# A Qualitative Analysis of Results from a Delphi Study to formalise a treatment protocol for children with early diagnosed Duchenne Muscular Disease (DMD) using giles®, an Artificial Intelligence (AI) agent.

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R-S-S Regulatory Scientific and Health Solutions

TREAT-NMD Neuromuscular Network

giles®

## INTRODUCTION & OBJECTIVES

Increasing prevalence of newborn genetic screening has led to earlier diagnoses of Duchenne Muscular Dystrophy (DMD). A treatment framework was published to provide clinicians and associated multidisciplinary teams with guidance on best practices in care and treatment for newly diagnosed DMD patients and their families. TREAT-NMD assembled a panel of experts in the DMD field to review the framework and a Delphi study was used to determine the level of consensus.

## METHODS

Fourteen panel members scored each action item of the treatment framework via anonymised online surveys using a seven-point Likert scale (-3 (strong disagreement) to +3 (strong agreement)), providing explanations for their ratings. Three criteria were defined for consensus: (i) Median score of ≥2, (ii) Interquartile Range (IQR) ≤ 1, (iii) Minimum score ≥ 0. Qualitative analyses of panellist plain text comments on their responses across two survey rounds were undertaken by giles®, an Artificial Intelligence (AI) agent using a large language model (LLM).

## RESULTS

12 out of 35 action items did not reach consensus in round 1 and were re-evaluated in round 2. Cronbach's Alpha[1,2] was reported at 0.861 and 0.802, for responses to action items in rounds 1 and 2, respectively, indicating 'good' internal consistency of the data from both rounds (Tables 1 and 2).

### Table 1: Round 1 Data: Internal Consistency

| Survey Data (Questions Used) | Survey Data (Questions Excluded) | Cronbach's Alpha [Internal Consistency Description] |
|---|---|---|
| **Q1 - Q35** | None (N/A) | 0.861 [Good] |
| **Questions achieving consensus criteria 2** | Q6.6, Q12.1, Q16.2, Q16.3 | 0.867 [Good] |
| **Questions achieving consensus criteria 3** | Q6.5, Q8.2, Q8.3, Q10.3, Q12.2, Q12.3, Q12.4, Q16.3 | 0.866 [Good] |
| **Questions achieving consensus criteria 1, 2, and 3** | Q6.5, Q6.6, Q8.2, Q8.3, Q10.3, Q10.4, Q12.1, Q12.2, Q12.3, Q12.4, Q16.2, Q16.3 | 0.886 [Good] |

Consensus Criteria 1: A median score of ≥2 indicates agreement.
Consensus Criteria 2: Interquartile Range (IQR) ≤ 1.
Consensus Criteria 3: Questions with no negative scores from any panellists.

### Table 2: Round 2 Data: Internal Consistency

| Survey Data (Questions Used) | Survey Data (Questions Excluded) | Cronbach's Alpha [Internal Consistency Description] |
|---|---|---|
| Q6.5, Q6.6, Q8.2, Q8.3, Q10.3, Q12.1, Q12.2, Q12.3, Q12.4, Q16.2, Q16.3 | None (N/A) | 0.802 [Good] |
| Questions achieving consensus criteria 1 | Q12.2, Q16.2, Q16.3 | 0.449 [Unacceptable] |
| Questions achieving consensus criteria 2 | Q6.6, Q8.3, Q10.3, Q12.2, Q12.3, Q12.4, Q16.2, Q16.3 | 0.604 [Questionable] |
| Questions achieving consensus criteria 3 | N/A[a] | N/A[a] |
| Questions achieving consensus criteria 1, 2, and 3 | N/A[a] | N/A[a] |

Note: [a]No questions or only 1 question achieved consensus based on the criteria defined.

The qualitative analyses included a summary of explanations provided by panellists for their ratings of the action items. 43% to 86% of panellists provided explanations for their ratings in round 2 and giles® was able to highlight where panellists aligned on reasonings for their scores. For the following action item in round 2: 'Recommended discussion points between the neuromuscular clinician and the family at the time of diagnosis (Conduct a transparent discussion regarding the seriousness of the condition avoiding catastrophic language such as 'fatal', 'lethal' and 'incurable' as well as focusing on the treatment options of this condition)', giles® provided the following summary:

*"11 out of 14 panellists provided an explanation of their scoring. The panellists generally agree on the importance of providing a balanced and sensitive approach when discussing a diagnosis of Duchenne Muscular Dystrophy (DMD) with families. They emphasize the need to convey the seriousness of the condition, including the lack of a cure and shortened life expectancy, while avoiding catastrophic language. The initial discussion should offer a general overview and allow families to ask questions, with more detailed conversations occurring over subsequent visits. Tailoring the discussion to the family's emotional state and receptiveness is crucial, as is providing some optimism about ongoing research and potential treatments. Transparency and honesty are key, but the information should be delivered in a way that offers a pathway forward, acknowledging that families may already be aware of the condition's severity from online sources. The panellists also highlight the importance of discussing holistic aspects of care, such as quality of life and emotional support, to help families cope with the diagnosis."*

## CONCLUSIONS

This Delphi study provided clarity and confidence around several central pillars of the early diagnosis framework. The giles® tool was able to successfully identify where panellists were aligned on each of the action items and where they differed in opinion by providing a short and consistent summary of all responses.

## REFERENCES

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