

21st November 2024

To the Myotubular and Centronuclear Myopathy community

It is with regret that we announce that the current clinical trial of tamoxifen in patients with x-linked myotubular myopathy has been halted.

This trial “**TAM4MTM: A Phase 1/2 Randomized Placebo-Controlled, Double-Blinded, Single Crossover Study to Determine the Safety and Efficacy of Tamoxifen Therapy for Myotubular Myopathy (XLMTM)**” enrolled patients in Canada and the UK over the period 2019 to 2023.

Upon safety review we have found that tamoxifen may impact liver function in children with XLMTM. For example, blood tests showing some liver enzymes that were higher than we would normally expect. As a result of these findings, it has been decided that the tamoxifen study will be halted. At this time, we are not concerned about any long-term effects related to tamoxifen and the liver for those participating in this trial, as the liver test results returned to normal once the tamoxifen was stopped.

We will continue to conduct and finalise analysis of the data collected in this study. These results will be available on clinicaltrials.gov and will, at some point, be presented in publication format which we will ensure is shared with the myotubular and centronuclear myopathy community.

Given the safety concerns raised in the trial, we strongly encourage patients/ families/ clinicians to NOT consider “off label” tamoxifen for XLMTM patients.

We would like to extend our thanks to those who participated in this study. While the data may not reflect the results that we hoped for, we believe that the learnings from this trial will help to inform on, and continue progress towards, developing therapies for XLMTM.

We also wish to thank the broader MTM & CNM community for their support, and the agencies and charities who enabled the development of the tamoxifen clinical trial.

Myotubular Trust will host a webinar **on Tuesday 3rd December at 5pm UK time**, where we will discuss the ending of the trial, and what it might mean for our understanding of XLMTM. We will address any questions you may have at that webinar.

Sincerely,

Dr Giovanni Baranello,
Great Ormond Street Hospital, London

Dr Jim Dowling,
Hospital for Sick Kids, Toronto