Hôpital de Montréal pour enfants
Centre universitaire de santé McGill

Montreal Children's Hospital
McGill University Health Centre

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NoMuRiC (Nonsense Mutation Readthrough in Cystinosis)

A Phase II Clinical Trial of ELX-02 for Treatment of Cystinosis

(McGill University Health Centre)

The purpose of this message is to invite cystinosis patients to join our upcoming study. We are particularly interested in a specific type of *CTNS* mutation (called a "nonsense mutation") which tricks the cell into stopping production of cystinosin protein before it is finished. Our study will test a new drug candidate, **ELX-02** (a novel eukaryotic ribosomal selective glycoside), to determine whether it causes the cell to disregard nonsense mutations and thereby offers the potential to restore cystinosin protein production in patients with this type of mutation.

Our study will determine whether there is a dose of **ELX-02** that can lower white blood cell cystine, without evidence of toxicity for the kidney or ear (problems that can occur with gentamycin, an aminoglycoside). Medically stable cystinosis patients with confirmed CTNS nonsense mutations who are > 18years (group1) or >12 years (group 2) are eligible. Participants will be housed in Montreal, Canada for 6 weeks for the trial to be conducted at the Centre for Innovative Medicine at the McGill University Health Centre. During the trial, blood and urine samples will be obtained and clinical tests of hearing and balance will be performed to monitor safety. It is expected that **ELX-02** will be administered by daily injections (similar to the administration of insulin or growth hormone).

You will not receive any personal benefits as a result of your participation in this research study. We are hopeful that the results will help us better understand cystinosis for the benefit of patients with CTNS nonsense mutations.

If you would like more information about the trial, please contact us by phone: (514) 412-4400 ext. 22953 or by email: murielle.akpa@muhc.mcgill.ca

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