

## **FREQUENTLY ASKED QUESTIONS:**

### ***MND study provides exciting new focus for potential drug treatments***

#### **1. Tell us more about the research. What did you find in this study?**

Researchers at the Euan MacDonald Centre for MND Research based at the UK Dementia Research Institute in Edinburgh created a model of motor neuron disease (MND) in a dish using stem cells from people with the most common inherited form of MND (the *C9orf72* mutation). They showed 3 things: (1) that the motor nerve cables (axons) are damaged; (2) that the energy source, or batteries (mitochondria), within the motor nerve cables was also damaged; and (3) that if one boosts the faulty batteries, one can repair the damage in the nerve cables. This latter finding was the new discovery and it opens up potentially new therapeutic possibilities. But, this is inevitably, and must be seen as, the first step in a journey.

#### **2. What happens next?**

Researchers in Edinburgh along with many others across the international research community will now work hard to exploit the same platform of human based discovery using stem cells from people with the commonest form of MND (sporadic MND) to screen hundreds and thousands of medicines, drugs, and compounds from nature, in an unbiased manner, to see if they can show the same effect – in other words, find promising candidates in a laboratory setting that boost the batteries of these motor nerve cells.

If they are able to do that, the next step will be to ask the question: “are these medicines safe and effective?” This would need a clinical trial to answer definitively.

#### **3. Could it be a medicine that exists already for other medical conditions and ‘simply’ has to be repurposed to have efficacy when it comes to MND?**

The honest answer is that we don't know, but it is certainly possible. To the best of our knowledge, there is no drug out there that specifically and safely targets the cellular batteries in motor nerve cells. But, that does not exclude the possibility that there may be medicines already out there that can do this.

And so what researchers can now do using the platform described above, using human stem cells, is screen many compounds, in a comparatively short time, to see if they can find the potential ‘magic bullet’, that does the trick ‘in the dish’. Once that discovery has been made, we would have got the bait to begin to think about accelerating its potential impact via formal evaluation using human clinical trials.

#### **4. How long will this likely take?**

We are cautiously hopeful that medicines that can begin to slow the disease will be identified in the coming decade. Our researchers are committed to making a difference and without your support and strength we would not be where we are now. We are now well placed, owing to advanced trial platforms, such as TRICALS and MND-SMART, coupled with the close collaboration between scientists and clinicians, to bring at pace any laboratory discovery into delivery through clinical trials. However, at the present time, there are no changes to the investigational medicinal compounds being tested in MND-SMART (trazodone and memantine).

**5. Is there anything that people with MND can be doing now to boost the batteries in their motor nerve cells?**

We are not aware of any proven means of boosting the cellular batteries in motor nerve cells in MND. One has to ensure that any intervention is both safe and effective and as such at the present time we cannot make any recommendations.

Many thanks for your interest and support.

Best wishes,

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THE UNIVERSITY  
of EDINBURGH



**MND-SMART**  
Clinical trials for MND



**MY  
NAME'S  
DODDIE**  
foundation

**mnda**  
motor neurone disease  
association

