**MEETING OF THE ALL-PARTY PARLIAMENTARY GROUP ON MOTOR NEURONE DISEASE**

**Tuesday 1st April 2025**

**11:00 - 12.00**

**Meeting Room R, Portcullis House, Westminster**

| Attendees | External Guests | Apologies |
|---|---|---|
| Ian Byrne MP<br>Lord Bellingham<br>Patrick Hurley MP<br>Christine Jardine MP<br>Douglas McAllister MP<br>Aphra Brandreth MP<br>Chris Evans MP<br>Sarah Hall MP<br>Ben Lake MP<br>John Slinger MP<br>Michael Payne MP<br>Tim Farron MP<br>Sir John Hayes MP<br>Representative of Lord Dubs<br>Representative of Emma Reynolds MP | Professor Ammar Al-Chalabi<br>Dr Jane Haley, MND Scotland<br>Dr Mike Rogers, MND Association<br>Dr Nick Cole, MND Association<br>Niall Murphy, MND Association<br>Sian Guest, MND Association<br>Jennifer Mills, MND Association<br>Richard Evans, MND Association<br>Emma MacLennan, Office of Lord Dubs<br>Sean McGrath, My Name's Doddie<br>Colette McDiarmid, MND Scotland | Olly Glover MP<br>Vikki Slade MP<br>Frank McNally MP<br>Monica Harding MP<br>Roger Gale MP<br>Sir Nicholas Dakin MP |

**Meeting Notes:**

1. **Welcome and Introductions**

Ian Byrne MP welcomed colleagues and thanked them for attending. He thanked members who joined the recent visit to King's College London and Professor Al-Chalabi's team for hosting the Group.

He then outlined the Agenda for the meeting.

2. **Results of the Priorities Survey**

Ian Byrne MP updated the Group regarding the results of the APPG on MND Priorities Survey, which was launched with the MND Association to better understand the challenges facing the MND community.

Ian Byrne MP thanked the MND Association Team for their work on the survey and the Community for completing it.

Over 300 completed the survey, 36% of whom were living with MND and the remaining 64% comprised of families, carers, and friends who had been affected by MND.

Top 3 things mentioned were as follows:

- Research and finding a cure (56% of respondents)
- Provision of care (20 of respondents)
- Financial support + cost of living (19% of respondents) but this might have changed given the recently announced Department for Work and Pensions reforms.

A full breakdown will be made available with minutes of this meeting.

Research was the top priority unsurprisingly given there is no cure. This is why our meeting today will focus on MND research.

**3. The clinical trials process: how drugs are discovered, developed and approved in the UK**

Dr Mike Rogers from the MND Association outlined the challenges around developing new treatments for neurodegenerative diseases and the need for more involvement of industry and more Government funding. With these, the landscape may change in the next 5-10 years.

The current treatment options available for MND are limited. The only approved medication in the UK is Riluzole which has been shown to slow disease progression by a few months. Some more recent data suggests it might slow disease progression more than initial clinical trials suggested but it is not a cure.

The drug development process is complex and challenging and requires collaboration from lots of stakeholders including researchers, people living with MND, regulators, and healthcare professionals. The complex nature and heterogeneity of MND presents significant challenges in drug development. There are a limited number of disease models that accurately replicate MND pathology.

The UK is a key player in MND research. We lead a lot of pre-clinical work and clinical trials and are involved in almost every important international trial. Pharmaceutical companies are particularly keen to be involved with the research happening in the UK.

The leading MND charities in the UK work closely together, including MND Scotland, the MND Association, My Name's Doddie, LifeArc and the UK MND Research Institute (UK MNDRI) who play a crucial role in how we tackle this condition together.

Dr Nick Cole from the MND Association gave an overview of some of the current clinical trials including EXPERTS-ALS and MND SMART. He discussed how MND community participation is crucial to making these happen. We are hopeful for some positive results from these.

Dr Mike Rogers guided the Group through the journey from research lab to NHS prescription. The timelines are highly variable and the process can take a long time.

- Starts with identification of potential therapeutics in the laboratory, this process takes about 10 years before the drugs move to clinical trials
- Clinical trials have 4 phases
- Around 70% of drugs get through Phase 1
- Phase 2 is a two-step process looking at how much of a drug should be given and how well it works. This is the most difficult phase to pass, with only 30-40% of drugs progressing through this stage
- Phase 3 tests a drug in large groups (hundreds of people) to evaluate efficacy before engagement with regulators
- Phase 4 involves monitoring in the broader population (thousands of people) after regulatory approval. Drugs are still monitored for safety throughout and beyond this phase and drugs are sometimes taken off the market after this point

The first regulatory engagement is with the Medicines and Healthcare products Regulatory Agency (MHRA), who regulate medicines supplied in the UK. The activity of the MHRA spans the entire medicine life cycle to ensure they are safe, effective and of high quality. If they are satisfied, a drug receives marketing authorisation and can be made available to buy privately at a price set by the manufacturer.

But we want drugs available on the NHS, which requires engagement with the National Institute for Health and Care Excellence (NICE) who decide whether a drug represents a cost-effective use of NHS resources. They conduct a technology appraisal of the drug which involves two routes: the Standard Technology Appraisal and the Highly Specialised Technology Appraisal.

Dr Mike Rogers noted that NICE decisions apply to England. In the other nations these are deferred to:

- Scotland: The Scottish Medicines Consortium (SMC)
- Northern Ireland: The Department of Health
- Wales: The All Wales Medicines Strategy Group (AWMSG)

The MHRA have a introduced several new processes to expedite approval of new medicines, which has been quite lengthy in the past.

Dr Nick Cole provided an update on Tofersen, a drug used for a small number of people with a genetic form of MND called SOD1 MND. It slows down and in some cases completely stops disease progression. It has been approved for use by the FDA and the EMA but not yet by NICE who incorrectly considered all forms of MND the same so appraised the drug down the incorrect route. It will now be considered using the Highly Specialised Technologies (HST) criteria.

In the meantime, the company who developed the drug, Biogen, are making it available for free for people who are able to access it, but not everyone is able to and we want to change that.

The development of Tofersen is a significant moment in MND research and a good example of how much hope emerging therapies are showing for some forms of MND like SOD1 MND and changes in the FUS gene. The regulatory landscape needs to better understand MND and adapt review processes and guidelines to ensure timely access to safe and effective treatments.

### 4. Question and Answer Session with the Expert Panel on MND Research

Lord Bellingham asked the panel why, if Tofersen was discovered in this country and developed by a UK company, are we behind the curve in terms of licensing?

Dr Nick Cole highlighted that the project was international but the research that underpinned it was carried out in the UK and the discovery of the biomarker in clinical trials which led to the approval by the MHRA was UK research.

Professor Ammar Al-Chalabi reaffirmed that the drug partly discovered in UK (the biomarker) with support from the US-based company Biogen. NICE initially decided to route through it through the standard technology appraisal (STA) route but this has very low thresholds so was unlikely to be approved as it is very specialised. The Team had to explore with NICE why they made this decision and explain why we didn't agree. It took some time to put it through HST which led to about 6-12 months delay.

Lord Bellingham also noted that these issues are being experienced by people with other neurological diseases e.g. parkinson's and vascular dementia. Are those organisations working closely with the panel to look at some of the common causes?

Dr Jane Haley noted that there is collaboration between different fields as there is likely to be shared mechanisms that underpin these diseases as neurons are dying in all. There are researchers who work on both, for example, there are researchers in the Dementia Research Institute who are also working on MND.

There is much interest in MND being the leading disease because we can do trials on a short timescale and get information relatively quickly and because the genetic causes are well understood (around 10% of cases). There is lots of collaboration but there could be more and hopefully the UK MNDRI can play a role in this.

Aphra Brandreth MP updated the Group regarding a constituent who is receiving Tofersen. It was great to hear first-hand from her about the positive impact of the drug. She has to travel two hours once a month to receive the drug. There are two questions arising from her case:

- She discovered she had SOD1 MND because her brother had died from MND and happened to have a conversation who suggested genetic testing and she was then able to access Tofersen. Are people getting the genetic testing they need to access these drugs?
- What can we do to help people like her to access it closer to home?

Dr Nick Cole stated that not everyone with a genetic change develops MND, but genetic testing capacity is low across the UK and is not available to everyone. There are similar issues with the delivery of Tofersen in that it must be delivered via lumbar puncture and not everywhere in the UK has the capacity for this.

Professor Ammar Al-Chalabi noted that his team offer genetic testing to everyone that comes to the clinic and it takes around 45 minutes to go through the process but the results can take up to a year which can be useless given the rapid progression of the disease. The Team get around this by requesting the results urgently which can help get results within 3 months but in other parts of the country it takes nearer to 18 months. The system also needs to be more patient friendly.

In terms of delivering Tofersen, it is delivered monthly via lumbar puncture which is not difficult but is not particularly pleasant. To do it, you need a hospital stay, a bed, staff etc and there is a staffing problem so even though the drug is available for free, the resources are not always there to deliver it. The staff need specialist expertise and knowledge and confidence to deliver which is not always the case. At King's, it is being provided by the NHS on good will to 8 people but now there are more who we can't give it to which is a terrible situation.

Christine Jardine MP asked about genetic testing. If the tests can take a year, they know someone who's died within a year so that is a significant problem. They were also not the first person in family with MND so could we move towards wider capacity for genetic testing e.g. all family members?

Professor Ammar Al-Chalabi noted that predictive testing is available but unless the family has SOD1, it is not something that is recommended for various complex reasons. But if other family members have SOD1, it is worth doing and the ATLAS trial is available for this at the moment.

Christine Jardine MP asked Dr Jane Haley whether the situation was the same in Scotland.

Dr Jane Haley MP outlined that in England, people with MND can request genetic tests and the difficulties are time frames. In Scotland, the time frames for delivery of the results are very fast. However, not everyone with MND is eligible for genetic testing. There are eligibility requirements which are interpreted differently on the ground.

Ian Byrne MP noted that the Group Officers are sending a letter to Karin Smyth MP, Minister of State, to ensure we can unblock access to Tofersen. This will be actioned this week.

Dave Setters said that during the campaign for the £50 million, we discovered that around £4-4.5 million was being spent on targeted MND research so we've got an extra £10 million per year due to the campaign. In USA, around 200 million spent on ALS which I believe includes lots of wastage. With relatively little money, Ammar and his team have come up with a work plan which has made progress on building sustainable research. Nick mentioned the EXPERTS ALS screening platform, which can reduce phase 2 to around 6 months before going to full trials. This is attracting international interest. Imagine what we can do if the money keeps coming in. If Ian Byrne MP and colleagues can help us, we can get there with Ammar and his colleagues.

Eleanor Dalley stated that she is a Tofersen patient and has been receiving the drug since the end of 2022. It took 18 months of fighting to get access and getting on to some of the clinical trials can be difficult. We learned this morning that the Lighthouse trial has stopped, members of the community are quite upset with the news this morning because they can no longer access the drug. We need impact quickly to see if these drugs work.

My desire to get Tofersen on NHS is important as she has lost 4 family members to MND. In the time since work with NICE, she has lost another family member who could have had access to the drug. People in the country right now with SOD1 who cannot access the drug and Eleanor noted that she is fearful for her child and other family members. Tofersen needs to be more available in more locations and we need the help of MPs to make this happen in some local areas.

Seckin McGuirk noted that this meeting means a lot to her and other patients. She was diagnosed with MND in 2023 and this was confirmed during 2024 but is still waiting for results of genetic testing. They had a test done in Turkey and results received within 4 weeks. She was a teacher and had to retire because the illness progressing rapidly and every passing day costing her. It is clear that every trust works differently so if you are in the right postcode you can access Tofersen but if not, you can't. She said "*It feels as though we are fighting constantly, which is very emotional and upsetting for myself and my family*". She asked how we can make this more fair so we can all be included with such an expensive drug being provided to the NHS for free and only handful of people who need it?

Dr Mike Rogers said that this is not an isolated case and the MND Association is working with MND care centres across the UK to get an understanding of the scale of the issue.

Ian Byrne MP asked if we have any idea of the scale?

Richard Evans stated that we do not have an exact answer but we think it is around 10-12 people who can't get it [Tofersen] currently and we think there are roughly 40 people who are getting it. These are not exact figures. We have met with NHS England and they are not minded to push forward to fix it. They don't want to pre-empt NICE's decision. This is why we are pressing ahead with conversations with the Department for Health and Social Care to find a solution while we wait for NICE's decision as we can't let this carry on for months.

Professor Ammar Al-Chalabi said there would also be about 20 people more people diagnosed per year who need it.

Dr Jane Haley highlighted that in Scotland there is a fear of delivery. As the technique is intrathecal, in neurological conditions there is a reluctance to use this technique to deliver drugs because have been some negative outcomes in cancer patients. In Scotland, nowhere will deliver using these techniques so everyone is sent to England. This is something we need to look at. We have sent letters and the tone indicated a resistance to deliver and we need to acknowledge a fear factor among neurologists and health boards. There isn't necessarily a need to change legislation but more to change hearts and minds, to encourage training etc.

Ian Byrne MP noted this is another issue for the Group to raise.

Eleanor Dalley stated that people from the community will travel and it is disappointing to hear that people are willing to take the drug but clinicians fear giving it. She queried whether lumbar punctures were given in Scotland as it is the same process.

Dr Jane Haley noted that it's not necessarily the neurologist that's reluctant, it might be the health board is reluctant give them permission.

Eleanor Dalley noted that it is also scary for the patients.

Dave Setters thanked Eleanor and people with SOD1 for their contributions. He wanted to note that if we get the results of MIROCALS in the near future and they are positive, this resourcing problem will be an issue for many more patients because it is for people with sporadic MND. This is a simple series of injections once a month, but resources are crucial.

Ian Byrne MP stated that the Group will take that up with the Minister as well.

## 5. Any Other Business

Lord Bellingham asked when the next meeting will be.

Niall Murphy responded that the next meeting will be in November, but there will be a Parliamentary Drop In for Global MND Awareness Day on the 19th of June, the details of which will be circulated to Members.

Lord Bellingham requested Parliamentary Questions to the Minister on this issue.
Niall Murphy agreed to provide these.

The meeting was closed at 12:00.