Case Study

Healthy Volunteer Study - Indication: Duchenne Muscular Dystrophy (DMD)

Phase 1 Randomised, Double-Blind, Placebo-Controlled Trial to Evaluate Safety, Tolerability, and Pharmacokinetics of a Single Intravenous Infusion of Ascending Doses of XXXXXX in Healthy Male Volunteers.

Phase: 1

Countries: 1

Sites: 1

LOCATION

United Kingdom

Manchester Early Phase Unit

SERVICES

- **Clinical Services**
- **Data Management**
- **Biostatistics**
- **Medical Writing**

REQUIREMENTS

MALE SUBJECTS AGED BETWEEN 18 AND 55 YEARS OLD

SUBJECTS WITH A BMI BETWEEN 18 - 32

STUDY PROCEDURE

MUSCLE BIOPSY A KEY COMPONENT OF THE STUDY

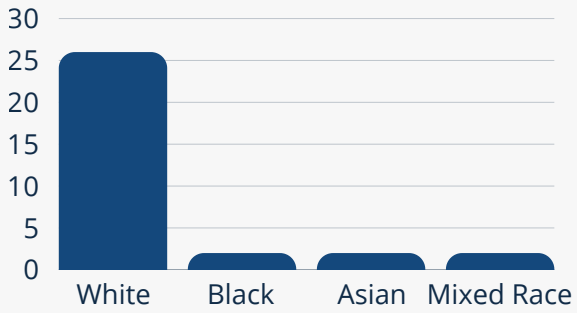
KEY STUDY DELIVERABLES

PARTICIPANT RECRUITMENT & DEMOGRAPHIC

32
RECRUITMENT TARGET

32
PARTICIPANTS ENROLLED

RECRUITED TO TARGET



4 COHORTS CONSISTING OF:


2 x Screenings


1 x 7-night Residential Stay (check-in: Day 1, Dose: Day 1)


1 x Muscle Biopsy Per Participant


3 x 1-Night Residential Stays (Day 14, 21, 28)


1 x Outpatient Visit (Day 42)

STUDY TIMELINE (NO. OF MONTHS)

MAC completed the study within 10 months

KEY STUDY DELIVERABLES

PROCEDURE

- 32 biopsies taken in total
- Core needle biopsy approach used (as opposed to open surgical biopsy). MAC considers this approach safer and better tolerated than open surgical biopsy, with fewer complications and a faster recovery for participants.
- A needle muscle biopsy taken from the biceps brachii muscle 72 hours ± 4 hours post end of infusion (Day 4), using a BD Elevation™ Needle Gun to evaluate potential exon skipping and XXXXXXX concentration in the muscle. At the biopsy site, local anesthetic was administered. Three cores were collected per participant for a total of 390 to 450 mg of muscle. After sufficient pieces of muscle tissue were collected, the wound was closed using sterile adhesive strips. Full needle muscle biopsy methods were provided in the Surgical Manual and Biopsy Processing Manual.
- The procedure was performed by Dr Giuseppe Fiore at MAC's fully owned MHRA-accredited Early Phase Unit.

Dr Giuseppe Fiore
Assistant Director of Early Phase Research

Dr Giuseppe Fiore is an MHRA-approved First-in-Human (FIH) Principal Investigator and Member of the Faculty of Pharmaceutical Medicine (FPM) with a Diploma in Experimental Therapeutics. He is a board-certified surgeon on the GMC Specialist Register with a Master's in Aesthetic Surgery and a Certificate in Human Pharmacology. Dr Fiore has over 10 years of experience as a Principal Investigator and Chief Investigator in early and late-phase clinical trials across multiple therapeutic areas.

Dr Fiore has led more than 200 studies involving small molecules and monoclonal antibodies, and is highly skilled in performing biopsies and other surgical procedures.

CAPABILITIES AND EXPERTISE

MUSCLE BIOPSIES

At MAC Clinical Research, muscle biopsies can be performed using either an open surgical approach or a less invasive percutaneous technique. These biopsies are performed mainly for research purposes, helping to identify specific biomarkers and assess the stage of neuromuscular or metabolic conditions in clinical trials.

The open biopsy involves surgically removing a small segment of muscle, while the percutaneous method uses a needle to extract a sample, reducing recovery time. Both approaches provide valuable insights that aid in the evaluation of investigational treatments, all while upholding the highest standards of patient safety.

We can comfortably perform 4-5 muscle biopsy procedures per day, depending on the study design of the protocol.

MAC EARLY PHASE UNIT

MAC's Early Phase Unit is an MHRA-audited and accredited Phase I centre located within and supported by the Manchester University Foundation Trust.

- Highly trained and experienced Early Phase team
- Specialist techniques are in place including EEG, pharmacodynamic measures, lumbar punctures, and aseptic techniques to support complex procedures
- Ultrasound Imaging System
- GMP and aseptic facilities to support IMP preparation
- In-house MHRA/UKAS-accredited bioanalysis and clinical pathology laboratories to ensure rapid, high-quality data analysis
- Specialised in recruiting and enrolling both patients and healthy volunteers into Phase I-II clinical research studies, where the safety of the study participants is our highest priority.

MAC'S EARLY PHASE UNIT LOCATED IN MANCHESTER, UK