Gene correction for a rare disease

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Angeles suffers from a severe and rare genetic disease called Acute Intermittent Porphyria (AIP). This means, one of her genes restrains her liver to produce a specific protein needed for the metabolism of the blood. During an AIP attack Angeles usually feels intense abdominal pain, and her mental status changes. Women live at an increased risk to trigger the symptoms, because porphyria is related to the menstrual cycle.

Until now there has been no effective treatment, but a new gene therapy developed within the EU-funded project AIPgene could provide patients an end to their ordeal and reactivate the production of the missing protein. The new gene therapy consists of a single injection of a viral vector containing the healthy gene. Such gene will then replace the mutated one in liver cells. This treatment is expected to stop the symptoms of the disease for five to ten years, depending on the life cycle of the cells in the liver.

During the phase I of a clinical trial Angeles and seven other patients have received this treatment, and so far AIP attacks have been reduced significantly among this group. In addition, there are no side effects visible. These promising results indicate that within a few years, the new therapy could be available to all patients.

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