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Re: Experimental Choroideremia treatment study

Dear Doctors,

We are very excited to announce that our site at the MUHC Centre for Innovative Medicine (CIM), at McGill, is participating in the NIGHTSTAR NSR-REP-01 'STAR' Study. This is a clinical study trial for male patients genetically diagnosed with **Choroideremia (CHM)**.

As you know, Choroideremia is currently an untreatable retinal degeneration that progresses to legal blindness by middle age. In this study, we will document the natural history of CHM patients, by performing various visual function and retinal structure measures. Some patients, with strict entry criteria, may be able to undergo experimental treatment in one eye, which consists of an experimental surgery introducing a healthy copy of the CHM gene (REP-1) under the retina.

Patients may be eligible for this study if they fulfill the following **Inclusion criteria**:

- Have a genetically-confirmed diagnosis of CHM
- Have active CHM disease clinically visible within the macular region in the study eye
- Have a visual acuity of 34-73 ETDRS letters in the study eye (which is 6/12-6/60 or 20/40-20/200 Snellen acuity). Patients outside these acuities are not eligible right now.

Having the following **Exclusion criteria** may exclude subjects from qualification:

- Have a history of amblyopia (lazy eye) in the study eye.
- Are unwilling to use barrier contraception for 3 months following treatment.
- Have had previous intraocular surgery performed within 3 months of Visit 1.
- Have any other significant ocular or non-ocular disease/disorder, which, in the opinion of the investigator, may either put the subjects at risk or may influence the results of the study, or the subject's ability to participate.
- Also a poor surgical candidate for the study (deemed by the surgical team)

- Contraindication to oral corticosteroid (e.g. prednisolone/prednisone).
- Clinically significant cataract (would need cataract surgery then 3 months before enrolling).
- Have received other gene/cell-based therapy at any time
- Have participated in another research study involving an investigational product in the past 12 weeks

If you have any questions, please do not hesitate to ask. We would greatly appreciate it if you would consider referring your patients with Choroideremia in the next few months who meet the patient profile above, and who would be willing to participate in the clinical study and possibly be surgically treated with the experimental AAV2-REP1.

Many thanks for your help and your time.

Warm regards,

A handwritten signature in blue ink, appearing to read "Robert Koenekoop". The signature is fluid and cursive, with a large initial "R" that loops back.

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