‘Gigantic leaps forward’ What’s happened in the 21 years since a teenager died undergoing gene therapy, stalling emergence of this biomedical breakthrough?

In 1999, [teenager] Jesse [Gelsinger] received a dose of the ornithine transcarbamylase gene, engineered into a recombinant adenovirus, at the University of Pennsylvania. The idea was for the gene to zero in on liver cells. However, soon after the treatment, he developed jaundice, inflammation, and multiple organ failure. Within four days, Jesse was dead.

Since then, genetic technology has made gigantic leaps forward. The human genome has been sequenced in its entirety, and the cost of sequencing has plummeted 100,000-fold. CRISPR-Cas9 has emerged as a precise and efficient tool to edit genes. Artificial intelligence and deep learning are being put to use in designing molecules. And gene therapy has morphed from a blacklisted experimental concept to a life-altering practical solution for a growing list of previously incurable diseases.

To date, the U.S. Food and Drug Administration (FDA) has approved five gene therapy products for a half-dozen diseases.

Beyond correcting genes, gene therapy is revolutionizing cell-specific delivery of therapeutic proteins and synthetic drugs, as well as inspiring basic research into unanswered questions on immunogenicity. But lest anyone think gene therapy has solved all of its earlier issues, the deaths last summer of two young boys in an experimental trial for a rare muscular disorder remind us that the field still has a lot of work to do to ensure gene therapy can be safely delivered for all patients.

Read the original post