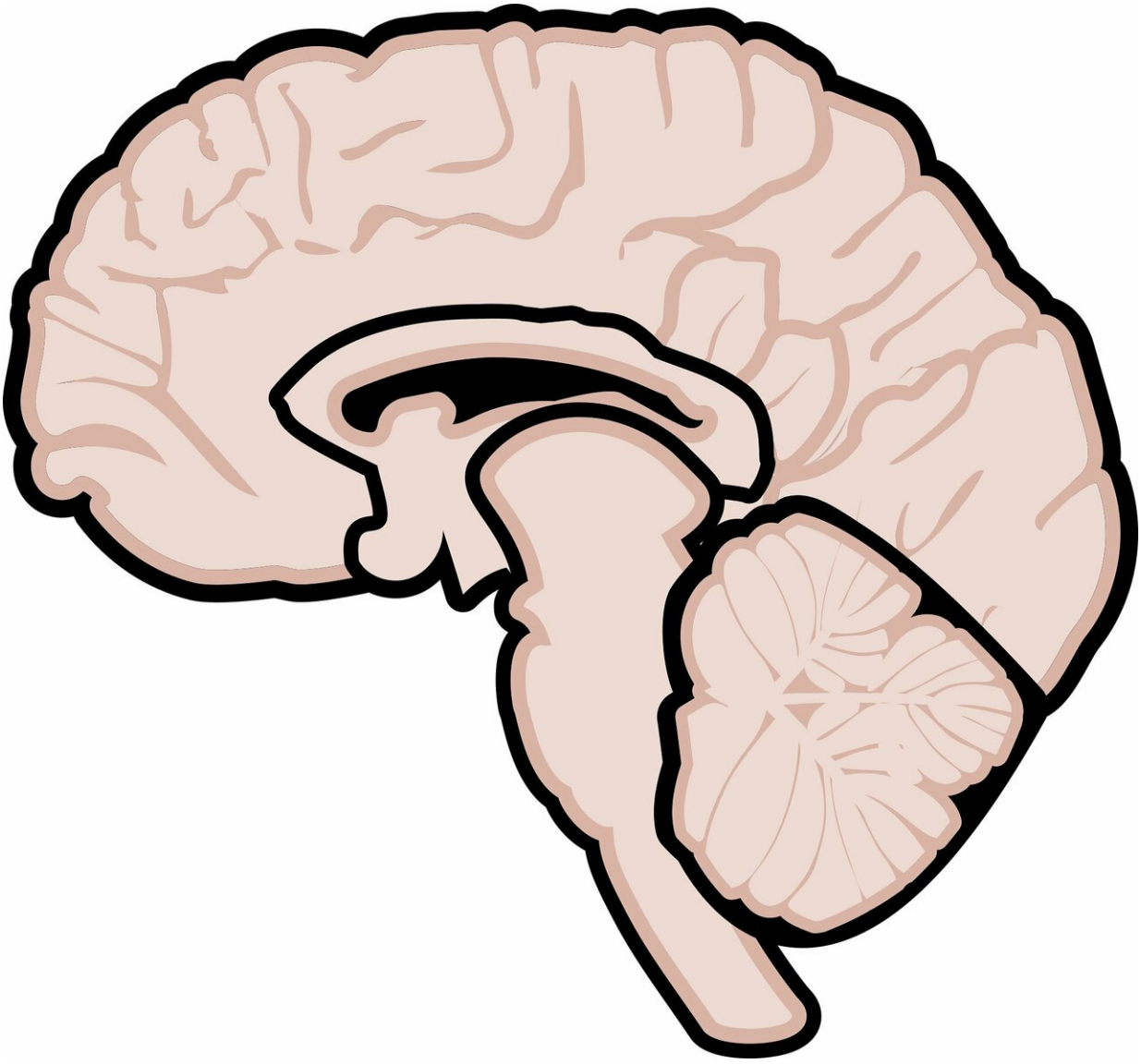
Clinical trial yields fewer relapses in multiple sclerosis patients treated with off-label drug

July 13 2022



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Patients with multiple sclerosis (MS) treated with the drug rituximab had a significantly lower risk of relapse compared with MS patients receiving standard treatment. This has been shown in a phase 3 clinical trial by researchers at Karolinska Institutet and Danderyd Hospital in Sweden published in *The Lancet Neurology*. Rituximab is not approved as an MS drug, but has proven to be effective in smaller studies and is therefore largely prescribed "off label."

The Phase 3 clinical trial is a multi-center study involving 195 patients from 17 hospitals in Sweden who were newly diagnosed with the most common form of MS, relapsing-remitting MS. Patients were randomly given either rituximab (Mabthera) or standard dimethyl fumarate (Tecfidera) treatment. During the 24-month follow-up, the occurrence of relapses, i.e., a temporary deterioration of the disease state, was investigated.

The results showed that those treated with rituximab had a five-fold lower risk of relapse. Only three out of 98 patients who received rituximab suffered relapses, compared to 16 out of 97 patients who received dimethyl fumarate. Magnetic resonance imaging (MRI) also showed that those who received rituximab had fewer new MS plaques, i.e., areas of damage or scarring in the central nervous system. No increased risk of adverse effects with rituximab was observed.

"The excellent efficacy and low cost of rituximab could make it an attractive first choice for newly diagnosed MS patients, not least in resource-poor areas. But more and larger studies are needed to confirm the drug's efficacy, long-term safety and cost-effectiveness for MS," says the study's first author Anders Svenningsson, adjunct professor at the Department of Clinical Sciences, Danderyd Hospital, Karolinska Institutet and chief physician at the neurology clinic at Danderyd

Hospital.

Rituximab is used for a variety of medical conditions but is not approved for the treatment of MS because there has been a lack of data from phase 3 clinical trials. However, the drug has been shown to have a good effect on relapsing-remitting MS and is therefore often prescribed off label, which means that the treating doctor alone assumes responsibility for the treatment.

"Since the patent has expired, there is no incentive from the pharmaceutical company holding the marketing rights to apply for a new indication. But now, in addition to accumulated clinical experience, we also have the documentation that is usually required to apply for an indication. Our study is an important step on the way for rituximab to become an approved MS drug," says Anders Svenningsson.

More information: Safety and efficacy of rituximab versus dimethyl fumarate in patients with relapsing-remitting multiple sclerosis or clinically isolated syndrome in Sweden: a rater-blinded, phase 3, randomised controlled trial, *The Lancet Neurology* (2022). DOI: 10.1016/PIIS1474-4422(22)00209-5 , www.thelancet.com/journals/lan … (22)00209-5/fulltext

Provided by Karolinska Institutet