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ATL1102 for DMD: Revised Clinical Study Plans
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Phase IIb Study Program

Title: A multicentre, randomised, double-blind, placebo-controlled and open label extension study to assess the efficacy, safety, and pharmacokinetic profile of two dose levels of ATL1102 administered by subcutaneous (sc) injection in non-ambulatory participants with Duchene Muscular Dystrophy (DMD).

Design:

- The study consists of a blinded phase and an Open Label Extension (OLE) Phase in which participants receive once weekly subcutaneous dosing for 49 weeks :
 - In the Blinded Phase participants will be randomised in a 1:1:1 ratio to 2 dose levels of ATL1102 (25 mg or 50 mg) or matching placebo, with stratification by corticosteroid use, administered over a 25 week Blinded Treatment Period.
 - In the Open Label Treatment Phase participants will continue treatment for a further 24 weeks. Participants receiving ATL1102 during the blinded phase will continue at the initial dose level (25 mg/week or 50 mg/week) to which they were randomised at entry into the study. Participants receiving placebo during the Blinded Phase will be assigned to either ATL1102, 25 or 50 mg dose level in accordance with the placebo dose level group to which the participant was randomised at entry into the study.
- The study is planned to be conducted in approx. 4 countries and 13 clinical trial sites.

Phase IIb Study Program *(Continued....)*

Sample size:

- Approximately 45 participants to be enrolled (15 per treatment arm) with 42 participants to complete the study

Target population:

- Participants with DMD (confirmed by genetic testing) and are non-ambulatory, defined as unable to walk 10 meters without assistance or help
- 10 to less than 18 years of age at screening, body weight of at least 25 kg
- PUL 2.0 Entry Item A score ≥ 2
- If on corticosteroid therapy, therapy was initiated at least six months prior to the baseline visit and a stable daily dose (≥ 0.25 mg/kg/day prednisone, prednisolone, or equivalent dose for deflazacort) for at least 3 months prior to baseline.

Phase IIb Study Program *(Continued....)*

Blinded Phase - Study Objectives

Primary objective:

To evaluate the effect of ATL1102 on upper limb muscle function in non-ambulant participants with DMD as assessed by change in the Performance of Upper Limb Module for DMD 2.0 (PUL 2.0) score compared to placebo

Secondary objectives:

To evaluate the effects of ATL1102:

- in a responder analysis on the PUL 2.0 score compared to placebo
- on upper limb muscle strength as assessed by change in percent predicted MyoGrip and MyoPinch compared to placebo
- on respiratory function as assessed by change in % predicted PEF and % predicted FVC compared to placebo
- on Quality of Life (PedsQL assessments) assessed by participants and their study partner (Parent/Guardian) compared to placebo
- safety and tolerability of ATL1102 including events associated with the Safety Monitoring Plan and Stopping Rules
- PK profile of ATL1102

Analysis Timepoint for the Blinded Phase of the Study:

The Primary efficacy and safety endpoint analysis of the unblinded 6 month data will be undertaken once the last participant randomised has completed the Week 25 efficacy and safety assessments in the Blinded Phase of the study.

Phase IIb Study Program *(Continued....)*

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