HINTS AND TIPS FEBRUARY 2014 from Roland Lewis Editor 01923 720198

Research Survey

We are supporting a new survey enabling people to have their say in setting future research prioroties for palliative and end of life care.
It welcomes feedback from those living with MND, current or bereaved carers, their families, and frontline social and healthcare professionals or clinitions.
Dr Belinda Cupid, our Head of Research said “We are asking for your help because we want to improve care, support and treatment for people in the last few years of their lives.We know that many people have important questions about care, support and treatment, and we want to find out what they are. It is an area that is currently under – researched and this ground-breaking project will be invaluable to help guide our future priorities.”
You can complete the survey online, over the 'phone or by asking for a paper version.
The deadline is 30th April 2014. To take part or find out more, visit www.palliativecarePFP.org.uk or phone 0207 0914153

The News

New research from the University of Sheffield could offer solutions into slowing down the progression of motor neurone disease (MND).

Scientists from the University of Sheffield’s Institute for Translational Neuroscience (SITraN) conducted pioneering research assessing how the devastating debilitating disease affects individual patients.

MND is an incurable disease destroying the body’s cells which control movement causing progressive disability. Present treatment options for those with MND only have a modest effect in improving the patient’s quality of life.

Professor Pamela Shaw, Director of SITraN, and her research team worked in collaboration with a fellow world leading MND scientist Dr Caterina Bendotti and her group at the Mario Negri Institute for Pharmacological Research in Milan, Italy. Together they investigated why the progression of MND following onset of symptoms varies in speed, even in the presence of a known genetic cause of the condition.

The research, published in the scientific journal Brain, investigated two mouse models of MND caused by an alteration in the SOD1 gene, a known cause of MND in humans. One of the strains had a rapidly progressing disease course and the other a much slower change in the symptoms of MND. The teams from Sheffield and Milan looked at the factors which might explain the differences observed in speed and severity in the progression of the disease. They used a scientific technique known as gene expression profiling to identify factors within motor neurones that control vulnerability or resistance to MND in order to shed light on the factors important for the speed of motor neurone injury in human patients.

The study, funded by the Motor Neurone Disease Association, revealed new evidence at the point of onset of the disease, before muscle weakness was observed, showing key differences in major molecular pathways and the way the protective systems of the body responded, between the profiles of the rapid progressing and slow progressing models. In the case of the model with rapidly progressing MND the motor neurones showed reduced functioning of the cellular systems for energy production, disposal of waste proteins and neuroprotection. Motor neurones from the model with more slowly progressing MND

showed an increase in protective inflammation and immune responses and increased function of the mechanisms that protect motor neurones from damage.

The research provides valuable clues about mechanisms that have the effect of slowing down the progression of disabling symptoms in MND.

Professor Shaw said that the state-of-the-art Functional Genomics laboratory in SITraN had enabled the research team to use a cutting edge technique called gene expression profiling.

“This enables us to ‘get inside’ the motor neurones in health and disease and understand better what is happening to cause motor neurone injury in MND,” she said.

“This project was a wonderful collaboration, supported by the MND Association, between research teams in Sheffield and Milan. We are very excited about the results which have given us some new ideas for treatment strategies which may help to slow disease progression in human MND.”

Dr Caterina Bendotti said: “MND is a clinically heterogeneous disease with a high variability in its course which makes assessments of potential therapies difficult. Thanks to the recent evidence in our laboratory of a difference in the speed of symptom progression in two MND models carrying the same gene mutation and the successful collaboration with Professor Pamela Shaw and her team, we have identified some mechanisms that may help to predict the disease duration and eventually to slow it down.

“I strongly believe that the new hypotheses generated by this study and our ongoing collaboration are the prerequisites to be able to fight this disease.”

Brian Dickie from MND Association added: “These new and important findings in mice open up the possibility for new treatment approaches in man. It is heartening to see such a productive collaboration between two of the leading MND research labs in Europe, combining their unique specialist knowledge and technical expertise in the fight against this devastating disease.”

Research news from the ALS International symposium Dec 2013 .
Clinical trials in a dish? Dr Brian Dickie (research director MNDA)

A packed room at the 24th International Symposium on ALS/MND was given a fascinating whistle stop tour covering stem cells, robots and cellular garbage clearing, by Dr Steve Finkbeiner of the University of California, as well as a glimpse into the future of developing ‘disease in a dish’ models of MND. Dr Finkbeiner outlined how his lab is attempting to conduct “clinical trials in a dish” by generating huge numbers of cultured neurons cells for automated ‘high throughput analysis’ of their health and death. As he says, “we’re basically trying to develop a comprehensive physical examination for nerve cells”.

Robotics in MND

The object of this work is of course not only to improve our understanding of how nerve cells function and misfunction, but principally to screen large numbers of potentially protective compounds. To do this, he has developed a sophisticated and sensitive robot-based system that can assess the individual state of health of thousands of neurons in culture over the period of weeks, or even months! A great thing about robots is that they work 24/7 – and this robot even has its own Twitter account to keep the investigators updated on its progress.

Watch the robots in action here:

http://www.youtube.com/watchv=QN9Fx0wyA9U&feature=youtu.be

Like his robots, Dr Finkbeiner is a busy guy, as this technology has exciting implications for

the whole spectrum of neurodegerenative diseases, including Parkinson's disease and Huntington's disease, which he is also working on.
But it was encouraging to see how extensively the MND research community is collaborating with him – including our own MND Association funded researchers Siddarthan Chandran and Chris Shaw.
The monitoring and analysis of these cells produces massive amounts of data, which requires huge computing power, so he has hooked up with Google, who have assigned a team of software engineers (rumour has it from their 'secret' GoogleX lab) to assist with the computational effort.
Just like no two MND patients are exactly the same, so Dr Finkbeiner thinks that we need to treat cells in the dish in a similar fashion, treating each as individuals, but hopefully also stratifying them into selective groups defined by common cellular functions, in the same way that patients may be stratified (eg limb onset vs bulbar onset) in a clinical trial.
Of course, these new technologies still need some ironing out, but we saw an exciting glimpse of things to come….

LIVING WITH MND - A NEW GUIDE

We are delighted to announce the arrival of our new care guide *Living with motor neurone disease*.

The guide has been developed to help people with MND achieve the best possible quality of life from the point of diagnosis, with an overview of how to manage the disease, its impact and top tips. It is presented in a slim-line A4 wallet folder with various inserts, such as our publications list.

The new guide is evidence based, user tested and has been reviewed by multiple experts in accordance with The Information Standard.

It deals with topics ranging from 'What is MND?' to living with the disease and how to access key services and support.

Living with motor neurone disease is clear, compact and well signposted, helping the reader navigate to selected content.

The pack, which is given to people upon diagnosis, includes various write-on pages, which are designed to help the user keep track of important records, such as equipment and medication.

The Association has also created a web version of the new guide, which includes the ability to download internal sections as separate items..

'*Living with motor neurone disease* provides a candid, but sensitive overview of MND and can direct you to where you can access support' said actress and Association ambassador Gina Bellman, whose mother lives with MND.

'MND often feels very isolating, but support from people in similar circumstances can be very encouraging. The guide includes quotes, tips and experiences from others affected by the disease.'

The guide has been produced with the support of the *Tesco Charity Trust* and the *Evan Cornish Foundation*, for which the Association is very grateful.

People living with or affected by MND can order the new guide from MND Connect, or download "Living with MND" from our website.
For more information about MND and a list of our current publications, please see our Publications List.

Use of riluzole to treat MND

You may have seen in the media a report into the use of a range of NHS approved drugs, including riluzole to treat MND.

Riluzole (sometimes known as Rilutek) is currently the only drug licensed for treating MND in the UK.

The National Institute for Health and Care Excellence (NICE) has approved its use for MND, which means it should be available to people who have been diagnosed with the disease.

However, the drug is not suitable for all patients. Treatment with riluzole should only be initiated on the advice of a neurological specialist experienced in the management of MND, and patients should be monitored for side effects. Please discuss this with your consultant.

While it may marginally slow down the progression of the disease, the extent to which riluzole extends life may be limited. In many cases this moderately beneficial effect offers positivity for those with the disease.

Riluzole may also have side effects, which mean some people choose not to continue taking the drug.

There are a number of ways to take positive action to maintain a sense of control and achieve the best possible quality of life with MND.

For more information, see our Information Sheet 9.

If you want to find out more about riluzole, or have difficulties accessing the drug, contact MND Connect, our helpline service, on 08457 626262, or mndconnect@mndassociation.org

The Association funds global research to find effective treatments and campaigns to ensure all those living with MND have timely access to appropriate care and support, to achieve the best possible quality of life.

NICE (National Institute for Health and Care Excellence) appraised medicines in the NHS in England - 2012, Health and Social Care Information Centre. Note: the report is based on the assumption that riluzole is suitable for all those living with MND.

End