optimistic

Summer 2016

Issue 4

In this issue

Welcome to the fourth OPTIMISTIC newsletter. You are receiving this newsletter as we think you might find it interesting. To continue to receive updates about this project then please sign up for the newsletter at www.optimistic-dm.eu or contact Libby Wood elizabeth.wood2@ncl.ac.uk.

There are many different aspects to OPTIMISTIC and and we have covered CBT and genetic testing in previous newsletters all available on the website: http://www.optimistic-dm.eu/optimisticnewsletter/ . This issue we answer some frequently asked questions and provide some more information on fatigue and how it affects people with myotonic dystrophy type 1.

Thank you for your interest in OPTIMISTIC!

THE OPTIMISTIC CONSORTIUM MEETS

All investigators and researchers working on the OPTIMISTIC study from France, Germany, the Netherlands and the United Kingdom met in Budapest in November 2015. The group discussed many different aspects of the study. It was great to see how well the study is going with over 250 participants recruited everyone is excited for the results in 2016.

Baziel Van Engelen is coordinating the project from Nijmegen in the Netherlands.

Local Contacts

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Study coordinator and Physiotherapist.
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FREQUENTLY ASKED QUESTIONS.

When will results be available?

The OPTIMISTIC consortium will start to analyse the data collected as part of optimistic later in 2016. There is a lot on information to look at so this will take a few months. These results will first be published in scientific journals for other doctors and researchers to learn more about myotonic dystrophy. We will produce some summaries that will be sent to patient organisations to include in newsletters and websites.

Will I get access to my individual results?

We won't be able to give you any results while you are taking part in the trial. This is because it is important for the science of the trial that we collect data for the trial before discussing the results with individual participants. You are of course free to discuss anything regarding your clinical care with doctor or other health professional as normal.

At your last OPTIMISTIC appointment, and once we have collected your trial data, the OPTIMISTIC person you normally see (e.g. doctor, physiotherapist or research associate) will be able to discuss your results with you if you would like this. Please remember that it might be necessary to make a further appointment to discuss some of your results because they will not all be available at the last appointment.

What happens to the blood and urine samples I provide?

All blood and urine samples are being stored at the Newcastle Biobank for Research Of Neuromuscular Disorders. This will allow researchers with permission to use these samples to learn more about myotonic dystrophy within the OPTIMISTIC consortium and also other researchers from around the world. Some blood is being analysed in Nijmegen and Glasgow for genetic and molecular biomarkers, there is more information about this in Newsletter 2 available on the OPTIMISTIC website.

Can I access treatment or therapy after the study ends?

OPTIMISTIC is a research study, this means we are testing to see if the combination of cognitive behavioural therapy and increased activity has an effect on levels of fatigue and wellbeing. We will not know if this is the case until all of the data has been analysed. At the moment it will not be possible to offer this treatment however we will contact you if this changes. If you have more questions about this please discuss it at your next appointment.

255 patients
recruited from
Four European Countries,
Germany, France, Netherlands, United Kingdom,
Largest
multi-national
trial in myotonic dystrophy

Are there other research studies I can participate in?

The amount of research into myotonic dystrophy type 1 is growing and there may be something else for you to participate in soon. The best way to be kept up to date about future research is to join the registry in your country. More information about the patient registries and to find the contact details for yours you can check the TREAT-NMD website (http://www.treat-nmd.eu/dm/patient-registries/DM/). In addition to taking part in your local registry information about clinical research is often listed on www.clinicaltrials.gov, if you search for "myotonic dystrophy" you can learn more.

SPOTLIGHT ON FATIGUE AND DAYTIME SLEEPINESS

Sleep related features of myotonic dystrophy type 1 are common, reported in approximately 3 out of 4 people. This feature of myotonic dystrophy often has a big effect on daily life and it is not uncommon for sleepiness or fatigue to have an impact on the ability to work or look after the family.

Some sleep related symptoms can include:

- Having difficulty staying awake after meals
- Falling asleep at work, in the car or while watching TV
- Long and unrefreshing naps without dreams
- Restless leg syndrome
- Morning headaches

Sleep related symptoms of myotonic dystrophy can be caused by a number of different things including a problem with lungs, heart or brain. Because of this variability it can be hard to identify the cause, it is best to discuss any of these types of symptoms with your doctor who might refer you to a sleep or respiratory specialist to discuss in more detail.

Depending on the reason for feeling sleepy or fatigued there may some treatments available to you.

More information:

There are some more resources about myotonic dystrophy and sleep related symptoms available.

Myotonic Dystrophy Foundation toolkit:

http://myotonic.org/resources/publications

Myotonic Dystrophy Foundation Video:

http://myotonic.org/2013-mdf-annual-conference-daytime-sleepiness-and-dm

Myotonic dystrophy Support Group leaflet:

http://www.myotonicdystrophysupportgroup.org/leaflets/

Stimulant medications e.g. Modafinil or Ritalin

The availability of these medicines varies from country to country and the people who are in charge of regulating drugs in Europe have not allowed them for use in myotonic dystrophy so can only be given under special circumstances. However, there are many reports that people with myotonic dystrophy and their families find them very beneficial. They can sometimes affect your heart so this has to be considered carefully by your doctor.

Night time Non-Invasive ventilation devices (CPAP, BiPaP)

Often people with myotonic dystrophy are offered an overnight sleep test. If the result of this test is that the sleepiness is caused by trouble with breathing overnight, people may be offered a non- invasive ventilation device (CPAP, BiPAP) to help breathing. This involves wearing a mask at night which although it can be uncomfortable many people find this very beneficial and have a better night's sleep and therefore are more alert during the day.

OPTIMISTIC and sleepiness

OPTIMISTIC is looking at the impact cognitive behaviour therapy (CBT) and increasing physical activity has on the lives of people with myotonic dystrophy type 1. One of the main outcomes will be to see how this affects sleepiness, fatigue and general levels of activity. The activity monitor you have been wearing is one of the ways the researchers will assess this along with looking at your answers to questionnaires. At the moment we don't know if this is making a difference but the researchers across the different sites will be analysing all the data we have collected to try to answer to this question.

Thank you for reading our Newsletter and for participating in OPTIMISTIC. From all of the people involved we have enjoyed having you involved.