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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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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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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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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