

MLBIO clinical studies – Description

Ongoing studies:

- Lead-in study also called by some individuals the “Natural history study”
- A study in healthy volunteers
- A treatment study in LGMD2I individuals also called by some people the “phase 2” study

Future studies:

- Phase 3 study – will precede market approval. Design has not been finalized. Timelines has not been determined.
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Lead-in study:

- Opened in 11 US and 1 European centers (recently opened in Copenhagen, DN).
- So far enrolled 55 patients in the US. Hope to enroll 80. Expect to have that done by end of June at the latest in the US and end of August in Copenhagen, DN.
- Duration is 1 year.
- Participants will be invited to participate in the phase 3 study.
- See summary at <http://www.grasp-lgmd.org/active-studies/>

Study in healthy volunteers, also called phase I:

- Almost done.
- The 2 cohorts have been enrolled. In this study, doses are escalated, meaning that first patient is given lowest dose to last patient given highest dose:
 - In first cohort, patients are given one dose up to 12g.
 - In 2nd cohort, patients are given one dose up to 9g twice a day over 7 days.
- Full results not available yet, no significant toxicity has been reported thus far. Some patients had soft stools.

Treatment study in LGMD2I individuals:

- At only one site: Virginia Commonwealth University (VCU) in Richmond, VA
- About to finish the enrollment of the 3 cohorts:
 - a) 1st cohort at 6g a day once a day.
 - b) 2nd cohort at 6g twice a day.
 - c) 3rd cohort at 12g twice a day.
- Total of 14-16 patients will be enrolled.
- Proceeds in phases
 1. 3-month treatment with one of the three dosages above (a, b, or c).
 2. If everything looks good (no toxicity), an additional 3-month treatment for all participants (in all cohorts 1,2, and 3) at 12g twice a day.
- Needle core biopsies will be performed 3 times: at the start, at 3 months, and then at 6 months.
- Biopsies will be used to perform a bioassay (change in improvement of FKR activity).
- Functional assays (walking, sitting, ...) will be performed for 24 months from start of study.

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