Duchenne Muscular Dystrophy Trial **Case Study**

RAPID ROLL OUT OF HOME TRIAL SUPPORT ENABLES VULNERABLE PATIENTS TO CONTINUE IN DMD TRIALS

Working closely with a global biotechnology company on a mission to engineer precision genetic medicine for rare diseases, MRN has delivered more than 180 home visits per month to keep trials running across a vulnerable, pediatric population during the COVID-19 pandemic.

Duchenne Muscular Dystrophy (DMD) is a rare disease caused by a genetic mutation. It effects approximately 1 in 3,500 -5,000 males born worldwide. It leads to the muscles in the body becoming weak and damaged over time and is eventually fatal.

The overall aim of this study was to evaluate the impact of a new IMP to treat the symptoms of DMD.

Two phase study

Administered in two phases, the pediatric study starts with patients aged 7 to 13 and goes up to young adults. During initial treatment the patients go through a phase of double-blind IMP administration before moving into an open label phase. Once that is completed the patients can move into a long-term extension study where patients can remain on treatment for up to 5 years.

> *"The idea is to have drugs licensed and moved to market; therefore, the extension phase is keeping the patient going on treatment to plug the gap until the drug goes to market, plus capture long term data."*
>
> Senior Project Manager, MRN

MRN / Duchenne Muscular Dystrophy Trial Case Study

Indication:
Duchenne Muscular Dystrophy

Australia, Belgium, Denmark, France, Israel, Italy, Poland, Spain, Sweden, UK

3 Studies

60+ Sites

90+ Number of patients

2680+ Total Number of visits

2018/2026 Ongoing since 2018 to 2026

Reducing vulnerability with home visits

Recruitment of patients for the trial has been simpler than many others, as the site teams recognize the benefits of MRN's Home Trial Support service to support this vulnerable patient population as they move through this suite of trials.

At site, multiple function, endurance, and capability tests are carried out, to evaluate muscle function status in the patients.

In weekly home visits, MRN's specialist healthcare professionals complete an IMP infusion and carry out regular vital signs and concurrent medication checks.

As a result of COVID-19, MRN was asked to integrate additional visits into the ongoing schedule. A number of the safety blood draws, which would normally be done on site, were completed as additional Home Trial Support Visits to ensure the safety of these vulnerable patients and reduce their risk of contracting COVID-19 during travel to and from the site.

> *"To be able to administer the IMP, many of the patients are given an intravenous port - a permanent fixture attached to the vein. Some patients have to be cannulated at every visit, which if done repeatedly over a long time can damage the vein. There is a reluctance to give the patients ports or devices until they reach a certain age, as they are still growing; so, it is essential that specialist, trained healthcare professionals carry out the home visits."*
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> Senior Project Manager, MRN

Patient onboarding

For 18 months from the end of 2019 and the majority of 2020 MRN was setting up sites and onboarding new patients for the initial study phases in Belgium, France, Israel, Italy, Spain, Sweden and the UK, while keeping the initial trial and the open label extension (OLE) phase running.

Almost all of the patients moved from the initial study into the OLE once this became available to them, and most are using the same healthcare professionals to maintain consistency and familiarity with the patients and their families.

This collaborative approach led to a vast increase in patient sign ups to the trial and the adoption of home trial support.

Protocol practices

In 2020 a requirement for additional safety testing was identified and a new protocol was rolled out.

The timing of applicable Ethics and Regulatory submissions vary from country to country, so some countries were approving at different stages. The study has moved on rapidly through the protocol amendments, with others expected in the future.

At any one time up to five different protocol amendments were being worked on across two studies at various sites in multiple languages. All the while MRN was onboarding sites for the OLE for this patient group. Some healthcare professionals also look after patients on both studies and so MRN had a complex job training everyone on all protocols, yet successfully managed with only three visits with minor protocol deviations during the whole process.

Significant success

"I'm so proud of the team for this last year; bringing on new sites, new countries, working through the COVID-19 pandemic, and successfully implementing a number of protocol changes! These are complicated studies with a vulnerable patient group, but it's so good to see the long-term impacts of a project like this. The ultimate reward would be that the MRHA approve the drug and the patients can remain on it licensed."

Senior Project Manager, MRN

Medical Research Network Ltd Talon House
Presley Way
Milton Keynes
Buckinghamshire
MK8 0ES
United Kingdom

Medical Research Network Inc.
540 Lake Cook Road
Suite 400
Deerfield
IL 60015
USA

Medical Research Network Japan KK,
32F Shinjuku Nomura Building,
1-26-2 Nishi Shinjuku Shinjuku-ku,
Tokyo 163-0532
Japan

Calle Francos Rodriguez N:51 Chalet 25
28039
Madrid

Medical Research Network Germany GmbH Zettachring 12A
70567
Stuttgart
Germany

E enquiries@themrn.co.uk
W www.themrn.io