The ALN-APP study - who is eligible? More about the cAPPricorn-1 phase 2 drug study in DCAA.

HCHWA-D
VEREINIGING KATWIJKSE ZIEKTE

Many of you already are aware of Alnylam's plans to study the effect of ALN-APP in Dutchtype CAA. Currently, Alnylam is in the process of selecting Perth as a site for the study. When approved, Australian regulators will have to approve the study as well. Meanwhile, Alnylam has finalized the protocol for the study.

The protocol specifies who can and cannot participate and what the study (cAPPricorn-1) will look like exactly. It is designed with a lot of input from both Dutchtype CAA and sporadic CAA experts. Together they decided on a design for the study that makes the most sense from a scientific and ethical perspective.
The choices they made are NOT final, because regulators can impose on changes in the protocol if they feel that would better the study or participants.

In some countries (the research is being conducted in 60 countries) cAPPricorn-1 is already approved and will start soon. For example, this summer the first people with sporadic CAA in Canada will participate. Because cAPPricorn-1 is taking off, Alnylam is obliged to place part of the protocol online on an American website (www.clinicaltrials.gov). This means that the not yet final design of the protocol for cAPPricorn-1 in people with DCAA is also online.

With part of the protocol going online, we are faced with a dilemma. We expect that the information shared will have an impact on you, even if it is not yet final. We know that many of you are anxiously awaiting whether you can participate and have been involved in the entire process of the research so far. Because we have promised to keep you informed and want to ensure, in consultation with Alnylam and the research teams, that the choices made are understandable to everyone, we are already including you, even though the design is not final.

We will soon plan a meeting in which we will give you the opportunity to ask all your questions. During this meeting we also pay attention to the question: do I want to be tested for the gene in order to participate in drug research?

If you have any burning questions, you can always contact Sanne van Rijn (svanrijn@hchwa-d.nl).

Inclusion- and exclusioncriteria

FROM WHAT AGE CAN YOU PARTICI-PATE?

The minimum age to participate in the study is 30 years. This age is based on what researchers have learned from studies such as DIAN and TRACK DCAA. From the age of 30 onwards, they see more changes in what we call 'biomarkers', ways to measure disease progress.

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DO YOU NEED TO KNOW WHETHER YOU CARRY THE GENE?

Only people who know that they are gene carriers can participate in the drug research, unlike TRACK DCAA. This is partly because it is safer, because it gives better results, because it 100% prevents people from discovering their genetic status because of side effects and because this way the study can be conducted faster. We will discuss this in detail during a meeting in the near future.

IS THERE A MAXIMUM AGE?

There is no maximum age up to which you can participate in the study. There are a number of limits: for example, how far DCAA has progressed. That is why, before you can participate, a screening is done at the research center.

IF YOU ARE IN TRACK DCAA, ARE YOU AUTOMATI-CALLY IN THE ALN-APP STUDY?

The inclusion criteria for TRACK DCAA and the drug study are not exactly the same. This is because Alnylam has learned a lot from research so far, thanks to you, and is applying this to the ALN-APP study. Anyone who participates in TRACK DCAA, knows that he or she is a gene carrier and is 30 years and older is invited for a so-called 'assessment'. Various tests will be used to determine whether you can participate. If not, you are invited to continue participating in TRACK DCAA.

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REPETION OF PAGE 1 INFO DUE TO THE IMPORTANCE OF INFORMATION

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Inclusion- and exclusioncriteria

ARE LUMBAR PUNCTURES PART OF THE ALN-APP STUDY?

ALN-APP is administered through a lumbar puncture, because that is the only way it ends up in the right place: the brain. Therefore you can only participate in the ALN-APP study if you agree to a number of lumbar punctures.

OR THERE OTHER INCLUSION CRITERIA?

In addition to the most important criteria mentioned earlier, there are a number of other things that determine whether you can participate, such as your BMI and pregnancy. These criteria will be shared in more detail later.

DO YOU MISS OUT ON A TREATMENT WHEN YOU ARE NOT ELIGIBLE?

No, we do not yet know whether ALN-APP works, which is why it is being investigated. If you choose to participate in the study, you have a chance that you will receive something that slows down the disease, but we will only know after the study finished whether or not that is true.

IS THERE A PLACEBO GROUP?

Yes, when you participate you may be placed in a so-called placebo group. This means that you do not get ALN-APP, but a liquid without the active drug. This is almost always part of a study, because people sometimes show improvement because they think they are receiving a drug (the placebo effect). By adding a placebo group, a comparision between the effect of ALN-PP and placebo can me be made

WANT TO LEARN MORE ABOUT ALN-APP? IN AUGUST 2023 THE PERTH RESEARCH TEAM AND ALNYLAM JOINED US FOR A WEBINAR ABOUT THE POTENTIAL DRUG. ASK SANNE FOR A LINK TO THE RECORDING AT SVANRIJN@HCHWA-D.NL