

## Clinical Research in ALS & Related Disorders for Therapeutic Development

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### Journal Articles

1. Turner MR, Benatar M. Ensuring continued progress in biomarkers for amyotrophic lateral sclerosis. *Muscle Nerve*. Jan 2015;51(1):14-18. PMID: 25288265, PMCID: PMC4270289
2. Rossor AM, Oates EC, Salter HK, et al. Phenotypic and molecular insights into spinal muscular atrophy due to mutations in BICD2. *Brain*. Feb 2015;138(Pt 2):293-310. PMID: 25497877, PMCID: PMC4306822
3. Gendron TF, van Blitterswijk M, Bieniek KF, et al. Cerebellar c9RAN proteins associate with clinical and neuropathological characteristics of C9ORF72 repeat expansion carriers. *Acta Neuropathol*. Oct 2015;130(4):559-573. PMID: 26350237, PMCID: PMC4575385
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6. Liu Y, Pattamatta A, Zu T, et al. C9orf72 BAC Mouse Model with Motor Deficits and Neurodegenerative Features of ALS/FTD. *Neuron*. 2016;90(3):521-534. PMID: 27112499
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- 12.** Gendron TF, Daugherty LM, Heckman MG, et al. Phosphorylated neurofilament heavy chain: A biomarker of survival for C9ORF72-associated amyotrophic lateral sclerosis. *Ann Neurol.* 2017;82(1):139-146. PMID: 28628244
- 13.** Jacquier A, Delorme C, Belotti E, et al. Cryptic amyloidogenic elements in mutant NEFH causing Charcot-Marie-Tooth 2 trigger aggregates formation and neuronal death. *Acta neuropathologica communications.* 2017;5(1):55. PMID: 28709447, PMCID: PMC5513089
- 14.** Mackenzie IR, Nicholson AM, Sarkar M, et al. TIA1 Mutations in Amyotrophic Lateral Sclerosis and Frontotemporal Dementia Promote Phase Separation and Alter Stress Granule Dynamics. *Neuron.* 2017;95(4):808-816.e809. PMID: 28817800
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