Phase 2/3 Study of Arimoclomol in Sporadic Inclusion Body Myositis: Study Design - IBM4809 (NCT: 02753530)


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NON-Clinical

Arimoclomol is an amplifier of the heat shock response under conditions of cellular stress (1). Heat shock response promotes clearance of intracellular protein aggregates, natural folding of nascent proteins, and refolding of misfolded proteins, reconstituting normal protein function (2, 3) - actions that are expected to have a disease modifying effect.

MAIN FINDINGS

Main findings at month 8 were (arimoclomol versus placebo):

- Change in the right-grip maximum voluntary isometric contraction (MVICT) score (1.26 ± 2.63 versus 0.26 ± 0.27)
- Change in the mean MMT score (−0.12 ± 0.22 versus −0.54 ± 1.86).
- Change in the IBMFRS score (−0.68 ± 1.58 versus −2.50 ± 0.54).

RESULTS & CONCLUSION

- The last subject (n=152) was enrolled on 22nd April 2019.
- Approximately 10 subjects have completed the 20 month follow-up.
- Approximately 10 subjects have completed the 20 month follow-up at last date of IMP.
- Post-baseline study visits will occur every 30 days (± 7 days) for visits 3 to 7 and the remaining visits will be every 60 days relative to baseline. The visits will be in-person at months 1, 2, 3, 4, 5, 6, 12, 16 and 20 while phone calls will be on a treatment period of 20 months and a post-treatment follow-up at 1 month last date of IMP.

REFERENCES

5. Rose MR, ENMC IBM working group. 2013: Neuromuscular disorders. NMD.

KEY RESULTS are anticipated in H2 2021

ORPHAZYME

Orphazyme A/S is a Danish biopharmaceutical company with a late-stage drug pipeline, developing new treatment options for orphan protein-misfolding diseases.

The lead program, arimoclomol, recently reported positive results from a Phase II/III study (NCT02641216) in Niemann-Pick disease type C (NPC).

Arimoclomol is currently in a Phase II trial (NCT02576887) in patients diagnosed with GD1/GD3.

In addition, arimoclomol is in phase I/Ib trials in amyotrophic lateral sclerosis (ALS), NCT03089142, with an open-label extension (NCT03629162) and in NCT01364902 for sporadic inclusion body myopathy (IBM), NCT02779140.

For more information, please visit www.orphazyme.com.