

Hereditary Angioedema Clinical Development Program

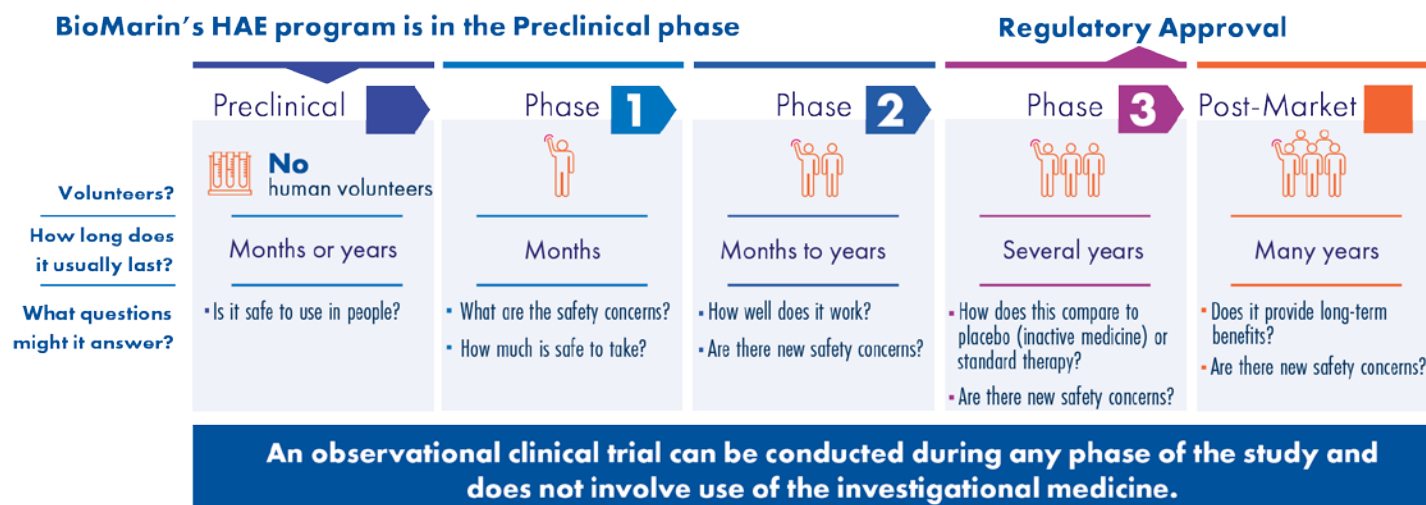
An Update for the Community

BioMarin is a global pharmaceutical company with 6 approved therapies and more than 20 years of experience in developing innovative medicines for rare genetic conditions. BioMarin is invested in following ground-breaking science to bring a big impact to small populations. **Patients are at the heart of what we do.**

In November 2019, BioMarin announced expanding research to include an investigational gene therapy for Hereditary Angioedema (HAE), known as BMN 331.

Before a new treatment can be made available, it must go through careful testing in clinical studies to see if it is safe and effective. BMN 331 has not yet begun clinical studies in humans and has not been proven safe or effective. BioMarin is actively working with regulatory agencies on an investigational new drug (IND) application in order to start clinical studies in 2021.

The bigger picture



About Gene Therapy



Adeno-associated virus (AAV) based gene transfer therapy is one method of gene therapy being investigated. It aims to introduce a functional (or working) gene into the body which is intended to allow the body to produce needed proteins that it lacks. In AAV gene transfer research, a functional gene is inserted into a neutralized viral shell (or therapeutic vector) which delivers the new gene into the target tissue via a single IV infusion. After administration, clinical trial participants are closely monitored to collect data regarding the impact of the infusion on safety and efficacy. There is no replacement or editing done at a genetic level—gene therapy research seeks to introduce a new gene into the body which is not intended to be passed down to future generations.

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About BMN 331

The main types of HAE are caused by a mutation (or change) in the SERPING1 gene. This gene is responsible for making a protein called C1-INH (C1-inhibitor) which plays an important role in controlling certain types of swelling in the body. When C1-INH is missing or not working correctly, sudden, unexpected swelling events can occur. BMN 331 is being researched to see if it can introduce a SERPING1 gene into the liver, to evaluate the safety risks as well as whether it can produce the desired protein. BMN331 has not been approved for use and has not been determined to be safe or effective.



Gene Therapy Manufacturing

BioMarin has built one of the first gene therapy manufacturing facilities of its kind in the world. This facility can support a number of disease areas. The BMN 331 research program will leverage development and manufacturing experience from BioMarin's ongoing gene therapy research in hemophilia A and phenylketonuria.



For additional information on the BioMarin HAE program:

- Contact BioMarin Medical Information at 1-800-983-4587 or medinfo@bmrn.com
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com