Paucity of bulbar function measures in inclusion body myositis trials.

Reply to: Current status of clinical outcome measures in inclusion body myositis: a systematised review

Sirs,

Decline in bulbar functions in sporadic inclusion body myositis (IBM) likely results from weakening of facial, oropharyngeal, and/or oesophageal musculature (1), and may manifest as swallowing and speech disorders (dysphagia and dysarthria, respectively). Dysphagia affects up to 80% of patients with IBM (1), whereas dysarthria has not been extensively investigated in this population, despite shared anatomical and physiological substrates. Bulbar deficits in IBM are progressive and may result in grave quality of life and health consequences, with aspiration pneumonia (typically secondary to dysphagia) being a frequent cause of death (2). However, there are no established guidelines for management of bulbar deficits in this population, likely resulting from failure to fully understand the underlying impairments and a delay in exhibiting or recognising bulbar involvement (1). A comprehensive understanding of the pathophysiology contributing to swallowing and speech deficits requires clinical investigations to report measures of bulbar function, and to identify or develop targeted treatment options.

The IBM Special Interest Group (SIG) of the International Myosotis Assessment and Clinical Studies Group (IMACS) was formed to recommend a core set of outcome measures in IBM trials. One tangible product thus far has been the work from IMACS which reviewed outcome measures reported in IBM clinical trials. We were disappointed to find that swallowing was rarely objectively evaluated – only in three trials reviewed – and even when reported, it was considered as a secondary/exploratory outcome. This represents a significant gap given the high prevalence of dysphagia, its varied manifestations, and potential consequences in IBM (1).

Further, there were no trials which included objective measures related to speech. There is limited evidence to suggest that at least some patients with IBM may present with dysarthria (4), which is known to also be detrimental to quality of life as impaired communication negatively impacts independence and social integration. Most existing evidence is based on clinical opinion and case reports, though one larger investigation in several neuromuscular diseases, which included patients with IBM, noted high prevalence of both dysarthria and dysphagia. The data reported for IBM, however, were grouped with other inflammatory myopathies (4). Our understanding of speech in IBM, is therefore limited.

Failure to recognise bulbar dysfunction in IBM clinically and in research is a great disservice to comprehensive care management for these patients. Further, it not only limits our understanding of bulbar pathophysiology and progression in IBM, but also limits patient access to existing treatment options, as well as identification and development of new treatment targets. Swallowing and speech rehabilitative treatments (*e.g.* respiratory or vocal exercises) have been shown to be beneficial for maintaining and even improving bulbar function in patients with other degenerative diseases (5). We hope this letter serves as a call to action to clinical investigators of IBM to not only include measures of bulbar function in their clinical trials, but to further investigate its underlying pathophysiology, so that targeted therapeutics can be developed and explored, in order to best inform clinical practice and improve patient outcomes.

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Competing interests: B. Roy has served as a consultant for Takeda Pharmaceuticals, Argenx, Alexion Pharmaceuticals (now part of AstraZeneca), and has stocks (<$5000) in Cabaletta Bio. He declares no direct conflicts related to this work. The other authors have declared no competing interests.

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References

1. MOHANNAK N, PATTISON G, HIRD K, NEEDHAM M: Dysphagia in patients with sporadic inclusion body myositis: management challenges. *Int J Gen Med* 2019; 12: 465-74. https://doi.org/10.2147/IJGM.S198031
2. MURATA KY, KOUDA K, TAJIMA F, KONDO T: A dysphagia study in patients with sporadic inclusion body myositis (s-IBM). *Neurol Sci* 2012; 33(4): 765-70. https://doi.org/10.1007/s10072-011-0814-y
3. ROY B, LUCCHINI M, LILLEKER J *et al.*: Current status of clinical outcome measures in inclusion body myositis: a systematised review. *Clin Exp Rheumatol* 2023; 41: 370-78. https://doi.org/10.55563/clinexprheumatol/ifacv3
4. KNUIJT S, KALF JG, DE SWART BJM *et al.*: Dysarthria and dysphagia are highly prevalent among various types of neuromuscular diseases. *Disabil Rehabil* 2013; 36(15): 1285-9. https://doi.org/10.3109/09638288.2013.845255
5. ROGUS-PULIA NM, PLOWMAN EK: Shifting tides toward a proactive patient-centered approach in dysphagia management of neurodegenerative disease. *Am J Speech Lang Pathol* 2020; 29(2S): 1094-109. https://doi.org/10.1044/2020_AJSLP-19-00136