



Phase 3 Study of Dexamipexole in ALS/MND

Information for MND Association members

Dexamipexole:

Dexamipexole (formally known as KNS-760704) is a newly synthesised drug created by Knopp Neuroscience Inc. Although it is not currently known how this drug works in the body, laboratory studies have demonstrated that this drug has neuroprotective properties. A phase II trial of KNS-760704, completed in 2009, found that the drug was safe and well tolerated by people with MND for up to 9 months. The trial results also showed trends suggesting the drug's potential for reducing the rate of decline in patients' functional capability, although the relatively small numbers of people involved in the study means that these findings cannot be relied upon.

During 2011 recruitment will commence for a phase three study of the drug dexamipexole at 61 study locations in the United States, Europe and Australia. The purpose of this study is to determine whether dexamipexole (150 mg twice daily) is safe and effective in the treatment of ALS/MND.

This is the first time that an international phase three clinical trial has included Australian sites. This development is testament to the work of Australian clinicians in collaborating and engaging with their international peers.

The trial will be spread over 61 sites so only a relatively small number of people will be recruited to the trial in Australia. In addition there will be very strict inclusion and exclusion criteria. These are specified on the website link below.

The trial will be a randomised, double blind, placebo-controlled study which means that half of the participants will be given a placebo (not the drug) and half will be given the drug. All involved will be 'blind' as to whether the participant is taking the placebo or the dexamipexole.

The research sites in Australia will be the MND Clinics at:

- Bethlehem Hospital, Caulfield South, Melbourne
- The Royal Brisbane and Women's Hospital, Herston, Brisbane
- The Prince of Wales Hospital, Randwick, Sydney
- St Josephs/Westmead Hospital, Westmead, Sydney

These sites are currently seeking ethics approval in preparation for when recruitment commences. This may take some months.

For more information and updates regarding recruitment see:

http://clinicaltrials.gov/ct2/show/study/NCT01281189?term=ALS&rank=77&show_locs=Y#locn

Information for members – clinical trial



The US National Institute of Health Clinical Trial website lists 501 ALS/MND studies that have been completed, are currently recruiting or are pending.

<http://clinicaltrials.gov/ct2/results?term=ALS>

About Clinical Trials:

Clinical trials are research studies in human volunteers that determine whether potential treatments are safe and effective. It is extremely important to establish that the side effects of any new drug are not more threatening than the disease itself and to prove beyond reasonable doubt that the drug is beneficial. The only fool proof way of doing this is by monitoring the effects of the drug in a group of patients and comparing the progress of these patients with the progress of a similar group not taking the drug.

Clinical trials are divided into four phases:

Phase I examines the safety of the potential new treatment, often in just a few (5 – 20) people. In many cases, this phase involves healthy volunteers rather than patients. Participants are monitored for adverse reactions or side effects; if any appear that are judged to be too dangerous, the drug will not advance any further through the clinical trials process.

Phase II determines the optimal dose size, timing of doses and drug delivery route (eg by mouth, by injection) for the next phase of testing. Although Phase II testing may provide some indication of the drug's ability to treat the disease, the number of patients involved in this phase is too small for such findings to be relied upon.

Phase III aims to show whether or not the drug actually has a beneficial effect on patients. This stage of testing will usually involve hundreds of patients, which is enough to allow a reliable assessment of the drug's efficacy. Phase III results will determine whether or not a drug is approved for use to treat the disease.

Phase IV occurs after the drug has been approved for sale. With the drug in general use, further data can be gathered on its effects in an extremely large number of people over an extended period of time.

If you require any further information please contact your state MND association or speak to your neurologist.

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