

Discovery leads to effective treatment of painful skin condition

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Researchers at Huntsman Cancer Institute, in collaboration with a worldwide group of physicians and scientists, have discovered a remarkable treatment for a rare, yet debilitating, skin condition.

The study, published online November 24 in *Molecular Therapy* describes a new treatment for pachyonychia congenita, an ultra-rare genetic skin condition caused by mutations in a gene called keratin. The disorder is characterized by painful, blistering calluses on the feet and limbs that limit a patient's ability to walk. Other skin and nail problems also occur.

The new treatment involves a relatively new class of drug called siRNA, and works by preventing the gene with the mutation from being expressed but permitting the healthy keratin genes to function normally. The study marked the first time that the skin of a human subject was treated with this type of drug. Researchers say that in this single patient trial the drug worked, had no serious side effects, and has vast potential because of its ability to specifically and potently target single molecules, making it an option for many other genetic diseases, including cancer.

"The result is exceptionally promising since it suggests that siRNAs can be used safely on the [skin](#) and also possibly to treat genetic disorders that would otherwise have few therapeutic options," says Sancy Leachman, M.D., Ph.D., the study's lead author. "The patient was treated with siRNA on her right foot and with placebo on the left foot. The callus on the right foot that received the siRNA fell off at the site of injection, but

this did not happen on the left foot."

Since the injections of the drug are particularly painful, the next step in the research will be to develop a more patient friendly, perhaps topical delivery of the medication.

This study marks a major collaborative accomplishment among international physicians, scientists, pharmaceutical companies, and charitable foundation members.

Source: University of Utah Health Sciences ([news](#) : [web](#))

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