Results In the first calendar 12 months of operation, the Melbourne MSU operated 30.5 service weeks and provided prehospital thrombolysis (tPA) to n=52 patients (44% of eligible infarcts) and directed n=33 patients for endovascular thrombectomy, of which 48% required bypass from the closest non-thrombectomy hospital. The overall median onset-to-tPA for MSU patients was 97.5 mins compared to the Australian metropolitan median of 150 mins. Thrombolysis in the first 'golden hour' increased to 13.5% from 3.3% in-hospital. Median onset-to-groin for MSU patients receiving EVT was 162 mins compared to 234 mins from historical controls.
Discussion Prehospital treatment and triage using the Mobile Stroke Unit in metropolitan Melbourne resulted in substantial improvements in commencement of reperfusion therapy. Workflow times are approximately halved for thrombolysis and endovascular thrombectomy respectively. Prehospital thrombolysis also allowed a >400% increase in the proportion of treatment in the first 'golden hour'.

012 MECHANICAL THROMBECTOMY IN PEDIATRIC STROKE: SYSTEMATIC REVIEW, INDIVIDUAL PATIENT-DATA META-ANALYSIS, AND CASE SERIES

[1,2]Kartik Bhatia, [1]Hans Kortman, [3,4]Chris Blair*, [2]Geoffrey Parker, [2]David Brunacci, [4,2]Tim Ang, [5]John Worthington, [6]Prakash Muthusami, [1]Timo Krings. *[1]Department of Interventional Neuroradiology, Toronto Western Hospital, Toronto, Ontario, Canada; [2]Department of Interventional Neuroradiology, Royal Prince Alfred Hospital, Sydney, NSW, Australia; [3]Department of Neurology, Liverpool Hospital, Liverpool, NSW, Australia; [4]Department of Neurology, Royal Prince Alfred Hospital, Camperdown, NSW, Australia; [5]Department of Neurology, Royal Prince Alfred Hospital, Sydney, NSW, Australia; [6]Department of Interventional Radiology, The Hospital for Sick Children, Toronto, Ontario, Canada*

10.1136/jnnp-2019-anzan.12

Introduction In adults, there is strong evidence demonstrating the superiority of mechanical thrombectomy (MT) plus intravenous thrombolysis over thrombolysis alone for the treatment of acute ischemic stroke due to large vessel occlusion (LVO). The role of MT in the paediatric stroke population is less clear. Here we present an updated systematic review addressing the use of MT in paediatric patients, including three cases from our centre in Sydney, Australia. We have also completed an individual participant data (IPD) meta-analysis of clinical and angiographic outcomes based on these results.
Method Our systematic review and IPD meta-analysis was performed according to PRISMA-IPD (Preferred Reporting Items for Systematic Reviews and Meta-Analyses: Individual Participant Data) guidelines. Primary outcomes measures were change in NIHSS (National Institute of Health Stroke Scale) score following MT, and mRS (modified Rankin Scale) score at final reported follow-up. The secondary outcome measure was final angiographic result using the mTICI (modified Treatment in Cerebral Ischemia) scale.
Results MT resulted in good long-term neurological outcomes (mRS 0–2) in 60/67 cases (89.6%;follow-up timing μ=4.1 months: 95%CI 2.9–5.3), good short-term neurological outcomes (reduction in NIHSS by 8 or more points or post-MT NIHSS of 0–1) in 37/52 cases (71.2%), and successful recanalization (mTICI 2b/3) in 57/67 cases (85.1%).
Conclusions
In paediatric patients, MT is an effective treatment for ischaemic stroke due to LVO. In the absence of a dedicated prospective registry and with randomized control trails unfeasible, this report represents the best available evidence for the use of MT in the paediatric setting.

013 THE IMPACT OF AGGRESSIVE BLOOD PRESSURE MANAGEMENT IN THE POST-THROMBOLYSIS SETTING

[1]Bethan Harper*, [2]Harry McNaughton, [3]Anna Ranta. *[1]Neurology, Capital and Coast District Health Board and University of Otago, Wellington, New Zealand; [2]Medical Research Institute of New Zealand, Wellington, New Zealand; [3]Capital and Coast District Health Board and University of Otago, Wellington, Wellington, New Zealand*

10.1136/jnnp-2019-anzan.13

Introduction High blood pressure (BP) post-thrombolysis has been associated with an increased rate of bleeding and poorer outcome. We noted frequent BPs of >180 mmHg with a target of keeping BP <180. We tested whether a more aggressive target of SBP <160 mmHg would result in fewer BP protocol violations.
Methods Patients were prospectively captured comparing patients thrombolysed during the 12 months before and 12 months following the introduction of a new more aggressive BP protocol, allowing for a 6 month transition period. Results were adjusted for baseline function and stroke severity using regression analysis.
Results Pre-protocol change 68 and post- 100 patients were thrombolysed. Baseline characteristics were similar between groups. There was a trend for a lower rate of SBPs >180 mmHg (adjusted OR 0.49; 95% CI 0.31–1.1; p=0.097) and a significantly higher rate of SBPs <120 mmHg (adjusted OR 3.06; 95% CI 1.52–6.17; p=0.002) in the aggressive BP protocol group; although events of extreme SBPs (>200 and <100 mmHg) were similar between groups. Favourable outcomes (mRS = 0–2) at 3 months were similar between groups (adjusted OR 1.27; 95% CI 0.58–2.8; p=0.56) as was the rate of symptomatic haemorrhages (adjusted OR 1.26; 95% CI 0.28–5.7; p=0.76). Model fit was improved by adding study group to the model.
Conclusions More aggressive post-thrombolysis BP management lowered the overall BP, but did not result in improved patient outcomes. Potential explanations include a small sample size, reduced cerebral perfusion off-setting reduced bleeding risk, or high BP being merely an epiphenomenon of worse outcome rather than causative.

014 AVXS-101 GENE-REPLACEMENT THERAPY (GRT) IN PRESYMPTOMATIC SPINAL MUSCULAR ATROPHY (SMA): STUDY UPDATE

[1]Michelle Farrar*, [2]Kathryn J Swoboda, [3]Meredith Schultz, [4]Hugh McMillan, [5]Julie Parsons, [6]Ian E Alexander, [3]Elaine Kernbauer, [3]Marcia Farrow, [3]Francis G Ogrinc, [3]Douglas E Feltner, [3]Bryan E McGill, [3]Sidney A Spector, [3]James L'Italien, [3]Douglas M Sproule, [7]Kevin A Strauss. *[1]Department of Neurology, Sydney Children's Hospital, Randwick, NSW, Australia; [2]Department of Neurology, Massachusetts General Hospital, Boston, MA, USA; [3]AveXis, Inc., Bannockburn, IL, USA; [4]Department of Pediatrics, Canada Children's Hospital of Eastern Ontario, Ontario, Ottawa, Canada; [5]Department of Neurology, Children's Hospital Colorado, Aurora, CO, USA; [6]Gene Therapy Research Unit, Children's Medical Research Institute and The Children's Hospital at Westmead, Sydney, NSW, Australia; [7]Clinic for Special Children, Strasburg, PA, USA*

10.1136/jnnp-2019-anzan.14

Introduction SMA is a neurodegenerative disease caused by biallelic deletion/mutation of the survival motor neuron 1

gene (*SMN1*). Copies of a similar gene (*SMN2*) modify disease severity. In a phase 1 study, *SMN* GRT onasemnogene abeparvovec (AVXS-101) improved outcomes of symptomatic SMA patients with two *SMN2* copies (2x*SMN2*) dosed ≤6 months. Because motor neuron loss can be insidious and disease progression is rapid, early intervention is critical. This study evaluates AVXS-101 in presymptomatic SMA newborns.

Methods SPR1NT is a multicenter, open-label, phase 3 study (NCT03505099) enrolling ≥27 SMA patients with 2–3x*SMN2*. Asymptomatic infants ≤6 weeks receive a one-time intravenous AVXS-101 infusion (1.1×10^{14} vg/kg). Safety and efficacy are assessed through study end (18 [2x*SMN2*] or 24 months [3x*SMN2*]). Primary outcomes: independent sitting for ≥30 seconds (18 months [2x*SMN2*]) or assisted standing (24 months [3x*SMN2*]).

Results From April–September 2018, 7 infants received AVXS-101 (4 female; 6 with 2x*SMN2*) at ages 8–37 days. Mean baseline Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) score was 41.7 (n=6), which increased by 6.8, 11.0, 18.0, and 22.5 points at day 14 (n=4), month 1 (n=3), 2 (n=3), and 3 (n=2). As of January 31, 2019, 15 asymptomatic infants have been enrolled in SPR1NT and dosed with AVXS-101. Updated data available at the time of the congress will be presented.

Conclusions Preliminary data from SPR1NT show rapid motor function improvements in presymptomatic SMA patients.

015 UNRAVELLING PSYCHOSIS IN MOTOR NEURONE DISEASE – A STUDY OF CLINICAL FEATURES, COGNITION, AND SURVIVAL

Emma M Devenney*, Rebekah M Ahmed, Jashelle Caga, Elizabeth Highton-Williamson, Eleanor Ramsey, Margaret Zoing, John Hodges, Matthew Kiernan. *University of Sydney, Camperdown, NSW, Australia*

10.1136/jnnp-2019-anzan.15

Introduction Psychotic symptoms are now recognised to occur in patients with MND, often in association with FTD, and particularly in *C9orf72* expansion carriers. As yet the impact of these symptoms on the clinical disease state is unknown and the relationship between severity and nature of these symptoms is not well understood. This study aimed to comprehensively explore the relationship between psychotic symptoms, clinical features, cognitive status and survival.

Methods In total 148 participants; MND (n=100) and MND-FTD (n=48), were enrolled in the study. A detailed clinical interview in addition to a neurological, neuropsychological and behavioural assessment, genetic testing and brain MRI was undertaken in each participant

Results Psychotic symptoms were present in 25% of the cohort. The majority of participants in the psychosis cohort were male (83%) and were negative for the *C9orf72* expansion (70%). Psychotic symptoms in younger patients were more likely to be florid, require medication and delay diagnosis. Within the MND subgroup, patients with psychotic symptoms were more impaired in the cognitive subdomains of attention, memory and executive functioning and exhibited more disinhibition, apathy and stereotypy, than patients without psychotic symptoms (all $p<0.01$), but no differences were identified for the MND-FTD subgroup (all $p>0.2$). Symptoms of depression were more common in those without psychotic symptoms ($p>0.1$). Survival was prolonged for patients with psychotic symptoms (HR=4.7, 95% CI: 2.1–10, $p<0.001$)

Conclusion MND with psychosis represents a distinct clinical, cognitive and behavioural phenotype that has a positive impact on survival and may represent an overlap with psychiatric disorders.

016 ONE DISEASE OR THREE: IS FRONTOTEMPORAL DEMENTIA – MOTOR NEURON DISEASE A DISTINCT ENTITY?

[1,2]Zhe (Jill) Long*, [1,3]Muireann Irish, [1,3]David Foxe, [1,2]John Hodges, [1,3]Olivier Piguet, [1,4,5]James Burrell. [1]*The University of Sydney, Brain and Mind Centre, Camperdown, NSW, Australia*; [2]*Sydney Medical School, The University of Sydney, Sydney, NSW, Australia*; [3]*School of Psychology, The University of Sydney, Sydney, NSW, Australia*; [4]*Concord Clinical School, The University of Sydney, Sydney, NSW, Australia*; [5]*Neurosciences 5West, Concord General Hospital, Sydney, NSW, Australia*

10.1136/jnnp-2019-anzan.16

Introduction Frontotemporal dementia-motor neuron disease (FTD-MND) is diagnosed when patients meet criteria for the diagnosis of both FTD and MND, but the mode presentation of this disorder is currently unknown. This study aimed to compare the mode of presentation, and profiles of behavioural and language disturbances, of FTD-MND with that of other FTD phenotypes using a data-driven approach.

Methods 31 FTD-MND, 119 bvFTD, 47 PNFA, 42 SD patients and 127 controls underwent comprehensive clinical, neuropsychological and neuroimaging assessments. Z-transformed scores were used to compare the severity of behavioural and language domains in each disease group. Two-step cluster analysis profiled patient subgroups. Voxel-based morphometry investigated differential patterns of cortical atrophy between groups.

Results Overall, FTD-MND patients presented with behavioural or language disturbances less frequently than FTD phenotypes, but mixed behavioural-language presentations were more common. FTD-MND patients demonstrated less severe disinhibition, apathy and semantic deficits relative to bvFTD and SD respectively.Behavioural and language deficits were of comparable severity in FTD-MND, unlike other FTD phenotypes where behaviour was worse than language (bvFTD) or language worse than behaviour (PNFA, SD).In cluster analysis, FTD-MND patients were evenly distributed across three subgroups designated as 'mild mixed', 'language dominant' and 'behavioural dominant'. Relative to the 'mild mixed' group, 'language dominant' patients demonstarted more atrophy of the anterior temporal lobe and peri-insular regions, while 'behavioural dominant' patients displayed more prefrontal atrophy.

Conclusion FTD-MND does not present as a uniform syndrome. Rather, there may be at least three subgroups that demonstrate distinctive cognitive, behavioural, and neuroanatomical characteristics.