



Expression Therapeutics, LLC



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Company Profile

Industry Sector: Biotherapeutics; Gene Therapy

Company Overview: Expression Therapeutics is a privately owned biotechnology company founded in 2005. The company is focused on developing novel treatments for persons with hemophilia A, a recessive, genetically inherited bleeding disorder caused by the deficiency of the blood clotting protein factor VIII. Hemophilia A occurs in approximately 1 in 7,500 males worldwide. People with hemophilia A can have life threatening bleeding episodes. Existing treatments for hemophilia A consists of intravenous infusion of recombinant factor VIII, leading to a temporary raise factor VIII levels, thereby permitting an effective clotting process. Due to high costs of treatment, approximately 70% of persons with hemophilia worldwide do not receive factor VIII therapy, which leads to a mortality rate approaching 100 percent by young adulthood. Currently, there is no cure for hemophilia A. Our work promises to dramatically change the treatment for hemophilia A through a novel, patented gene therapy technology that has the potential to cure hemophilia A for the lifetime of the patient. Additionally, our high expression factor VIII technology can substantially decrease manufacturing costs, thereby providing affordable treatment to persons with hemophilia A world-wide.

Target Market(s): Persons with hemophilia A



Key Value Drivers

Technology*: High expression fVIII

Competitive Advantage:

Gene therapy provides the potential to cure hemophilia A, instead of lifelong treatment, and is therefore transformative. Development of a lower cost recombinant fVIII biotherapeutic for the treatment of persons with hemophilia A can provide a life-saving treatment for patients where alternative treatments are not available.

Plan & Strategy:

Expression Therapeutics intends to develop both gene therapy and high expression fVIII technologies for the treatment of persons with hemophilia A through Phase I/II clinical stage while securing strategic alliances or partnerships.

*Technology funded in part through NIH-NHLBI Phase II SBIR R44HL114241 "Bioengineered factor VIII gene therapy for hemophilia A".



Management

Founders:

H. Trent Spencer, Ph.D., President

Christopher B. Doering, Ph.D.

John S. 'Pete' Lollar, M.D.

Mohan Rao, Ph.D.



Product Pipeline

- 1. Pipeline One:** Lentiviral and AAV-based gene therapy for the treatment of persons with hemophilia A.
- 2. Pipeline two:** Engineered FVIII therapeutic products for high level expression and longer activated half-lives.
- 3. Pipeline Three:** High expression recombinant fVIII therapeutics for immune tolerance induction (ITI) treatment of persons with hemophilia A with inhibitors or acquired hemophilia A patient.
- 4. Pipeline four:** High expression recombinant fVIII therapeutics for the treatment of persons with hemophilia A in developing countries.