How gene therapy might treat diseases once thought untreatable

What is gene therapy?

[Gene therapy] is a way to fix a gene defect in a person's cells. One way is with a retrovirus. When these viruses infect cells, they are able to transcribe genetic information into the cell's DNA. So we genetically engineer the virus to incorporate the gene a person needs.
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Two gene therapy treatments have recently been approved by the European Medicines Agency. There are hundreds of clinical trials going on now. For instance, the results of a trial treating an immune deficiency disease called SCID were recently published. Years after the treatment, there has been no indication of any cancer in the patients.

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When you extract cells from the patient, genetic alteration can be done remotely. Will that make gene therapy more accessible?

An initiative has just been launched by David Williams at Dana-Farber/Boston Children's to centralize manufacturing of patient haematopoietic stem cells. It will provide training to enable satellite centers to administer gene therapy, so people can remain in their local hospital.

This initiative could also be applied to clinical trials. There are already some in which this is being tested. You really need standardization and coordination of the work for gene therapy to become more widespread.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Let's take on untreatable diseases, says gene therapy pioneer