

Gene therapy is designed to introduce genetic material into cells to compensate for abnormal genes or to make a beneficial protein. The vector can be injected or given intravenously (by IV) directly into a specific tissue in the body, where it is taken up by individual cells. Other viruses, such as adenoviruses, introduce their DNA into the nucleus of the cell, but the DNA is not integrated into a chromosome. If a mutated gene causes a necessary protein to be faulty or missing, gene therapy may be able to introduce a normal copy of the gene to restore the function of the protein. Instead, a carrier called a vector is genetically engineered to deliver the gene. Researchers must overcome many technical challenges before gene therapy will be a practical approach to treating disease.