Targeted gene therapy enhances treatment for Pompe disease

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Gene therapy to replace the protein missing in Pompe disease can be effective if the patient's immune system does not react against the therapy. Targeted delivery of the gene to the liver, instead of throughout the body,suppresses the immune response, improving the therapeutic effect, according to an article published in Human Gene Therapy, a peer-reviewed journal from Mary Ann Liebert, Inc. The article is available free online at the Human Gene Therapy website.

"The current unmet medical need in Pompe disease is for prevention of immune responses against standard-of-care enzyme replacement therapy ," says coauthor Dwight Koeberl, MD, PhD. "However, we foresee a future application of the dual vector strategy described in this paper, including a liver-expressing vector along with a ubiquitously expressing vector, which might achieve much higher efficacy than either vector alone."

In the article "Immunodominant Liver-Specific Expression Suppresses Transgene-Directed Immune Responses in Murine Pompe Disease," Ping Zhang and coauthors from Duke University Medical Center (Durham, NC), targeted a gene delivery vector carrying the therapeutic gene to the livers of mice with Pompe disease. Not only did the liver-specific expression of the protein induce immune tolerance, but when combined with non-targeted delivery of the therapeutic gene it also boosted the overall effectiveness of the treatment.

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