



All-Party Parliamentary Group on Motor Neurone Disease
House of Commons
London
SW1A 0AA

Karin Smyth MP, Minister of State for Health (Secondary Care)
Department of Health and Social Care
39 Victoria Street
London
SW1H 0EU

Cc: Lord Vallance, Minister of State for Science, Research and Innovation

28 March 2025

Dear Minister,

Access to tofersen through Biogen's Early Access Programme (EAP)

We are writing to you as officers of the All-Party Parliamentary Group on Motor Neurone Disease (MND). MND is a fatal, rapidly progressing disease that affects the brain and spinal cord. It kills a third of people within a year of diagnosis and more than half within two years. MND leaves people locked in a failing body, unable to move, talk and eventually breathe.

There is no cure.

But there is hope for people living with a rare form of MND caused by a mutation in the SOD1 gene which affects a small proportion of the MND population, estimated at 60-100 people in the UK at any time.

Tofersen is a new and promising treatment for MND, developed by Biogen. It's the first effective treatment since riluzole in the 1990s. While riluzole only slightly extends life, tofersen can significantly slow the disease, offering people with SOD1 MND the hope of living many more years. Tofersen has already received marketing authorisation from the Federal Drug Administration in the USA and the European Medicines Agency.

Tofersen has not yet been approved for use in the UK, but Biogen is in discussions with regulators. For now, Biogen is offering the treatment for free to the NHS for people with SOD1 MND through their Early Access Programme (EAP).

However, the APPG on MND has been made aware of eligible patients with the SOD1 gene that are unable to access tofersen through the EAP due to capacity constraints. Tofersen is delivered via lumbar puncture which requires expertise in this form of drug administration such as neuroradiology and pharmacy support. However, services are not commissioned to deliver the treatment which means many are offering the treatment as 'goodwill' above and beyond their usual commissioned

responsibilities. This means that people with SOD1 MND are missing out on a transformative treatment with the potential to significantly slow disease progression and extend life. This is despite Biogen making the drug available and free of charge to the NHS.

Based on engagement with care centres across the country, the MND Association estimates there are up to 12 eligible people with SOD1 MND who are unable to access tofersen via the EAP. This is not only a cause of concern for the MND community but should be for the NHS, too. Services are benefiting from an innovative treatment, at no cost, which significantly improves patient outcomes.

This is causing significant distress to those people who have been diagnosed with a terminal illness, discovered there is an effective treatment available, but then have been told they cannot access it due to capacity constraints.

We are writing to you to ask if you would work with partners at NHS England to find a solution that would ensure people with SOD1 MND are able to access tofersen through the EAP.

A modest increase in support would make a significant difference, ensuring these individuals are not denied the opportunity to benefit from a potentially life-changing treatment.

If a solution is not found, these patients will likely experience significant disease progression and a reduced life expectancy.

We look forward to hearing from you and commend you on the important work you and the department are doing to support people with motor neurone disease and other neurological conditions.

Kind regards,

Ian Byrne MP

Chair, APPG on MND

MP for Liverpool West Derby

Aphra Brandreth MP

Vice Chair, APPG on MND

MP for Chester South and Eddisbury

Michael Payne MP

Vice Chair, APPG on MND

MP for Gedling

Olly Glover MP

Vice Chair, APPG on MND

MP for Didcot and Wantage