BIOMARIN®

The Next Frontier:
Gene Therapy for Hemophilia A Undergoing Clinical Trials

Drs. Gordon Vehar, Wing Yen Wong and Barrie Carter

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]

Within a month of 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.

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

BioMarin's investigational program in hemophilia A was featured in 2017 at the American Society of Hematology meeting and in *The New England Journal of Medicine*.[1] Multiple clinical studies are underway, including two Phase 3 trials of BioMarin's investigational gene therapy, valoctocogene roxaparvovec, in men with severe hemophilia A: GENEr8-1 and GENEr8-2.

BioMarin's Gene Therapy Facility in Novato, California USA

Track Record of Manufacturing Complex Medicinal Products

BioMarin has constucted the first gene therapy manufacturing facility of its kind and was the 2018 winner for the project execution by the International Society for Pharmaceutical Engineering. We remain steadfast in our goal of meeting potential patient demands at commercial scale for our products through state-of-the-art production facilities.

References:
1. Rangarajan S, et al. *N Engl J Med*. 2017;337 2519-2530.
2. Tratschin JD, et al. *Mol Cell Biol*. 1984;4(10) 2072-81.
3. Vehar GA, et al. *Nature*. 1984; 312(5992):337-42

To learn more about BioMarin's hemophilia program, please visit www.clinicaltrials.gov or contact BioMarin Medical Information at medinfo@bmrn.com