Gene therapy using HIV helps children with fatal diseases, study says

Italian researchers have used a defanged version of HIV to replace faulty genes — and eliminate devastating symptoms — in children suffering from two rare and fatal genetic diseases.

Improved gene therapy techniques prevented the onset of metachromatic leukodystrophy in three young children and halted the progression of Wiskott-Aldrich syndrome in three others.

The advance represents a major stride for a field that has struggled to translate experimental successes in lab animals into safe and effective treatments for people, experts said. Researchers may be able to use the team's method as a template, modifying it to treat a variety of diseases.

Read the full article here: Gene therapy using HIV helps children with fatal diseases, study says