Case Study: In Vivo Gene Therapy and the Story of Jesse Gelsinger

In vivo gene therapy involves the direct introduction of therapeutic genes into a patient's body, often using viral vectors to deliver the desired gene to target cells. While this approach holds promise for treating a variety of genetic disorders, early trials revealed significant challenges and risks.

The Jesse Gelsinger Incident:

Jesse Gelsinger was an 18-year-old with ornithine transcarbamylase (OTC) deficiency, a metabolic disorder that prevents the body from breaking down ammonia. Although he managed his condition with a strict diet and medication, he volunteered for a clinical trial at the University of Pennsylvania in 1999, hoping to help others with more severe forms of OTC.

During the trial, Jesse received an adenoviral vector containing a corrective gene. Shortly after, he experienced a severe immune response which led to multiple organ failure. Jesse Gelsinger passed away four days after receiving the gene therapy.

Aftermath:

Jesse's death halted many gene therapy trials and led to intense scrutiny of the field. Investigations revealed potential conflicts of interest among the researchers and the possibility that some adverse events from earlier trials were not adequately reported. This tragedy highlighted the importance of transparency, informed consent, and careful oversight in clinical research.

Legal and Ethical Implications:

The Gelsinger case underscores the potential risks of emerging biotechnologies and raises questions about the responsibilities of researchers, the rights of participants, and the role of oversight bodies in ensuring safety.

Discussion Questions:

1. What ethical responsibilities do researchers have when conducting clinical trials, especially with experimental treatments?
2. How should potential conflicts of interest be managed in biomedical research to ensure patient safety and scientific integrity?
3. How can informed consent be improved to ensure participants fully understand the risks and benefits of experimental treatments?
4. What role should oversight bodies (like the FDA) play in regulating emerging biotechnologies, and how can they ensure a balance between innovation and patient safety?

Read https://www.bluebirdbio.com/our-science/our-approach-to-gene-therapy

Questions:

1. What are the main steps involved in bluebird bio's ex vivo gene therapy process?

2. How does bluebird bio's gene addition therapy differ from traditional stem cell transplants?

3. What are the main diseases treated with bluebird bio's gene therapy?

4. Have these therapies been approved?