The Next Frontier: Investigational Gene Therapy for Hemophilia A

BioMarin’s scientific and clinical leaders have made significant contributions in the field of hemophilia A.

BioMarin’s program in hemophilia A was featured in 2017 at the American Society of Hematology meeting and in The New England Journal of Medicine. Two Phase 3 trials of BioMarin’s investigational gene therapy, valoctocogene roxaparvovec, are underway in men with severe hemophilia A: GENER8-1 and GENER8-2.

In 1984, Dr. Barrie Carter, VP of Vector Biology at BioMarin, first described using an adeno-associated virus as a gene therapy delivery vehicle (Mol Cell Biol 1984).²

In that same year, Dr. Gordon Vehar, VP of Research at BioMarin, led the scientific team that first cloned factor VIII, the blood-clotting protein that is defective or missing in persons with hemophilia A (Nature 1984).³

Dr. Wing Yen Wong, VP of Clinical Science at BioMarin, brings decades of experience translating the science to the clinic for patients with hemophilia A, both as a treating physician and in developing novel therapies.

Track Record of Manufacturing Complex Medicinal Products
BioMarin has built one of the largest gene therapy manufacturing facilities in the world. We remain steadfast in our goal of meeting potential demands for our products through state-of-the-art production facilities.

References: