Nature Reviews Neurology | Published online 19 Mon 2017; doi:10.1038/nrneurol.2017.76

In the news

CLINICAL TRIALS EXCITE AT AAN 2017

Over 13,000 neurologists from around the globe gathered for the 69th American Academy of Neurology (AAN) Annual Meeting in Boston, Massachusetts, USA from 22–28th April 2017. Over 2,700 abstracts were presented, and results from several clinical trials generated much excitement.

One highlight was data from two phase III clinical trials in patients with spinal muscular atrophy (SMA): ENDEAR and CHERISH. The trials demonstrated the safety and efficacy of nusinersen, an antisense oligonucleotide that modulates splicing of *SMN2* and increases the production of functional survival motor neuron protein (SMN) in patients with SMA. Children with SMA who received the drug were more likely to achieve motor milestones, such as sitting, crawling and walking, than were controls who received a sham injection. Nusinersen is now the first FDA-approved drug for treatment of SMA, but the high estimated cost of the therapy and its intrathecal method of administration were the subjects of lively discussion. Findings were also presented from the phase I trial of AVXS-101 — a new viral gene therapy for SMA that introduces a fully functional human SMN gene into patients — which showed a good safety profile and promising improvements in the achievement of motor milestones by children with SMA.

Several other advances in paediatric neurology were highlighted at the meeting, including data from the GWPCARE1 trial, which showed that cannabidiol treatment in children with Dravet syndrome reduced seizure frequency compared with placebo treatment. Results from the CHAMP trial for paediatric migraine were also discussed; the trial tested the efficacy of amitriptyline and topiramate (antidepressants often prescribed to treat paediatric migraine) but found no benefit over placebo. The trial raises difficult questions about how children who have migraines should be cared for in the absence of effective therapies.

Encouraging results from two phase III trials of treatment for chronic migraine in adults were also presented. The STRIVE and ARISE trials monitored the effect of erenumab, a human monoclonal antibody against the calcitonin gene-related peptide receptor (CGRP). During the discussion of the trials, plenary speaker Peter Goadsby remarked that "blockade of CGRP will be the first mechanism-specific, migraine-preventative treatment ever". Erenumab was found to reduce the number of monthly migraine days in recipients, compared with a placebo control, and showed limited adverse effects, in contrast to the non-mechanism-specific treatments normally given to patients with migraine.

Finally, new AAN guidelines on sudden unexpected death in epilepsy (SUDEP) were also presented at the meeting. The researchers found that generalized tonic–clonic seizures are a major risk factor for SUDEP and that individuals who have three or more of this type of seizure a year are 15 times more likely to undergo SUDEP than those who have fewer than three seizures a year. The guidelines highlight the importance of active management of epilepsy therapies to control seizures in patients.

Charlotte Ridler