

Title: Improving the understanding of how patients with non-dystrophic myotonia are selected for myotonia treatment with mexiletine (NaMuscla): outcomes of treatment impact using a European Delphi panel

Authors: Ann-Marie Chapman¹, Marieke Schurer^{2*}, Laure Weijers², Amer Omar³, Hiba Lee⁴, Alla Zozulya-Weidenfeller³, Crispin Ellis⁵, Shaneil Sonecha⁵, Christiane Schneider-Gold⁶

*Correspondence: mschurer@bresmed.com

¹ BresMed Health Solutions Ltd, Sheffield, UK

² BresMed Netherlands BV, Utrecht, The Netherlands

³ Lupin Atlantis Holdings SA, Zug Switzerland

⁴ AGH Partners Ltd, London, UK

⁵ Lupin Healthcare Ltd, Slough, UK

⁶ St. Josef-Hospital, Bochum, Germany

Selection criteria Delphi panellists

Selection criteria for the UK experts

- The participant must work in one of the 18 neuroscience specialist centres as determined by NHS England, or work in one of the main centres in Scotland or Wales.
- The centre should be known to treat at least five NDM patients (quantifiable expertise).
- The participant must be known as a neurology expert in the treatment of NDM patients (qualitative expertise), who Lupin believes would have the knowledge and capability to answer the questions posed in the Delphi survey.
- The participant must be previously responsive to a Lupin invitation, to avoid irritation to any potential expert who would be highly unlikely to engage.

Selection criteria for experts from outside the UK

Experts from outside the UK must:

- Be a key opinion leader in the field:
 - Internationally recognized by peers as expert in the fields of Myotonia
 - Members of neurology societies and neuromuscular research societies
- Have proven international expertise in the field of NDM, as demonstrated through:
 - Experience in clinical research as well as management of patients with NDM,
 - Publications, and
 - Experience as an advocate and speaker on relevant conferences.