

## **FAQs: Tofersen**

This document is designed to answer questions you may have following the publication of the Tofersen trial results.

It is best to direct specific questions about your own situation to your neurologist or MND specialist.

### **What is Tofersen?**

Tofersen is a potential treatment which targets a specific genetic mutation within a specific gene (called SOD1) thought to cause MND in around 2% of people living with MND. This genetic mutation results in the formation of abnormal SOD1 protein which is toxic to motor neurones. Tofersen has been designed to stop the production of this abnormal protein.

### **What do the trial results mean?**

108 patients took part in the Tofersen clinical trial, funded by the biotechnology company Biogen.

Results of the open label extension of the Phase 3 clinical trial have shown significant improvement in patient mobility and lung function after 12 months. Researchers found that taking the drug for 12 months identified the best physical improvements but did show some improvement at six months. The results also showed that the group of participants who received Tofersen six months earlier in their disease lived longer than participants who started Tofersen six months later.

This latest trial data suggests that the earlier Tofersen is given to a person with MND the more beneficial it may be.

### **When will Tofersen become available in the UK?**

These results are promising but the drug has not yet been approved for use for MND anywhere in the world.

However, the drug is currently under review by the Food and Drug Administration (FDA) in the USA. A decision on approval is expected by January 2023, after which it could be made available to people living with MND with the SOD1 mutation in the USA.

At present the sponsor of the study (Biogen) has not discussed what the approval will look like in the UK. However, we would expect approval to be sought through the usual channels. We are in discussions with Biogen about this and will share information as we have it on our usual channels.

### **Can I access the drug now?**

Possibly but it is a complex process. If you have been diagnosed with SOD1 MND, there is an early access programme available which allows participants access to Tofersen without being on the clinical trial and before it has been approved. Your neurologist needs to go through the process for you. Please discuss this with them directly. The process for neurologists is available on [Biogen's website](#).

**I have familial MND. Will this drug help me?**

At present, Tofersen has only been tested in people with MND who have a SOD1 gene mutation.

These results provide increasing confidence that Tofersen is having a beneficial effect in people living with SOD1 MND. We hope that this work will lead to other gene therapies which may be helpful for people with another genetic basis of their MND.

**I have sporadic MND. Will this drug help me?**

No. This drug has been specifically designed to treat familial MND caused by mistakes in the SOD1 gene.

**How do I get genetically tested to see if my MND is caused by a particular gene?**

In the first instance you need to speak to your healthcare professional. It may be possible. However, the UK National Genomic Test Directory criteria allow genetic testing only in people with a family history of MND or in those with MND who showed symptoms before the age of 40.

**Does the outcome mean we're closer to finding a treatment for other types of MND?**

We hope so. There are further studies looking at genes known to play a part in the development of MND or the speed of progression. We hope the learnings from the Tofersen trial findings can help to accelerate the development of therapies that will be useful for the disease.

**Would Tofersen slow progression or halt it?**

At this point, it is too early to tell. Trials like this are a positive step to understanding the extent of how much the drug could impact progression. The researchers involved in the trial and its analysis are suggesting some changes in disease progression occurred.

**Is there a clinical trial for Tofersen happening at the moment? How can I get on it?**

There is a phase 3 trial called ATLAS which is ongoing. This trial is for people who have not yet been diagnosed with MND but have the SOD1 mutation. This trial is looking to determine the value and optimal timing to begin treatment with Tofersen in people who have a SOD1 gene mutation but have not yet started to show symptoms (pre-symptomatic). The trial is currently recruiting in the UK at a site in Sheffield. Interested participants can contact the MND Care Centre Coordinator on 0114 222 2263.

**Statement:**

Dr Brian Dickie, Director of Research at the MND Association said "These latest results provide increasing confidence that Tofersen is having a beneficial effect in people living with SOD1 MND and will hopefully provide proof of concept that gene therapies may be helpful for other forms of MND. We are closely following the recent news that Tofersen will be reviewed by the FDA in the U.S. and are in contact with Biogen to discuss what the regulatory approval process will look like in the UK."

**MND Association  
September 2022**