



## **BioMarin Announces New Study Drug in Friedreich's Ataxia Entered Into Pipeline: A Communication for Advocacy Groups**

**October, 2017**

BioMarin is pleased to announce that it plans to begin a clinical drug development program for BMN 290, a potential treatment for Friedreich's Ataxia, which has no approved disease-modifying therapies.

BMN 290 is a selective chromatin modulation therapy that works by inhibiting the silencing of the frataxin gene. In cellular models, BMN 290 increases frataxin expression more than two-fold and the compound has been shown to penetrate the central nervous system and cardiac tissues. BioMarin expects to file an Investigational New Drug (IND) application for BMN 290 to the U.S. Food and Drug Administration in the second half of 2018, and we are in the early stages of designing our clinical program.

### **Who is BioMarin?**

BioMarin Pharmaceutical Inc. (BioMarin) is a global biotechnology company that develops and commercializes therapies for people with serious and life-threatening rare disorders. BioMarin is a U.S. company with global headquarters in San Rafael, California. It has regional offices in London, Dublin, Sao Paulo, and Hong Kong, with national offices in additional countries.

Founded more than 20 years ago, BioMarin's portfolio consists of six approved products and multiple clinical and pre-clinical products in research and development. These include treatments for both life-threatening conditions and those that are chronic in nature, with an ongoing and often severe long-term impact on affected individuals and caretakers.

Please know that BioMarin values your experience and knowledge of Friedreich's Ataxia, and we look forward to working with and learning from you as we move forward with this clinical program. For inquiries from advocacy organizations, please contact [patientadvocacy@bmrn.com](mailto:patientadvocacy@bmrn.com). For further information on future BioMarin clinical studies, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) or contact BioMarin Medical Information at [medinfo@bmrn.com](mailto:medinfo@bmrn.com) or by phone: Toll Free 1-800-983-4587; Local (651) 523-0310. Your medical team remains the best source of information for you about any health effects of Friedreich's Ataxia.