A.3

The Canadian registry for amyloidosis research: a national multi-disciplinary registry for real-world evidence

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doi: 10.1017/cjn.2023.79

Background: The Canadian Registry for Amyloidosis Research (CRAR) is a nationwide disease registry of transthyretin (ATTR) and light-chain (AL) amyloidosis. Recent advances in disease-modifying therapy have improved prognosis, however there is a critical need for real-world evidence to address knowledge gaps, particularly longer-term therapeutic outcomes and surveillance strategies. Methods: A multi-stakeholder process was undertaken to develop a consensus dataset for ATTR- and AL-amyloidosis. This process included surveys to rank the importance of potential data items, and a consensus meeting of the CRAR steering committee, (comprised of multidisciplinary clinical experts, and patient organization representatives). Patients and patient organizations supported the development and implementation of a patient-reported dataset. Results: Consensus data items include disease onset, progression, severity, treatments, and outcomes, as well as patient-reported outcomes. Both prospective and retrospective (including deceased) patient cohorts are included. Further baseline data will be presented on an initial cohort of patients. Conclusions: CRAR has been established to collect a longitudinal, multidisciplinary dataset that will evaluate amyloidosis care and outcomes. CRAR has launched at multiple specialty amyloidosis centers nationally and is continually expanding. The growth of this program will provide opportunities to assess real-world safety and efficacy and inform the cost-effectiveness of therapies while supporting patient recruitment for research.

A.4

Apomorphine effects on Parkinson’s disease fluctuation-related pain

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doi: 10.1017/cjn.2023.80

Background: Fluctuation-related pain (worse in OFF periods) is a frequent and disabling symptom in Parkinson’s disease (PD). As evidence-based treatments to treat pain in PD are limited, exploring alternatives to treat it are imperative. Apomorphine is the only antiparkinsonian agent compatible with levodopa in improving PD motor symptoms and is usually well tolerated. We explored the effects of apomorphine in PD fluctuation-related pain. Methods: Small pilot double-blind, placebo controlled, randomized crossover study evaluating the safety and efficacy of subcutaneous apomorphine vs. placebo on fluctuation-related PD pain including participants experiencing pain during OFF periods. Primary outcomes: changes in a Visual Analogue Scale for pain and MDS-UPRDS III from baseline to 30 and 60 minutes after injections (two doses, separated by 60 min) and adverse events. Domperidone was used as premedication to avoid nausea/vomiting. Results: 16 patients were screened and 11 completed the study. All participants tolerated both treatments without significant side effects. Efficacy results remain blinded until the end of February 2023 and will be shown at the conference. Conclusions: Apomorphine, recently approved by Health Canada as an adjunctive therapy in PD patients and experiencing “off” periods, has shown to be safe when used to treat fluctuation-related PD pain. Efficacy outcomes will be soon available.

A.5

Neurovascular complications of veno-venous extracorporeal membrane oxygenation in critically ill COVID-19 patients

doi: 10.1017/cjn.2023.81

Background: Veno-venous extracorporeal membrane oxygenation (VV-ECMO) is an invasive intervention for patients with respiratory failure associated with COVID-19. This meta-analysis aims to determine the incidence of neurovascular complications in COVID-19 patients requiring VV-ECMO. Methods: Systematic literature search of MEDLINE, Embase, PsycINFO, and Cochrane databases was performed to identify studies that reported neurovascular complications of adult COVID-19 patients on VV-ECMO for respiratory failure. Case series and reports were excluded. Studies with 95% or more of its patients on VV-ECMO were pooled for meta-analysis. Results: Eighteen studies (n=1968) were included for meta-analyses. In COVID-19 patients requiring VV-ECMO, the incidences of intracranial hemorrhage and ischemic stroke were 11% [95% CI, 8–15%] and 2% [95% CI, 1–3%], respectively. Intraparenchymal and subarachnoid hemorrhages accounted for 73% and 8% of all intracranial hemorrhages, respectively. The risk ratio of mortality in COVID-19 patients with neurovascular complications on VV-ECMO compared to patients without neurovascular complications was 2.24 [95% CI, 1.46–3.46]. Conclusions: COVID-19 patients requiring VV-ECMO have a higher incidence of intracranial hemorrhage compared to historical data in non-COVID-19 patients (11% vs. 8%), while the incidence of ischemic stroke is similar (2%) in both cohorts. COVID-19 patients with neurovascular complications on VV-ECMO are at an increased risk of death.

A.6

CSF1R-related adult-onset leukodystrophy with axonal spheroids and pigmented glia (ALS) presenting as corticobasal syndrome (CBS): a case report and literature review

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doi: 10.1017/cjn.2023.82

Background: Colony stimulating factor 1 receptor (CSF1R) mutations have various clinical, often overlapping, phenotypes. Methods: Case report and literature review. Results: We present a case of a previously independent 49-year-old woman with a 3-