Gene therapy evolves to treat blood cancers and numerous other rare disorders

Gene therapy has come a long way since its first human proof-of-concept trials in the 1990s. The approach—which involves fixing or replacing a disease-causing gene or changing its activity—has recorded some remarkable successes and some devastating missteps.

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Over the past few years not only has the discipline changed but the very definition of gene therapy has evolved. Today the field includes not just direct, permanent changes to a cell’s DNA but also transient changes to how genes are translated into proteins. Researchers have now reported a number of success stories: they have alleviated some cases of blindness, cured cancers, addressed the underlying cause of sickle cell disease, and begun to treat congenital disorders, such as spinal muscular atrophy, that might otherwise be lethal.

For both patients and the public, the potential for such treatments evokes not fear so much as an abundance of hope. And with that hope comes other problems: problems of overexpectation, of affordability and of accessibility. Current gene therapy approaches are pricey and not easily available, both issues that limit their possible reach. Solving these problems may be the field’s next big challenge.

This is an excerpt. Read the original post here.