Genetically Engineered Blood-Clotting Factor Encyclopedia Article

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Excessive, uncontrolled bleeding can be fatal. One well-known disease associated with this phenomenon is hemophilia. Most hemophiliacs bleed uncontrollably because a single gene on the X chromosome lacks the instructions that tell the cell how to make a specific protein. This protein, called factor VIII, is required for blood to clot normally. Purified factor VIII extracted from human blood became available around 1960, but it was very expensive. Furthermore, viral impurities in the factor VIII obtained in this manner placed many hemophiliac patients at risk of contracting serious diseases, including hepatitis and, later, AIDS. In the early 1980s, scientists at Genentech, Inc., and Chiron Corporation in California and at the Massachusetts-based Genetics Institute began developing genetic engineering techniques to obtain pure, inexpensive factor VIII artificially. Genetic engineering refers to methods of rearranging genes--removing or adding them or transferring them from one organism to another. At Genentech, Richard Lawn, Gordon Vehar, and their coworkers succeeded in isolating the normal gene for factor VIII in healthy people and inserting it into laboratory-grown hamster cells, where it joined with the DNA (deoxyribonucleic acid) of the hamsters. The hamster cells then used the genetic instructions in the DNA to make pure human factor VIII. In April 1984, after many months of work, tests showed that the genetically engineered factor VIII is able to clot hemophiliac blood. A major problem, however, with this promising method of treating hemophilia inexpensively and safely is that it is difficult to control the amount of Factor VIII that the cells produce, and too much factor VIII causes the blood to stop circulating properly. Although it will take several more years of work before the gene itself can be introduced directly into a patient, tests are currently underway to determine the best dosage of artificial factor VIII for hemophiliac patients.