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AVXS-101 GENE-REPLACEMENT THERAPY (GRT) FOR SPINAL MUSCULAR ATROPHY TYPE 1 (SMA1): PIVOTAL PHASE 3 STUDY (STR1VE) UPDATE

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10.1136/jnnp-2019-anzan.58

Introduction SMA1 is a neurodegenerative disease caused by bi-allelic survival motor neuron 1 gene (*SMN1*) deletion/mutation. In the phase 1 study, *SMN* GRT onasemnogene abeparvovec (AVXS-101) improved outcomes of symptomatic SMA1 patients. We report preliminary data of STR1VE, a pivotal study (NCT03306277) evaluating efficacy and safety of a one-time intravenous AVXS-101 infusion.

Methods STR1VE is a phase 3, multicenter, open-label, single-arm study in SMA1 patients aged <6 months (bi-allelic *SMN1* loss, 2x*SMN2*). Primary outcomes: independent sitting for ≥30 seconds (18 months) and survival (14 months). Secondary outcomes: ability to thrive and ventilatory support (18 months). Exploratory outcomes: Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) and Bayley Scales of Infant and Toddler Development scores.

Results Enrollment is complete with 22 patients dosed. Mean age at symptom onset, genetic diagnosis, and enrollment was 1.9 (0–4.0), 2.1 (0.5–4.0), and 3.7 (0.5–5.9) months. At baseline, no patient required ventilatory/nutritional support, and all exclusively fed by mouth. Mean baseline CHOP-INTEND score was 32.6 (17.0–52.0), which increased 6.9 (–4.0–16.0, n=20), 10.4 (2.0–18.0, n=12), and 11.6 (–3.0–23.0, n=9) points at 1, 2, and 3 months. Updates will be provided at the congress.

Conclusions Preliminary data from STR1VE show rapid motor function improvements in SMA1 patients, paralleling phase 1 findings.

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BURDEN OF MIGRAINE IS AUSTRALIA: A SYSTEMATIC LITERATURE REVIEW

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10.1136/jnnp-2019-anzan.59

Introduction Migraine is a disabling neurological disease characterised by recurrent attacks of moderate to severe headaches. This systematic literature review (SLR) aimed to investigate the clinical, humanistic, and economic burden of chronic migraine (CM), episodic migraine (EM), and of current preventive migraine treatments in Australia.

Methods The methodology of this SLR was aligned with the National Institute for Health and Care Excellence (NICE) guidelines. An electronic database search was conducted in Embase, MEDLINE and the Cochrane Library, with a time frame of 2008 to 2018.

Results In total, 1,122 records were identified and 168 of these were included for data extraction. The prevalence of migraine in Australia is estimated at 18.9%. Of those, 44% of people with EM and 86% of people with CM reported moderate-to-severe disability. Over one-third (36%) of people with EM and nearly two-thirds (64%) of people with CM reported visiting a healthcare provider in the previous three months. No data relating to the economic burden of migraine were returned by the searches. In people with EM and CM, anti-calcitonin gene-related peptide (anti-CGRP) preventive treatments for migraine safely, effectively and significantly reduced the mean number of monthly migraine and/or headache days from baseline compared with placebo.

Conclusions Migraine is associated with a substantial burden, and people living with migraine feel the impact in their day-to-day lives. Anti-CGRPs are a promising class of preventive treatments for all people with migraine. Longer-term studies are needed to determine if the positive effects of anti-CGRPs are sustained over greater time periods.

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SIGNAL RECOGNITION PARTICLE ANTIBODY ASSOCIATED NECROTIZING MYOSITIS WITH 'BURNT-OUT' PARAVERTEBRAL MUSCLE ATROPHY

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10.1136/jnnp-2019-anzan.60

Introduction Necrotizing autoimmune myositis (NAM) is an increasingly recognised myositis.¹ While diagnosis is primarily from muscle pathology, antibodies to signal recognition particle (SRP) or 3-hydroxy-3-methylglutaryl-coenzyme A reductase (HMGCR) are also associated.

Case A 67 year old woman presented with proximal weakness and elevated creatine kinase (CK) levels following a complicated AMI, CABG and commencement of atorvastatin. Muscle biopsy confirmed necrotizing myositis and SRP (not HMGCoAR) antibodies were positive. Recovery and rehabilitation was slow and her CK did not normalise for 12 months. She was treated with immunotherapy including intravenous

immunoglobulin, oral and intravenous corticosteroids, azathioprine, and mycophenolate.

Residual hip, abdominal and truncal weakness remained, affecting her gait and posture. CK levels during this period remained normal. MRI demonstrated extensive paravertebral muscle loss with fatty replacement without oedema. Small myopathic units without active features were present on EMG.

Conclusion This case illustrates several important aspects of NAM, which remains a rare disorder. Recovery can be incomplete in up to 40%, especially if control is delayed.² In our opinion, normalisation of CK is an important target and may be necessary for full recovery, and rising CK may predict relapse.³ We suggest MRI and EMG of weak muscles may assist in distinguishing persisting myositis from 'burnt-out' disease, clarifying whether immunotherapy should be increased or not. However relapse on weaning immunosuppression remains frequent. While SRP antibodies in patients who had also taken statins has been previously reported, the quick sequential onset in this case suggests possible causality.

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073 ACUTE SPINAL INFARCTION: TREATMENT AND OUTCOMES WITH AND WITHOUT HYPERBARIC OXYGEN THERAPY AT FIONA STANLEY HOSPITAL

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10.1136/jnnp-2019-anzan.61

Introduction Spinal cord infarction is a potentially devastating disorder commonly presenting with anterior spinal artery syndrome developing over minutes to hours, accounting for an estimated 1% of stroke presentations. Aetiologies range from aortic surgical complications, systemic hypotension and other rare causes including fibrocartilaginous embolism and vascular malformations. Diagnosis is clinical combined with restriction on diffusion-weighted MRI. There are no specific treatment guidelines. The evidence for hyperbaric oxygen therapy (HBOT) in acute spinal infarction is mixed. This case series describes ten cases of acute spinal infarction at Fiona Stanley Hospital (FSH), half of which received HBOT.

Methods Data for all MRI-proven spinal cord infarctions at FSH between 2014 and 2018 were reviewed.

Results Ten patients, median age 55years (31–74), 60% male. Aetiologies: three fibrocartilaginous emboli, five likely atheroembolic disease, one antiphospholipid syndrome, one cryptogenic. Sixty percent presented with flaccid paraplegia ranging from levels C4 to T11. Five patients received HBOT within a median time of 31 hours from symptom onset, with average 15 treatments. Three patients received triple therapy of HBOT, pentoxifyline and high-volume lumbar-puncture

drainages and had median MRC muscle power of 5- on discharge, compared with 2+ power in those who did not. Nine required inpatient rehabilitation, of average eight weeks' duration, with median disability at discharge of 3 on the Modified Rankin Scale.

Conclusion Spinal infarctions can be severely disabling. Fibrocartilaginous embolism may be more common than previously thought. More research is needed on the role of acute triple therapy with pentoxifyline, spinal drains and HBOT given the marked difference in this case series.

074 EARLY AUSTRALIAN EXPERIENCE WITH ERENUMAB FOR CHRONIC MIGRAINE

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10.1136/jnnp-2019-anzan.62

Introduction Erenumab is available in Australia since October 2018. We review the effectiveness and safety in 2 Australian headache centres.

Methods Erenumab (70 mg or 140 mg) was prescribed. We monitored headache days, migraine days, analgesic use, adverse reactions, Headache Impact Test-6 (HIT6) score and Migraine Disability Assessment (MiDAS) score, at baseline and at 3 months. Primary outcomes were reduction in headache and migraine days, and adverse effects. Secondary outcomes were improvement in functional scores and analgesic use.

Results 65 patients (ages 18–73; mean 44 years) commencing Erenumab were assessed before and after 3 monthly treatments. The duration of chronic migraine (CM) ranged from 1 to 40 years, with 3 to 16 previous failed prophylactic treatments.

There was a >50% response in overall headache days and migraine days in 29% (19/65) and 46% (27/59), respectively. There was a modest (10–49%) response in overall headache days and migraine days in 29% (19/65) and 27% (18/59), respectively. There was no improvement in headache days and migraines in 42% (27/65) and 27% (14/59), respectively.

At onset, the mean HIT-6 and MiDAS scores were 66 and 65, decreasing after 3 treatments to 59 and 32, respectively. The mean monthly days taking triptan and codeine medications reduced from 9 and 6 days, to 5 and 3 days, respectively.

There were few reported side effects.

Conclusion This Australian cohort in tertiary referral refractory migraine patients achieved a significant rate of reduced headache and migraine days with good safety and tolerability.

075 A CASE OF SPONTANEOUS CSF RHINORRHEA IN A PATIENT WITH MARFAN SYNDROME

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10.1136/jnnp-2019-anzan.63

Introduction Spontaneous cerebrospinal fluid leak is uncommon condition and frequently associated with hereditary