reducing Hex4 levels and was well tolerated in clinical studies in patients with LOPD. Sponsored by Amicus Therapeutics, Inc.

P.102

Clinical predictors of disease progression and survival in ALS: insights from the Canadian Neuromuscular Disease Registry

D Daudu (London) J Arocha Perez (London)* K Henley (Calgary) V Hodgkinson (Calgary) A Abrahao (Toronto) H Briemberg (Vancouver) M Chum (Hamilton) A Genge (Montreal) A Marrero (Moncton) S Kalra (Edmonton) W Johnston (Edmonton) R Massie (Montreal) G Matte (Montreal) M Melanson (Kingston) C O'Connell (Halifax) K Schellenberg (Saskatoon) S Taylor (Halifax) L Zinman (Toronto) Canadian Neuromuscular Disease Registry L Korngut (Calgary) C Shoesmith (London)*

doi: 10.1017/cjn.2025.10259

Background: Amyotrophic Lateral Sclerosis (ALS) leads to progressive functional decline and reduced survival. Identifying clinical predictors like ALSFRS-R and FVC is essential for prognosis and disease management. Understanding progression profiles based on diagnostic characteristics supports clinical trial design and assessment of treatment response. This study evaluates disease progression and survival predictors in ALS patients from the CNDR. Methods: 1565 ALS patients in the CNDR were analyzed to assess baseline ALSFRS-R, FVC, time from symptom onset to diagnosis, and their association with disease progression and survival. Results: At diagnosis, ALSFRS-R was 44.7 (SD = 5.46), with 72.3% scoring ≥44. Mean FVC was 84.2% (SD = 23.3), with 78.3% of patients having FVC ≥65%. ALSFRS-R declined at 1.06 points/month (SD = 1.33), with faster progression in patients diagnosed within 24 months (1.61 points/month). Patients with ALSFRS-R ≥44 had a median survival of 41.8 months, compared to 30.9 months for those <44 ($p < 0.001$). Similarly, FVC ≥65% was associated with longer survival (35.4 vs. 29.5 months, $p = 0.002$). Conclusions: ALSFRS-R and FVC at diagnosis predict survival and inform clinical decision-making. These findings highlight the importance of early diagnosis and targeted interventions to slow disease progression and improve patient outcomes.

P.103

Effectiveness and safety of ravulizumab in generalized Myasthenia Gravis (gMG): Updated analysis from a global registry

*P Narayanaswami (Boston) MT Pulley (Jacksonville) SP Macwan (Rancho Mirage) JM Winkley (Lexington) L Zeinali (Mississauga) C Liu (Baar) V Juel (Durham) R Tandan (Burlington) F Saccà (Napoli) AJ Gordon (Lake Barrington) A Yegin (Boston) JF Howard, Jr. (Chapel Hill) M Nicolle (London)**

doi: 10.1017/cjn.2025.10260

Background: The complement C5 inhibitor (C5IT), ravulizumab, is approved in Canada for the treatment of anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG). Updated effectiveness and safety results from the ongoing MG SPOTLIGHT Registry (NCT04202341) are reported. Methods: MGFA classification and MG-ADL total scores were assessed in patients who received ravulizumab only (ravu-only) or transitioned from eculizumab to ravulizumab (ecu-to-ravu), with data available prior to C5IT initiation ("pre-C5IT") and ≥1 assessment post-initiation ("post-ravu"). Results: Of 52 patients with 2 post-ravu assessments, average treatment duration was 10.4 months at last assessment (LA). Mean±SD MG-ADL scores improved (pre-C5IT: 7.6±3.6; LA: 3.4±3.3), as did the proportions of patients with minimal symptom expression (MSE, MG-ADL≤1) (pre-C5IT: 1/52 [2%]; LA: 17/52 [33%]) and MGFA classification 0-II (pre-C5IT: 18/45 [40%]; LA: 40/45 [89%]). In the ravu-only subgroup, outcomes improved (pre-C5IT vs LA): MG-ADL, 6.3 ±3.0 vs 4.0±3.4; MGFA 0-II, 9/14 [64%] vs 12/14 [86%]. The ecu-to-ravu subgroup sustained continued gradual improvement from last eculizumab assessment to LA: MG-ADL, 4.4±4.2 vs 3.0±2.8; MGFA 0-II, 19/21 [90%] vs 20/21 [95%]. Ravulizumab was well tolerated; no meningococcal infections were reported. Conclusions: These results demonstrate the long-term effectiveness and safety of ravulizumab in routine clinical practice in patients with gMG.

P.104

A portrait of generalized Myasthenia Gravis in Canada: analysis of the Adelphi MG II disease specific programme

O Blanchard (Montreal) K Quansah (Toronto) AE Batista (Titusville) A Erman (Toronto) H Connolly (Bollington) ZA Siddiqi (Edmonton)*

doi: 10.1017/cjn.2025.10261

Background: Generalized myasthenia gravis (gMG) is a rare, chronic, autoimmune disease characterized by muscle weakness and fatigue. This study aims to describe the natural history, disease burden and treatment patterns of gMG patients in Canada. Methods: Data was analyzed from the Adelphi MG II DSP™, a gMG patient-level cross-sectional database, collected through surveys between February-June 2024. Neurologists provided sociodemographic, symptomatology, and treatments data. Results: Fifteen neurologists provided data for 46 gMG patients. The cohort's mean (SD) age was 58.1 (14.7) years, 52.2% male, 82.6% White/Caucasian and 89.1% were anti-AChR Ab positive. Mean time since diagnosis was 3.4 (3.1) years, 22% reported a change in employment status due to gMG. Most had public insurance (68.9%). Disease severity was mostly MGFA class II (78.2%) patients. Common symptoms included eyelid ptosis (76.1%), dysarthria (50.0%), and dyspnea (54.3%) – mean MG-ADL was 5.6 (5.1). During their disease course, 34.9% experienced ≥1 myasthenic crisis, while 25.6% reported symptom exacerbation. At time of survey, patients had used 1.8 (0.9) lines of maintenance treatment. Most prescribed treatments (alone or in combinations) were pyridostigmine (95.6%), corticosteroids (48.9%), non-steroidal immunosuppressants (42.2%), Immunoglobulins (31.1%), and biologics (22.2%). Conclusions: gMG patients continue to experience symptoms burden and crisis/exacerbations. These findings highlight an unmet need for