10 April 2024

The Rt Hon Andrew Stephenson CBE MP
Minister of State for Health and Secondary Care
Department of Health and Social Care

Dear Minister,

We write as a group of people living with motor neuron disease (MND), representing the wider MND patient community, to express our distress at a recent decision by NICE regarding a breakthrough treatment for our disease.

The National Institute for Health and Care Excellence (NICE) has recently refused to accept an application from the pharmaceutical giant, Biogen, to follow the most suitable pathway for assessing the marketing/licensing of its proven, effective and life changing treatment, Tofersen, for motor neurone disease (MND) specifically caused by a mutation in the SOD1 gene.

Already approved in the USA and in Europe, the refusal is a body blow to patients and a potentially monumental setback for personalised medicine in the UK. Above all, it sends out the message that the UK is not open for business to the world's leading pharmaceutical companies and their innovative and proven treatments. It is even more shocking given that leading UK research establishments have been at the heart of the trials for this treatment.

Eleanor Dalley, who lives with the specific genetic mutation and is accessing the drug via an early access program (EAP), funded by Biogen, says:

> "I'm the fourth person in my family with SOD1 MND and the only one still living. This treatment was the miracle my family have prayed for but now it looks like it could be withdrawn. This decision makes me so angry. Are our lives worth so little? The UK system is failing us!
>
> For NICE to ignore the results of the trial and the leading UK neuroscientists is unbelievable. It is the first treatment in 30 years shown to work by prolonging lives. It is likely to prevent people from developing SOD1 MND in future, including my daughter and other family members. If this lifeline drug does not get the necessary approvals, hope dies for helping future generations."

SOD1 patients and Patients United have been buoyed with the support of Geoff Burrow, father of rugby league player, Rob, who lives with the disease and does so much to raise awareness and funds for it. He says:

> “Rob does not have the SOD1 gene, but the Burrow family is united with all of those living with MND. The fact that Tofersen works, even if only for a small percentage of patients, is a major breakthrough showing a pathway to discover other drugs targeted at specific genes known to be involved. We must change the NICE decision and give hope to the whole MND community.”

We fully support the recent letter of 27 March to you from Professor Ammar Al-Chalabi and Professor Chris McDermott, co-directors of the UK MND Research Institute, seeking an urgent meeting to discuss the impact on people with MND but also on research and pharmaceutical investment in the UK.

We urge you to demand that NICE reverse their shortsighted decision.

Yours sincerely,

Geoff Burrow (parent of son with MND)
Rob Burrow
Stephen Darby
Eleanor Dalley (living with SOD1 MND)
Carol Anderson
Nicola MacFarlane
Emma MacLennan (parent of son lost to a genetic variant of MND)
Lee Millard
David Setters
Jennie Starkey
Nicola Waters