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Update: Gene Therapies for Wilson Disease

Ultragenyx is adding a fourth treatment group to the first stage of its UX701 Wilson disease gene therapy trial with a tweaked dose of its gene therapy and immunotherapy regimen.

"A higher dose and optimized immunomodulation should enhance the clinical effect of this gene therapy and the ability to remove current standard-of-care in an even broader set of patients," said Eric Crombez, MD, chief medical officer at Ultragenyx.

So far, 15 patients have been enrolled in three dosing groups. After six months of following the study participants, six have come off of their chelator and/or zinc therapy and a seventh patient has begun tapering off their standard-of-care medicine. Improvements in copper metabolism were seen and from a safety perspective, UX701 has been well-tolerated with no unexpected adverse events.

The intent is that a higher gene therapy dose and optimized immunomodulation should enhance the treatment's effectiveness and the ability to remove current standard-of-care in more patients.

The Wilson Disease Association will provide updates on when Ultragenyx will begin enrolling patients in this fourth treatment group.

Vivet Therapeutics has made the difficult decision to terminate its VTX-801 gene therapy trial after infusing four patients at two different dosing levels. Data gathered so far from the two doses that were tested shows that while the gene therapy was safe, it did not show a significant enough effect. Bumping up to the next dosing level would require additional funding.

Vivet continues to believe VTX-801 has potential as a treatment option at a higher level dose and is working on strategic alternatives for further development of VTX-801.

The company realizes that this may come as a disappointment to the patient community, and they want to commend and thank everyone for their dedication, collaboration and support since commencing the study.

The Wilson Disease Association extends its sincere appreciation to the four WD study participants for their courage and altruism in volunteering to participate in this gene therapy trial. We are also grateful to our study investigators for their time and effort is studying this novel therapy.

Prime Medicine, Inc. will present potentially promising data on its preclinical gene editing studies at two upcoming scientific meetings. In the laboratory, or preclinically, scientists were able to correct the faulty *ATP7B* gene that causes WD with its gene editing technology.

For more information visit: https://wilsondisease.org/living-with-wilson-disease/treatment/genetic-therapies/