold including nearly 300 genes associated with neurodegenerative diseases. The study adds to growing evidence that the tissue microenvironment may shape the identity and function of microglia in the central nervous system. The results are published on May 25 in *Science*.

Industry News

[Gene Therapy Biotech AveXis Targets SOD1 ALS](#)

AveXis is one step closer to developing a potential gene therapy for SOD1 ALS. The gene therapy company, based in Cleveland, Ohio, announced this month it has obtained the rights to develop treatments for ALS using REGENXBIO's gene therapy delivery vehicle. The emerging vector, derived from adeno-associated virus 9 (AAV9), is being increasingly utilized to deliver potential therapies into the CNS for neurological diseases. The strategy, developed by Nationwide Children's Hospital's **Brian Kaspar** in Ohio, is one of a growing number of potential gene therapies for SOD1 ALS that aims to reduce levels of misfolded SOD1 in the CNS and in the muscles by silencing the expression of the SOD1 gene. >> [Read more.](#)

Check out [our website](#) to read more of the latest research advances in ALS.

Funding Opportunities:

The Weston Brain Institute will be providing [seed funding](#) for high-risk, high-reward projects in the Netherlands and Ireland, including those on FTD. Applications open in October 2017.

July 2017

[Paulo Gontijo Award.](#) The winner presents at the 2017 International Symposium on ALS/MND in Boston, Massachusetts. Register online by **July 1, 2017**.

[Research and Trampoline Grants.](#) AFM-Téléthon. Applicants outside France are encouraged to collaborate with a lab located in France. Application due by **July 4, 2017**.

[Rapid Response 2017.](#) Weston Brain Institute. High-risk high reward translational research. FTD. Canada only. LOI due by **July 5, 2017**.

NEW! [Harrington Rare Disease Scholar Award.](#) In collaboration with Takeda Pharmaceuticals. Focus: Neurology and Gastroenterology. LOI due by July 19, 2017.

[Accelerating Drug Discovery for FTD.](#) Alzheimer's Drug Discovery Foundation and the Association of Frontotemporal Dementia. LOI due by July 31, 2017.

August 2017

[Judith and Jean Pape Adams ALS Research Grants.](#) Judith and Jean Pape Adams Foundation. Application due by August 4, 2017.

[The Betty Laidlaw MND Research Prize.](#) MND Australia. Applications due August 25, 2017.

[MND Research Grants](#). MND Australia. Applications due August 25, 2017.

[Postdoctoral Fellowships](#). MND Australia. Applications due August 25, 2017.

September 2017

[MDA Venture Philanthropy Program](#). Muscular Dystrophy Association. LOI due by September 1, 2017.

[Research Grants](#). Frick Foundation for ALS Research. Application due by September 30, 2017.

Check out our [updated list](#) of grants and awards.

Job Opportunities:

[Assistant Professor, Psychiatry](#). University of California. Irvine, CA.

[Senior Research Investigator](#), MRC Harwell. Harwell, England.

[Research Scientist, Neuroinflammation](#). Krembil Research Institute. Toronto, Canada.

[Neuropathologist](#). University of California. Davis, CA.

[Postdoctoral Fellow, Van Den Bosch Lab](#). VIB. Leuven, Belgium.

[Postdoctoral Fellow, Gao Lab](#). University of Massachusetts Medical Center. Worcester, MA.

[Lab Manager, NCRU](#). University of California. San Francisco, CA.

[Research Specialist, Plotkin Lab](#). University of Pennsylvania. Philadelphia, PA.

[Research Assistant, Neurodegenerative Disease](#). Karolinska Institutet. Stockholm, Sweden.

[Scientist I/II, Neuroimmunology, Biogen](#). 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:

June 2017

June 24-27, 2017. Amsterdam, Netherlands. [3rd Congress of the European Academy of Neurology](#).

June 26-27, 2017. Buckinghamshire, England. [Meeting the Challenges of Modelling Neurodegenerative Disease in Mice](#).

July 2017

July 8-11, 2017. Edinburgh, Scotland. [European Meeting on Glial Cells in Health and Disease](#).

July 23-28, 2017. Stowe, Vermont. [Gordon Research Conference on Amyotrophic Lateral Sclerosis \(ALS\) and Related Motor Neuron Diseases](#). **Abstracts due: June 25, 2017.**

September 2017

September 7-9, 2017. Ottawa, Canada. [Ottawa International Conference on Neuromuscular Disease and Biology](#). Abstracts due: July 15.

September 16-21, 2017. Kyoto, Japan. [World Congress on Neurology](#).

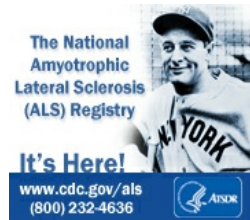
September 17-19, 2017. Sitges, Spain. [Neuro-Immune Axis: Reciprocal Regulation in Development, Health and Disease](#).

October 2017

October 3-5, 2017. Clearwater Beach, Florida. [The 16th Northeast ALS Consortium Meeting](#). Abstracts due: August 4.

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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