

## Study reveals that a drug used in the treatment of epilepsy may be a promising therapy for Machado-Joseph's Disease



A study carried out at the Center for Neuroscience and Cell Biology at the University of Coimbra (CNC-UC) reveals that carbamazepine, a drug used in the treatment of epilepsy, may be a promising therapy for Machado-Joseph disease.

Coordinated by Luís Pereira de Almeida, professor at the Faculty of Pharmacy of the University of Coimbra (FFUC) and principal investigator at CNC-UC, and by Ana Ferreira, from CNC-UC, this work is already published in the scientific journal *Neuropathology and Applied Neurobiology*.

Machado-Joseph disease, also known as type 3 spinocerebellar ataxia, is an incurable hereditary neurodegenerative disease caused by a change in a gene. This alteration gives rise to a mutated form of the ataxin-3 protein that tends to accumulate in the form of aggregates in the brain, also leading to neuronal dysfunction and death. The disease causes problems with gait, balance, speech, swallowing, eye movements and sleep.

Carbamazepine is an approved drug for other pathological indications, including epilepsy, and therefore, it can be quickly reused to treat other diseases, which turns out to be beneficial. In order to find new pharmacological therapies to treat Machado-Joseph disease, researchers tested the potential of this drug as a promoter of autophagy (abnormal protein elimination mechanism), through *in vitro* and *in vivo* models of the disease.

The team decided to test this drug after realizing, **“in previous work, that the autophagy pathway is dysregulated in Machado-Joseph disease, meaning that the mutant protein is not effectively eliminated. On the other hand, we observed that the stimulation of autophagy, through a gene therapy strategy, promoted neuroprotection in disease models”**, explains Ana Ferreira, the first author of the scientific article.

In experiments carried out with mice, **“after the administration of carbamazepine, in a specific treatment regimen, we realized that the drug was able to increase autophagy and promote the degradation of the mutant protein. In addition, the treatment reduced motor deficits and neuropathology in animal models of disease”**, highlights the CNC researcher.

In addition to Luís Pereira de Almeida and Ana Ferreira, Sara Carmo-Silva, José Miguel Codêso, Patrick Silva and Clévio Nóbrega, also CNC researchers, and Alberto Martinez and Marcondes França Jr., from the Faculty's Department of Neurology, also participated in the study. of Medical Sciences at the State University of Campinas (Brazil).

By demonstrating that the drug carbamazepine can be useful to treat Machado-Joseph disease, and possibly other similar diseases, this study represents **“a step forward in the search for an effective treatment for this neurodegenerative disease, although it is far from being a cure”**, conclude the authors of the study.

The scientific article is available at: <https://doi.org/10.1111/nan.12763>

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