AB0807 PATIENT-REPORTED OUTCOMES ABOUT QUALITY OF LIFE IN PATIENTS WITH IDIOPATHIC INFLAMMATORY MYOPATHY

Keywords: Quality of life, Patient reported outcomes, Work-related issues

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Background: Idiopathic inflammation myopathy (IIM) defines a group of chronic autoimmune-mediated diseases that most commonly affect the skin, muscles, and lungs [1]. Despite considerable advances over the past decades in improving life expectancy compared with the general population, IIM patients still experience substantially impaired health-related quality of life (HRQoL) [2]. The value of incorporating patient-reported outcomes in clinical trials is increasingly appreciated. The 36-item Short-Form Health Status Survey (SF-36) has been proposed to evaluate HR-QoL in IIM patients. However, because it is very time-consuming, the actual completion rate of the questionnaire is extremely low, and the SF-36 cannot be used directly in cost-effectiveness analyses. The Euro-Qol 5-Dimension (EQ-5D) is another tool for assessing HR-QoL and has been used extensively for chronic diseases. Wolfe et al. [3] verified the association between EQ-5D and SF-36 in rheumatic diseases. Moreover, workforce losses in patients with IIM are underestimated, and even health-related absenteeism imposes an economic burden on society [4].

Objectives: To explore the feasibility and validity of the EuroQol 5-dimension (EQ-5D) and Work Productivity and Activity Impairment (WPAI) surveys as patient-reported outcomes of health-related quality of life (HR-QoL) in idiopathic inflammation myopathy (IIM).

Methods: This cross-sectional study surveyed patient’s outcomes using the Manual Muscle Testing-8 (MMT-8), Myositis Disease Activity Assessment Visual Analog Scale (MYAOC), Myositis Damage Index (MDI), Disease Activity Score (DAS), and Physician/Patient Global Assessment (PGA/PtGA). HR-QoL was determined using EQ-5D, 36-item Short-Form Health Status Survey (SF-36), and WPAI questionnaire. The relationship between IIM-related parameters and HR-QoL was assessed using ordinal logistic and quantile regression.

Results: We enrolled 189 patients with IIM. Decreased MMT-8 and increased MYAOC, DAS, MDI-global, and PGA/PtGA were associated with higher EQ-5D values. For the 25th–75th percentile of WPAI, greater activity impairment was associated with lower MMT-8 and higher age of onset and PGA. Poorer overall working productivity impairment was associated with higher MYAOC (cutaneous and skeletal), MDI-global and PtGA were associated with increased activity and overall working productivity impairment in most quantities (P < 0.05).

Conclusion: The EQ-5D and WPAI may be valid patient-reported outcomes to evaluate HR-QoL in ambulatory patients with IIM.

REFERENCES:

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AB0808 A PROOF OF BIOLOGICAL CONCEPT TRIAL OF CM101 TO TARGET CCL24 IN SYSTEMIC SCLEROSIS: A BIOMARKER INFORMED, PRECISION MEDICINE APPROACH

Keywords: Systemic sclerosis, Biomarkers, Clinical Trials

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Background: Systemic sclerosis (SSc) is an inflammatory and fibrosing autoimmune disease of unknown etiology. However, it has been established that endothelial cells, immune cells, and fibroblasts play important roles in SSc initiation and progression. CCL24 (eotaxin-2) is a chemokine secreted by fibroblasts and endothelial cells, which promotes trafficking of proinflammatory immune cells through the CCR3 receptor. Levels of CCL24 and CCR3 in the skin and serum of patients with SSc were found to be upregulated compared with healthy controls [1]. Serum CCL24 levels have been shown to correlate with extracellular matrix turnover biomarkers, to predict decline in forced vital capacity (FVC) and the diffusion capacity of the lungs for carbon monoxide (DLCO), and to be associated with a worse digital ulcer burden in patients with SSc [1]. Blockade of CCL24 with CM101, a humanized IgG1 anti-human CCL24 monoclonal antibody, significantly reduced endothelial cell activation in vitro and in skin and lung immune cell infiltration and fibrosis in experimental SSc animal models.

Objectives: To present an innovative trial design which allows for comprehensive disease assessment and determination of the biological activity of CM101 in patients with SSc.

Methods: Informed by the predictive value of CCL24 on vascular and fibrotic clinical outcomes in a large SSc observational cohort, the CM101 clinical development team aims to bridge biomarker science and clinical trial design by implementing a phase 2a clinical trial that includes evaluation of the