Nature Reviews Neurology | Published online 23 Oct 2017

IN BRIEF

NEUROMUSCULAR DISEASE

Tadalafil fails to halt the progression of Duchenne muscular dystrophy

Tadalafil, a drug that seems to improve blood flow in skeletal muscle during exercise by boosting nitric oxide–cGMP signalling, has shown promise in preclinical models of Duchenne muscular dystrophy (DMD). However, a new phase III trial found little evidence of benefit in the clinic. The trial included 331 individuals with DMD aged 7–14 years, who were randomly assigned to receive 0.3 mg/kg or 0.6 mg/kg tadalafil or a placebo daily for 48 weeks. Participants were assessed on the 6-minute walk distance (6MWD) and the Performance of Upper Limb (PUL) test at baseline and at 12-week intervals. Tadalafil did not prevent a decline in performance on the 6MWD; however, the lower dose of the drug was associated with some stabilization of PUL scores in boys aged >10 years. The investigators suggest that the effects of tadalafil on upper limb function warrant further exploration.

ORIGINAL ARTICLE Victor, R. G. *et al.* A phase 3 randomized placebo-controlled trial of tadalafil for Duchenne muscular dystrophy. *Neurology* http://dx.doi.org/10.1212/WNL.0000000000004570 (2017)

MOVEMENT DISORDERS

Patients with spinocerebellar ataxia 38 benefit from DHA supplementation

Dietary supplementation with docosahexaenoic acid (DHA) — an omega-3 fatty acid that is essential for brain development and function — produces symptomatic improvements in patients with spinocerebellar ataxia 38 (SCA38), according to an open-label trial published in *Annals of Neurology*. SCA38 is caused by mutations in *ELOVL5*, which encodes an enzyme that is involved in DHA synthesis. Ten patients with SCA38, all of whom had already participated in a 16-week randomized controlled trial of DHA supplementation, took DHA capsules daily for a further 40 weeks. At the end of this period, the participants showed clinical improvements on two different ataxia rating scales, as well as amelioration of the cerebellar hypometabolism that is observed in individuals with SCA38.

ORIGINAL ARTICLE Manes, M. *et al.* Docosahexaenoic acid (DHA) is a beneficial replacement treatment for spinocerebellar ataxia 38 (SCA38). *Ann. Neurol.* http://dx.doi.org/10.1002/ana.25059 (2017)

PARKINSON DISEASE

Caffeine and nicotine do not provide symptomatic relief in Parkinson disease

Coffee drinking and cigarette smoking have been linked to a reduced risk of Parkinson disease (PD), but recent clinical trials indicate that neither caffeine nor nicotine can relieve the symptoms of this condition. In the Café-PD trial, 121 patients with PD were randomly assigned to receive 200 mg caffeine or placebo capsules twice daily for up to 18 months. The caffeine treatment did not significantly improve the motor manifestations of PD, as assessed on the United PD Rating Scale (UPDRS). In the other trial, which involved 40 patients with PD, half of the participants received transdermal nicotine therapy; the treatment produced no significant improvements in UPDRS scores over a 39-week period. These findings indicate that the inverse relationship between caffeine and nicotine intake and PD risk is unlikely to be attributable to symptomatic effects.

ORIGINAL ARTICLES Postuma, R. B. *et al.* Caffeine as symptomatic treatment for Parkinson disease (Café-PD): a randomized trial. *Neurology* http://dx.doi.org/10.1212/WNL.0000000000004568 (2017) | Villafane, G. *et al.* High-dose transdermal nicotine in Parkinson's disease patients: a randomized, open-label, blinded-endpoint evaluation phase 2 study. *Eur. J. Neurol.* http://dx.doi.org/10.1111/ene.13474 (2017)