Gene Therapy Development Process

**Basic Research**
Disease identified in patient population.
Researchers identify gene that causes disease and understand the pathobiology of the disease.

**Discovery & Proof of Concept**
Viable gene therapy is developed and tested on disease model.
Proof of Concept – experiments provide evidence that the treatment works to cure/stop the disease.

**Early Preclinical Development**
Strategic and operational plan are established for the program.
Studies completed to validate efficacy and a decision is made on the final gene therapy design.

**Late-Stage Preclinical Development**
Vector manufactured. PharmTox-Dosage and Safety tested.
Human Clinical Trial design begins development with advisors.
IND Enabling datasets are finished and IND submission is prepared.

**Regulatory submission to initiate Clinical Trials**
Clinical Trials Phase 1,2, & 3
Approved for Commercialization