Blocking blood-brain barrier proteins may improve ALS drugs' effectiveness

1 December 2014

Through research in mice, scientists have found that proteins at the blood-brain barrier pump out riluzole, the only FDA-approved drug for ALS, or Lou Gehrig's disease, limiting the drug's effectiveness. However, when the investigators blocked these proteins, the effectiveness of riluzole increased and the animals experienced improved muscle function, slower disease progression, and prolonged survival.

The findings suggest that blocking these transporter proteins at the blood-brain barrier might improve delivery, and ultimately, efficacy, of drugs used to treat ALS and other brain disorders.

"This is a proof-of-principle study that sheds light into a basic pathological mechanism at play in ALS that negatively affects our ability to deliver drugs efficiently," said Dr. Piera Pasinelli, senior author of the *Annals of Clinical and Translational Neurology* study.

"If the findings hold true in clinical trials, we'll have the opportunity not only to improve the efficacy of an already approved ALS drug, but also to design better therapeutic strategies moving forward."

More information: Jablonski, M. R., Markandaiah, S. S., Jacob, D., Meng, N. J., Li, K., Gennaro, V., Lepore, A. C., Trotti, D. and Pasinelli, P. (2014), Inhibiting drug efflux transporters improves efficacy of ALS therapeutics. *Annals of Clinical and Translational Neurology*. DOI: 10.1002/acn3.141

Provided by Wiley