INTERNATIONAL ALLIANCE OF ALS/MND ASSOCIATIONS

# Collaborative Medicinal Development – CuATSM
# Spring 2020

## Background

An Australian company called Collaborative Medicinal Development, LLC are clinically developing a compound called CuATSM. In 2018 results were released from a phase 1 clinical trial that was designed determine only if CuATSM is safe for humans and to determine what dose (if safe) would be ideal to test in a further clinical trial (phase 2 or 3). Later trials would be designed to further examine safety and to eventually determine if it has the ability to alter ALS disease progression. Specific aspects of the phase 1 trial make it not ideal for interpretation of CuATSM effect on disease progression.

On January 7th 2019, television spots and a media release stated that CuATSM slowed disease progression by 70% in the phase 1 clinical trial participants. This resulted in an international outcry for people living with ALS to access CuATSM, with particularly strong interest in Australia and the UK. Online petitions yielded a need for response and questions have arisen in multiple Alliance organization countries. A need for a balanced and scientifically accurate response that does not ruin the optimism around CuATSM's potential is needed.

In late 2019, a phase 2/3 clinical trial of 80 people started recruiting in Australia (NCT04082832). It is setup to give a better indication of a treatment effect than the phase 1 trial, though the sample size would require a very large slowing of disease progression to be significant.

## Recommendation

**The SAC recommends that Alliance members refer to CuATSM as a drug that has shown potential to treat ALS in laboratory animal models and was recently studied for human safety in a small phase 1 clinical trial by the company Collaborative Medicinal Development, LLC in Australia. At the doses tested, using clinical grade CuATSM, it was considered safe, but a press release also states that the company has seen a substantial slowing of disease progression. This clinical trial was not designed to make such a public statement and a number of aspects in its design require this result to be taken with as much caution as possible. Essentially, there is no substantiated scientific evidence that CuATSM has any advantageous effect on ALS in humans. Furthermore, until there is a peer-reviewed publication of the data, it is not possible at this time to evaluate the safety beyond the claims of the release.**

**The ongoing phase 2/3 clinical trial will futher assess safety at the chosen dose and is designed to get a better indication of a potential to positively affect ALS disease course. The trial size is small and caution may again need to be taken when interpreting the results. The field remains hopeful that CuATSM will work, but the proper trials need to be done before anything can be known.**

**Please refer to the MND Association blog for a more detailed explanation:**
https://mndresearch.blog/2019/01/15/whats-the-story-with-cuatsm/