Latest support and research news from MND Scotland

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MND Scotland
Making time count

Friend, an update from the support and research teams at MND Scotland.

Welcome to the latest edition of our newsletter.

In August last year we launched this newsletter to offer dedicated content for the people we support. Our aim was to share the latest news and developments in one place, on a quarterly basis. We asked for feedback on the newsletter and have been refining the content based on your comments.

With that in mind, we heard loud and clear that alongside the help we offer you also want to hear about the hope that research gives us for effective treatments and a cure.

MND Scotland's mission is a world without MND so funding research has been a priority for the organisation since it's inception in 1981. We can only do this thanks to generous donations from our fundraisers. Please tell us what research you'd like to know more about and we will do our best to cover suggestions in future newsletters.

We hope this edition of the newsletter includes more of the news you want to hear about. We're always open to feedback so please continue to share any thoughts you have with communications@mndscotland.org.uk.

Angela Harris, Director of Support Development, and Jane Haley, Director of Research

Update on emerging treatments

There have been a number of updates in the clinical trial and UK drug regulatory pathway space this year. We have tried to highlight most through our website articles as they happen but have also compiled a summary below.

We know that the different clinical trial types can be confusing, so we have produced a short document that provides an outline for you. You can access that here.

AMX0035 /TUDCA

In March, two international phase 3 clinical trials testing TUDCA reported their top line data. The PHOENIX trial, based in the USA and run by Amylyx, was testing TUDCA in combination with another drug. The TUDCA-ALS trial was academic-led and based in Europe, but did recruit in the UK, and tested TUDCA without additional drugs. Both trials did not meet either their primary or secondary endpoints, conclusively demonstrating that TUDCA was not effective at slowing progression of MND.

More information on AMX0035 / TUDCA

MIROCALS

The MIROCALS clinical trial, which tested low dose interleukin 2 and reported interim top-line data in December 2022, which appeared to be positive, has not yet published the full dataset from the trial.

This delay has been incredibly frustrating and upsetting for people with MND. Along with the MND Association, My Name'5 Doddie Foundation and neurologists, we have been continually applying pressure to the trial consortium, asking for timely publication.

In the expectation of the results being published soon, the charities have also contacted the UK regulatory bodies to try and prepare for a possible application, including the MND Association initiating a process with NHS England to request that low-dose interleukin 2 was prioritized through their Medicines Repurposing Programme.

The charities have also worked with people with MND and neurologists to prepare a pack of information that would help clinicians prescribe low dose interleukin 2 under a ‘specials’ route should positive data be published.

Read our most recent update on MIROCALS

MND-SMART

This clinical trial, which is anchored in Edinburgh, continues to recruit across the UK and most people with MND in Scotland are eligible. It is currently the only multi-arm platform trial for testing drugs for MND in Europe and has been incredibly successful at setting up recruitment sites in many areas that have not run trials before.

MND-SMART now has 22 sites in all four nations and has transformed the MND clinical trial landscape in Scotland and the UK. In Summer 2023, the first two drug arms were discontinued when neither memantine nor trazodone showed any benefit for people with MND.

MND-SMART has delivered these clear results less than four years since launching, in half the time it would have taken for a traditional two-arm study. The trial now continues with an arm three drug, amantadine launched in April 2023, and further new drug arms are planned for 2024 and 2025.

Find out more on the MND-SMART website

Bereavement support

Research shows that children and young people need more support when dealing with the loss of a loved one to MND.

We are working closely with bereavement organisations to gather best practice and the most appropriate way to help our children and young people.

This includes working with Global Neuro Ycare, an American organisation that provides the guidance, tools and resources to support children who have a family member with MND, known as ALS in America.

They are launching a new resource, LUKI & the Lights, which will soon be available on our website.

When Anjo Snijders was diagnosed with a form of MND aged 35, he and his wife, Sascha, wanted to explain MND to their young kids in a way that was accessible, fun and thoughtful. Both teachers, Anjo and Sascha began to look for resources to help explain MND to their children. In the absence of anything helpful, they created LUKi.

LUKi & the Lights is a silent film - using no language, only sound, to tell the story of LUKi and MND so it is accessible to viewers around the world.

Stay tuned to our social media channels and website for the launch of LUKI & the Lights, and further resources to support children and young people.

Separately, we received really positive feedback from the first cohort of the seven week Grief Workshop Pilot that ran in February. The aim of the Grief Workshop is to provide individuals with an opportunity to share their experience and find support in a safe setting with others in a similar situation. Email us at support@mndscotland.org.uk for information on future workshops.

Watch a trailer for LUKi & the Lights on YouTube

Spotlight on ...TDP-43

TDP-43 is a protein present in the nucleus of neurons and is important for maintaining a healthy and functioning neuron. In 95% of MND cases, TDP-43 undergoes an alteration that causes it to misfold and form clumps in brain and spinal cord tissue. These can cause damage to the neurons and contribute to their breakdown. Because the brain and spinal cord are not easily accessible, the accumulation of misfolded TDP-43 cannot be identified in these tissues while individuals are living.

Dr Jenna Gregory is an MND Scotland researcher at the University of Aberdeen who has found misfolded TDP-43 in other body tissues in MND patients. By testing historical surgical samples from people with MND they found that TDP-43 clumps can be found in cells from the colon, skin, lymph nodes and gallbladder prior to clinical manifestation of MND.

In March, Jenna also unveiled a new method for identifying TDP-43 clumps in post-mortem brain tissue. The team applied a technique currently used in cancer diagnosis and treatments, called an aptamer. An aptamer is a short segment of Riboneucleic acid (RNA) which can detect and bind to a molecular target, indicating its presence in a sample. In this case the target is mis-folded TDP-43. The sensitivity and specificity of the aptamer allows it to identify very small amounts of the misfolded protein, whereas other techniques cannot demonstrate such small amounts.

This opens up the possibility that aptamers could be used to detect TDP-43 in low levels in living tissues that are more easily biopsied, such as the colon. Although only post-mortem brain tissue has been tested so far, Jenna and her team have taken a step forward in helping to identify possible diagnostic markers and the tools to monitor them. **You can read more about Jenna’s research on her website.**

Following on from the identification of TDP-43 clumps outside the brain, Kristine Roberts, will be undertaking an MND Scotland and Chief Scientist Office co-funded project with Jenna’s group. Kristine's project will investigate the possible role of immune cells in MND, including testing the theory that some immune cells become overworked from clearing the TDP-43 clumps formed in peripheral body tissues before the brain, and so don’t remove them as effectively.

If this is the case, there is the potential to explore adapting therapies currently used in some cancers which focused on re-activating these immune cells, allowing them to resume removing materials from cells.

Read more about Kristine's project on our website

Feedback on grants

Our grants system is designed to help and support you and your family. MND can have a huge financial impact, not just on the person but their loved ones too.

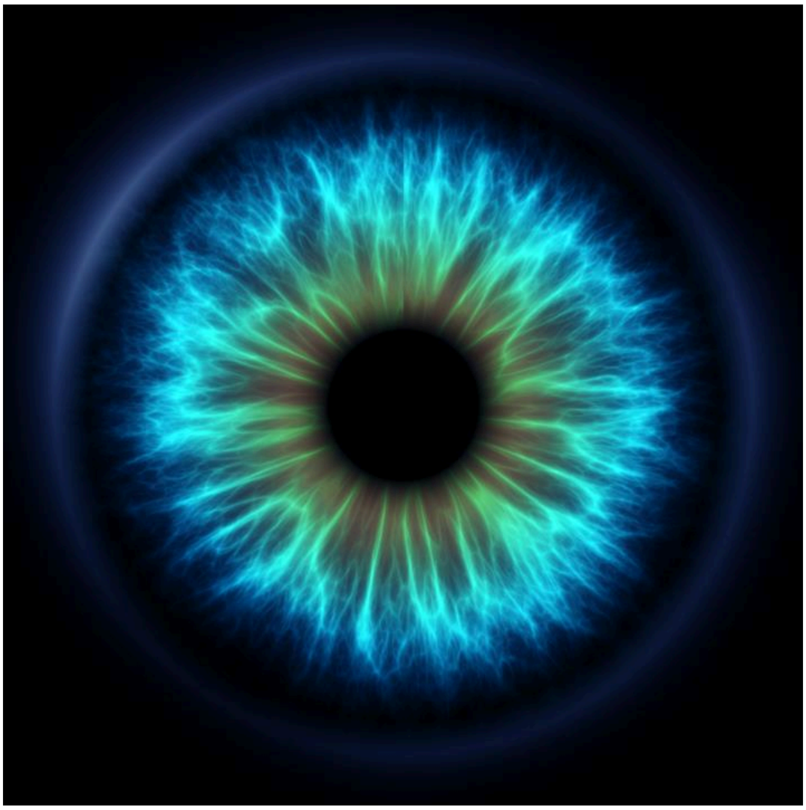
We wanted to share some of the feedback we've received from families telling us what receiving a grant had meant to them:

"*The portable ramp allows me to visit my son and daughter at their homes. I was able to go to my granddaughter’s 7th birthday tea, which wouldn’t have been possible without the ramp.*"

"This grant will make a huge difference to me and my family. As we can continue to have a car that suits my needs at this time. I struggle getting in to certain cars that are lower down. And the boot size allows me to take my rollator in the car. Which means I can get out and about from the house and lead a normal a life as possible."

"*The person with MND had a wish to attend Newcastle Greyhound and football ground. The trip really made him excited and happy; his words were that he wished the day would never end!*

The staff at the greyhound stadium made the visit memorable for him by naming a dog race after him and allowing him to present the winner trainer with a trophy."



Looking to the future of research

Although on the periphery of MND, researchers at the University of Edinburgh have been investigating blood vessel abnormalities and changes in the retina of the eye as a potential marker in neurodegenerative diseases. The pilot study focused on Alzheimer’s but, as the research is progressing, other neurodegenerative diseases are being considered in their future work such as MND, especially when there is also associated frontotemporal dementia.

As they expand their study, the researchers hope to access Scotland’s bank of retinal images from opticians and use artificial intelligence (AI) techniques to identify small changes in blood vessels and retinal cells and identify changes between individuals, including those with neurodegenerative conditions such as Alzheimer's, multiple sclerosis and MND.

They would like to encourage people who suffer from neurodegenerative conditions, and those related to them, to undergo eye tests involving retinal scans. Having a larger number of images to analyse will improve the reliability of the results achieved at the end of the study and, we hope, provide a new research pathway for developing diagnostic techniques for neurodegenerative diseases.

Find out more on their website

MND Insights community survey

We are pleased to support LifeArc to gather views from the MND community on what potential new drugs for MND should look like. LifeArc is seeking to uncover the most important research questions and urgently address the needs of MND research.

Would you like to share your experiences?

The survey is open to anyone who fits one or more of the following criteria:

- Those living with MND

- Caregivers or supporters for someone with MND

- People who have lost someone close to MND

- Those with a gene that puts them at a high risk of developing MND, or those who have a family history of the condition

Take part in the LifeArc survey

Join the International Alliance on Global Awareness Day

On Global MND Awareness Day, 21 June, The International Alliance of ALS/MND Associations is holding a webinar to raise the profile of ALS/MND through the impact of the Ice Bucket Challenge.

They will share the history of research and treatment developments over the past 10 years and how the Ice Bucket Challenge helped on a global scale. They'll be inviting the families of the three founders of the challenge to share their memories, as well as organisations that have used the challenge in their activities.

Sign up on the Alliance website

We welcome feedback on our quarterly newsletter. If you have any comments or suggestions, please get in touch communications@mndscotland.org.uk.

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MND Scotland is the only motor neuron disease (MND) charity providing direct support to people living with MND in Scotland. We campaign to create lasting change and invest in pioneering MND research to find a cure.