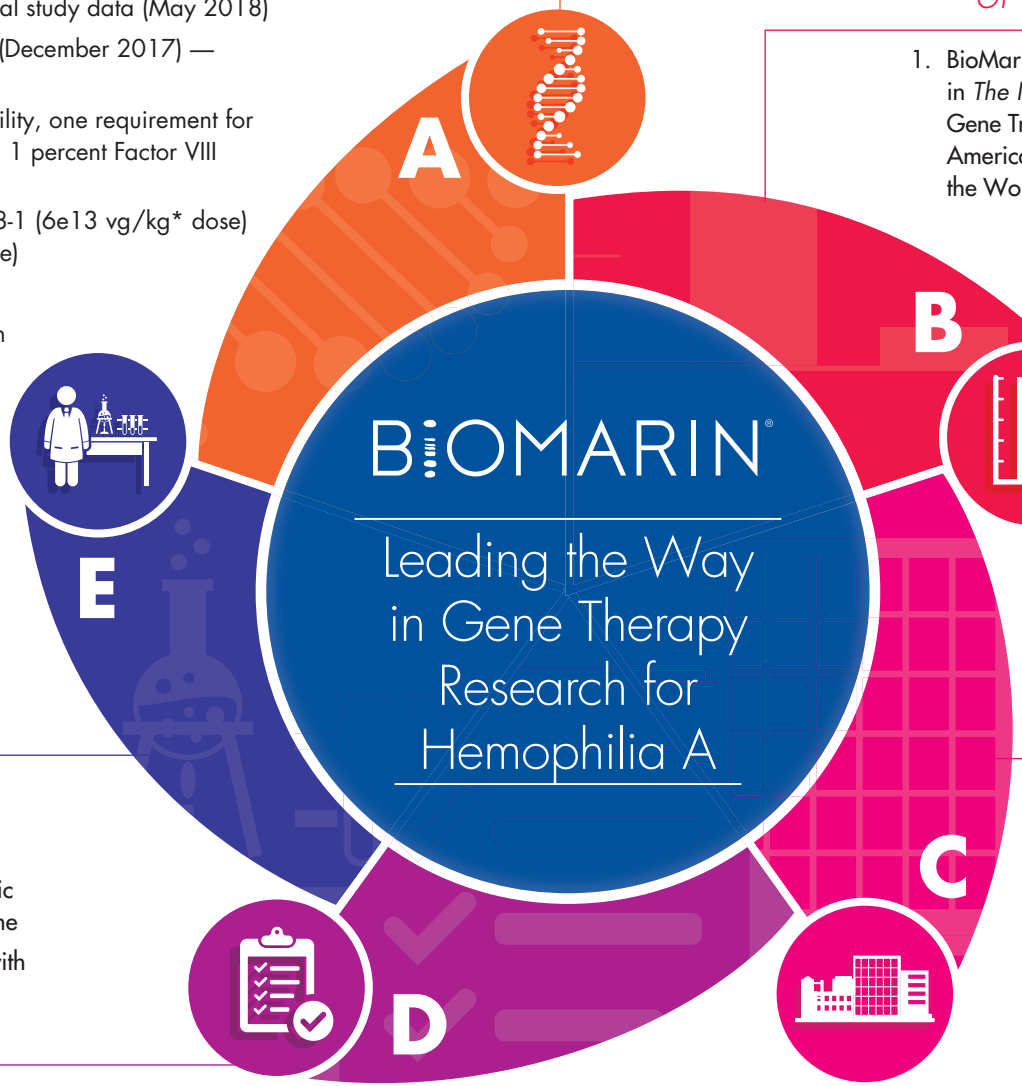
A Most Advanced Hemophilia A Gene Therapy Clinical Trial Research Program in the Industry

1. First and only to report two-year clinical study data (May 2018)
2. First to initiate a Phase 3 clinical trial (December 2017) — the study will enroll 130 participants
3. Six clinical trials underway. For eligibility, one requirement for participants is that they have less than 1 percent Factor VIII activity level:
   a. Two global Phase 3 trials: GENEr8-1 (6e13 vg/kg* dose) and GENEr8-2 (4e13 vg/kg* dose)
   b. Phase 1/2 dose-escalation study
   c. Phase 1/2 trial in participants with pre-existing adeno-associated vector (AAV5) antibodies
   d. Two non-interventional studies: AAV5 seroprevalence and baseline characteristics in participants with hemophilia A

B Research Results Published In *New England Journal of Medicine*

1. BioMarin's program in hemophilia A was featured in 2017 in *The New England Journal of Medicine*, AAV5–Factor VIII Gene Transfer in Severe Hemophilia A, as well as at the American Society of Hematology meeting in 2017 and at the World Federation of Hemophilia 2018 World Congress



C One of the First Gene Therapy Manufacturing Facilities of its Kind

1. BioMarin ownership allows complete control over scheduling, quality and commercial scale production (not possible for many other gene therapy companies that "contract out" manufacture of gene therapy)
2. Facility in Novarto, CA, spans 18,000 square feet
3. Ability to produce 4,000 doses/year of valoctocogene roxaparvovec

E Program Led by Premier Scientific & Clinical Experts

1. Dr. Barrie Carter, the scientist who first introduced using the AAV as a gene therapy delivery vehicle
2. Dr. Gordon Vehar, who led the scientific team that first cloned the Factor VIII gene
3. Dr. Wing Yen Wong, a hematologist with decades of experience in the clinical arena and in developing therapies for hemophilia patients

D Program Aligned with FDA's Draft Guidance (July 2018)

Program follows recommendations for hemophilia gene therapy clinical trials regarding:

1. Efficacy endpoints
2. Study design
3. Study population
4. Statistical considerations
5. Study monitoring
6. Patient experience

*4e13 vg/kg and 6e13 vg/kg refer to doses of 4 and 6 x10^{13} vector genomes per kilogram body weight, respectively

Valoctocogene Roxaparvovec Two-Year Safety

Overall, valoctogogene roxaparvovec has been well-tolerated by participants across all doses, including the two participants who received the lowest doses of 6e12 and 2e13 vg/kg, respectively. No participants developed inhibitors to Factor VIII, and no participants withdrew from the study. The most common adverse events (AEs) across all dose cohorts were alanine aminotransferase (ALT) elevation (11 participants, 73%); arthralgia (9 participants, 60%); aspartate aminotransferase elevation (8 participants, 53%); headache (7 patients, 47%); back pain and upper respiratory tract infection (6 participants, 40%), and fatigue, insomnia and pain in extremity (5 subjects, 33%). Two participants reported serious adverse events (SAEs) during the study. One participant was hospitalized for observation after developing Grade 2 pyrexia with myalgia and headache within 24 hours of receiving valoctocogene roxaparvovec. The event resolved within 48 hours following treatment with paracetamol, an over-the-counter treatment for pain and fever. The event was assessed as related to valoctocogene roxaparvovec. The other SAE was assessed as not related to valoctocogene roxaparvovec, attributed to a planned knee surgery to treat hemophilic arthropathy, and Grade 1 in severity. No complications were reported.