



## Clinical trial results:

## Beta-agonist Efficacy and Tolerability as Adjuvant therapy in Myasthenia Gravis

### Summary

|                          |                  |
|--------------------------|------------------|
| EudraCT number           | 2019-000895-40   |
| Trial protocol           | DK               |
| Global end of trial date | 30 December 2024 |

### Results information

|                                |               |
|--------------------------------|---------------|
| Result version number          | v1 (current)  |
| This version publication date  | 13 March 2026 |
| First version publication date | 13 March 2026 |

### Trial information

#### Trial identification

|                       |            |
|-----------------------|------------|
| Sponsor protocol code | BETA-MG-01 |
|-----------------------|------------|

#### Additional study identifiers

|                                    |             |
|------------------------------------|-------------|
| ISRCTN number                      | -           |
| ClinicalTrials.gov id (NCT number) | NCT03914638 |
| WHO universal trial number (UTN)   | -           |

Notes:

### Sponsors

|                              |   |
|------------------------------|---|
| Sponsor organisation name    | Aarhus University Hospital                                  |
| Sponsor organisation address | Palle Juul-Jensens Boulevard, 8200 Aarhus N, Denmark,       |
| Public contact               | Jan L. S. Thomsen, Aarhus University Hospital, jathms@rm.dk |
| Scientific contact           | Jan L. S. Thomsen, Aarhus University Hospital, jathms@rm.dk |

Notes:

### Paediatric regulatory details

|  |    |
|--|----|
| Is trial part of an agreed paediatric investigation plan (PIP)       | No |
| Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial? | No |
| Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial? | No |

Notes:

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**Results analysis stage**

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|  |                  |
|--|------------------|
| Analysis stage                                       | Final            |
| Date of interim/final analysis                       | 20 October 2025  |
| Is this the analysis of the primary completion data? | Yes              |
| Primary completion date                              | 30 December 2024 |
| Global end of trial reached?                         | Yes              |
| Global end of trial date                             | 30 December 2024 |
| Was the trial ended prematurely?                     | No               |

Notes:

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**General information about the trial**

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Main objective of the trial:

To investigate the efficacy and tolerability of oral Salbutamol as adjuvant therapy in patients with generalized myasthenia gravis on stable medications with residual symptoms.

Protection of trial subjects:

Follow-up

Background therapy: -

Evidence for comparator: -

|   |               |
|---|---------------|
| Actual start date of recruitment                          | 01 April 2019 |
| Long term follow-up planned                               | No            |
| Independent data monitoring committee (IDMC) involvement? | No            |

Notes:

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**Population of trial subjects**

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**Subjects enrolled per country**

|                                      |             |
|--------------------------------------|-------------|
| Country: Number of subjects enrolled | Denmark: 30 |
| Worldwide total number of subjects   | 30          |
| EEA total number of subjects         | 30          |

Notes:

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**Subjects enrolled per age group**

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|   |    |
|---|----|
| In utero                                  | 0  |
| Preterm newborn - gestational age < 37 wk | 0  |
| Newborns (0-27 days)                      | 0  |
| Infants and toddlers (28 days-23 months)  | 0  |
| Children (2-11 years)                     | 0  |
| Adolescents (12-17 years)                 | 0  |
| Adults (18-64 years)                      | 23 |
| From 65 to 84 years                       | 7  |
| 85 years and over                         | 0  |

## Subject disposition

### Recruitment

Recruitment details:

June 2019 to august 2024 at Aarhus, Aalborg and Odense

### Pre-assignment

Screening details:

44 screened, 37 assessed for eligibility, 30 eligible.

### Period 1

|                              |  |
|------------------------------|--|
| Period 1 title               | Period 1 (overall period)              |
| Is this the baseline period? | Yes                                    |
| Allocation method            | Randomised - controlled                |
| Blinding used                | Double blind                           |
| Roles blinded                | Subject, Investigator, Carer, Assessor |

Blinding implementation details:

Active and placebo encapsulated in identical caps.

### Arms

|                              |            |
|------------------------------|------------|
| Are arms mutually exclusive? | Yes        |
| <b>Arm title</b>             | Salbutamol |

Arm description: -

|  |              |
|--|--------------|
| Arm type                               | Experimental |
| Investigational medicinal product name | Salbutamol   |
| Investigational medicinal product code |              |
| Other name                             |              |
| Pharmaceutical forms                   | Capsule      |
| Routes of administration               | Oral use     |

Dosage and administration details:

4 mg. three times daily

|                  |         |
|------------------|---------|
| <b>Arm title</b> | Placebo |
|------------------|---------|

Arm description: -

|  |          |
|--|----------|
| Arm type                               | Placebo  |
| Investigational medicinal product name | Placebo  |
| Investigational medicinal product code |          |
| Other name                             |          |
| Pharmaceutical forms                   | Capsule  |
| Routes of administration               | Oral use |

Dosage and administration details:

1 capsule three times daily

| <b>Number of subjects in period 1</b> | Salbutamol | Placebo |
|---------------------------------------|------------|---------|
| Started                               | 15         | 15      |
| Completed                             | 13         | 15      |
| Not completed                         | 2          | 0       |
| Adverse event, non-fatal              | 2          | -       |

## Baseline characteristics

## End points

### End points reporting groups

|                                |            |
|--------------------------------|------------|
| Reporting group title          | Salbutamol |
| Reporting group description: - |            |
| Reporting group title          | Placebo    |
| Reporting group description: - |            |

### Primary: QOL15

|                        |         |
|------------------------|---------|
| End point title        | QOL15   |
| End point description: |         |
|                        |         |
| End point type         | Primary |
| End point timeframe:   |         |
| Baseline to week 8.    |         |

| End point values                 | Salbutamol          | Placebo            |  |  |
|----------------------------------|---------------------|--------------------|--|--|
| Subject group type               | Reporting group     | Reporting group    |  |  |
| Number of subjects analysed      | 15                  | 15                 |  |  |
| Units: -3,9                      |                     |                    |  |  |
| number (confidence interval 95%) | -4.7 (-6.6 to -2.7) | -0.8 (-2.8 to 1.2) |  |  |

### Statistical analyses

|   |                                |
|---|--------------------------------|
| Statistical analysis title  | Mixed effects                  |
| Statistical analysis description:   |                                |
| Mixed effects linear regression with treatment, visit, treatment-by-visit interaction, sequence and period as fixed effects and subject as random effect. |                                |
| Comparison groups   | Salbutamol v Placebo           |
| Number of subjects included in analysis   | 30                             |
| Analysis specification  | Pre-specified                  |
| Analysis type   | superiority                    |
| P-value   | < 0.05                         |
| Method  | Mixed models analysis          |
| Parameter estimate  | Mean difference (final values) |
| Point estimate  | -3.9                           |
| Confidence interval   |                                |
| level   | 95 %                           |
| sides   | 2-sided                        |
| lower limit   | -6.7                           |
| upper limit   | -1.1                           |

|                      |                    |
|----------------------|--------------------|
| Variability estimate | Standard deviation |
|----------------------|--------------------|

## Secondary: QMG

|                        |           |
|------------------------|-----------|
| End point title        | QMG       |
| End point description: |           |
| End point type         | Secondary |
| End point timeframe:   |           |
| Baseline to week 8     |           |

| End point values                 | Salbutamol          | Placebo           |  |  |
|----------------------------------|---------------------|-------------------|--|--|
| Subject group type               | Reporting group     | Reporting group   |  |  |
| Number of subjects analysed      | 15                  | 15                |  |  |
| Units: -3,0                      |                     |                   |  |  |
| number (confidence interval 95%) | -2.6 (-3.4 to -1.7) | 0.4 (-0.4 to 1.3) |  |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: MGC

|                        |           |
|------------------------|-----------|
| End point title        | MGC       |
| End point description: |           |
| End point type         | Secondary |
| End point timeframe:   |           |
| Baseline to week 8     |           |

| End point values                 | Salbutamol          | Placebo            |  |  |
|----------------------------------|---------------------|--------------------|--|--|
| Subject group type               | Reporting group     | Reporting group    |  |  |
| Number of subjects analysed      | 15                  | 15                 |  |  |
| Units: -1,9                      |                     |                    |  |  |
| number (confidence interval 95%) | -2.9 (-4.1 to -1.7) | -1.0 (-2.2 to 0.2) |  |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: MG-ADL

End point title MG-ADL

End point description:

End point type Secondary

End point timeframe:

Baseline to week 8

| End point values                 | Salbutamol          | Placebo            |  |  |
|----------------------------------|---------------------|--------------------|--|--|
| Subject group type               | Reporting group     | Reporting group    |  |  |
| Number of subjects analysed      | 15                  | 15                 |  |  |
| Units: -1,0                      |                     |                    |  |  |
| number (confidence interval 95%) | -1.4 (-2.0 to -0.7) | -0.4 (-1.0 to 0.3) |  |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Neuro-QoL

End point title Neuro-QoL

End point description:

End point type Secondary

End point timeframe:

Baseline to week 8

| End point values                 | Salbutamol           | Placebo            |  |  |
|----------------------------------|----------------------|--------------------|--|--|
| Subject group type               | Reporting group      | Reporting group    |  |  |
| Number of subjects analysed      | 15                   | 15                 |  |  |
| Units: -7,8                      |                      |                    |  |  |
| number (confidence interval 95%) | -8.9 (-12.0 to -5.7) | -1.1 (-4.2 to 2.1) |  |  |

### Statistical analyses

No statistical analyses for this end point



## Adverse events

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### Adverse events information<sup>[1]</sup>

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Timeframe for reporting adverse events:

Baseline to week 8 in both treatment periods.

|                 |            |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

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### Dictionary used

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|                 |        |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

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|                    |    |
|--------------------|----|
| Dictionary version | 22 |
|--------------------|----|

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Frequency threshold for reporting non-serious adverse events: 5 %

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### Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.

Justification: See publication on adverse events. This EudraCT system is not set-up to manage cross-over designs. There were no serious adverse events.

Palpitations 12 (salbutamol) / 3 (placebo)

Hypertension 2(salbutamol) / 5 (placebo)

Tremor 16 (salbutamol) / 0 (placebo)

Head 7 (salbutamol) / 3 (placebo)

**More information**

**Substantial protocol amendments (globally)**

Were there any global substantial amendments to the protocol? No

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**Interruptions (globally)**

Were there any global interruptions to the trial? Yes

| Date             | Interruption | Restart date |
|------------------|--------------|--------------|
| 02 December 2019 | Covid-19     | -            |

Notes:

**Limitations and caveats**

None reported