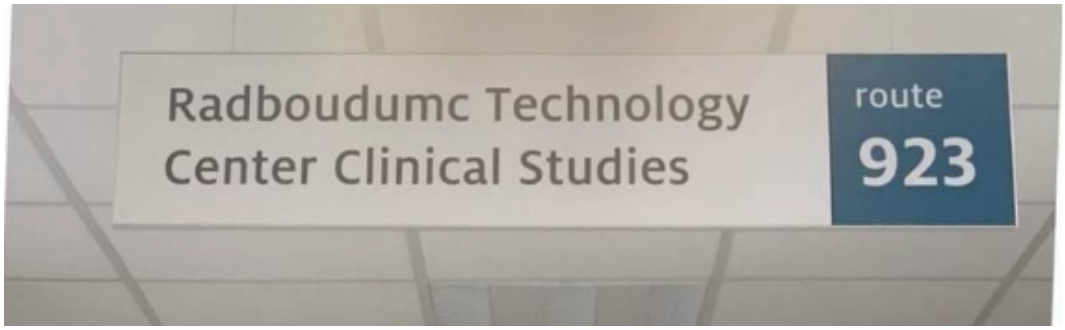


COMING SOON: NEW PATIENT STUDY IN THE NETHERLANDS INTO A PHASE 3 MD DRUG

In November 2024, Radboudumc and Maastricht UMC+ each hope to recruit ten MD patients to try out a potentially effective MD drug. What can we expect, and who can take part? Physician-researcher Joost Kools of Radboudumc explains.

By Rianne Lindhout

MD (myotonic dystrophy) cannot yet be treated effectively with medication. That seems about to change, as several pharmaceutical companies are developing drugs for MD. These are medicines that may reduce the symptoms of myotonic dystrophy if patients take them for life. Developing medicines takes years. Furthest along in the long chain from development to use is the drug Del-desiran from manufacturer Avidity.

Joost Kools is a physician-researcher at Radboudumc. He is involved in various studies in which patients with different conditions test medicines. 'In a few dozen MD patients outside the Netherlands, it has been shown that the drug produces initial improvements in muscle strength, activity level and the typical myotonia (the speed at which you can relax a tightly clenched fist, for example). Various doses have already been tried. The manufacturer now knows which dose works best without causing too many side effects.' But we are not there yet, Kools continues: 'The effects have been established in a relatively small group of patients. To gain more certainty about the effect, a study in a larger number of patients is needed.'

MD PATIENTS IN THE NETHERLANDS WILL RECEIVE AN INVITATION

That is why it is now time for a phase III study: a larger group of patients will try out the optimal dose. The Dutch MD Expertise Centre has been given the opportunity to contribute to the study. Kools: 'Radboudumc and Maastricht UMC+ may each recruit ten patients from around November. We want to give patients from all over the Netherlands the chance to take part.' This means that every MD patient who has registered with Myodraft or the Online MD Registry can expect an invitation to sign up.

REGISTER AS A PATIENT

So if you are an MD patient and have not yet registered, be sure to do so. Myodraft is for patients who are being treated at Radboudumc or Maastricht UMC+. They visit one of these hospitals every year, sometimes slightly more often. The MD Expertise Centre collects clinical data about them. The Online MD Registry is for patients who are being treated at other hospitals. Kools: 'We don't collect all kinds of clinical data there, but we can approach these patients, for example for drug research.'

The phase I/II study currently under way into a drug from Dyne is only taking place among Myodraft patients, Kools says. 'Because Dyne was the first drug study we were going to run and only seven

patients could take part, we were very strict in the pre-selection. We will do things differently with Avidity and all other future trials. Everyone who is registered will receive an invitation. We will then look at the applications to see who is suitable.'

NOT EVERY PATIENT IS ELIGIBLE FOR THE STUDY

It is quite possible that many more people will want to take part than the 20 patients who can. For this reason, Kools and his colleagues are devising a fair lottery system. In addition, participants must meet certain conditions. Kools: 'For this study, these are not yet known exactly.' He can, however, list the most common conditions patients must meet for comparable studies:

- There is often an age limit; usually you must be between 18 and 65 years old.
- You often need to have more than 100 CTG repeats. This concerns the extent to which you have the hereditary abnormality that causes MD.
- You must not have any other diseases that could be a problem in the study, such as Parkinson's disease, dementia or cancer.
- You must have relatively good blood values. These can fluctuate somewhat due to myotonic dystrophy, and we take that into account. The main point is that there is no major kidney or liver damage.
- No pregnancy or wish to have children at this time.
- BMI <35, in other words: you must not be severely overweight. Calculate your BMI here (*body mass index*)

PROS AND CONS OF TAKING PART IN RESEARCH

As a patient, you naturally hope that an experimental drug can help you already, while it will take years before it reaches the market and is available to all patients. But you do not know for certain whether the drug works, and taking part in a drug study involves a lot. Kools: 'You have to go to the hospital regularly, for example every four weeks.' A patient from Groningen, for instance, should ask themselves whether it is feasible to travel to Radboudumc or Maastricht UMC+ every four weeks for perhaps as long as three years. That can be demanding, and not only because of the many hospital visits. 'You will also be extra focused on your disease, because you will be keeping a close eye on yourself in the hope of noticing results.'

PLACEBO OR THE REAL DRUG

There is a chance that at the start of the study you will receive a placebo: a dummy drug. This is done to compare its effect with that of the real drug. On the other hand, in a later phase you often do receive the real drug. And if the drug works, there is a chance you may continue to use it. Kools: 'If a participant benefits from the drug, in general you cannot simply stop it at the end of the study. That is why a study is sometimes extended indefinitely, with participants continuing to use the drug and having occasional check-ups, so that additional research data keeps coming in. Or an agreement is made on *compassionate use*, where participants continue using the drug without further research. Whether and in what form an extension takes place differs per study.'

Rianne Lindhout is a freelance science editor. She recently started writing articles for the MD Foundation about current developments around the disease, after it was also diagnosed in her family.



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