



Communication to FARA/Patient Community on IND Approval of ASP2016

Astellas is pleased to share with the Friedreich Ataxia (FA) community that the US FDA has cleared our IND with a fast track designation for a Phase 1 trial of our investigational gene therapy for the treatment of cardiomyopathy in patients diagnosed with FA. ASP2016 is an investigational AAV gene therapy designed to be administered via intravenous infusion to deliver a full-length functional copy of the frataxin gene to the heart of FA patients to treat cardiomyopathy associated with FA. Cardiomyopathy is the leading cause of death in people living with FA.

Richard Wilson, SVP and Primary Focus Lead for Genetic Regulation said *“We are thrilled to see A2016 enter clinical development. Friedreich Ataxia can be a truly devastating disease with few treatments available, and no approved treatments addressing the cardiac complications that can so often prove fatal in patients living with FA. At Astellas, we are committed to working closely with FARA and the FA community to help progress A2016 through clinical development.”*

Astellas is dedicated to the development of genetic medicines with the potential to transform patients' lives. We will share more information about our investigational program at the FARA Research Reception this summer and at the FARA Research Symposium in October. For more information about us and our commitment to patients and families, visit [Astellas Gene Therapies Homepage - Astellas Gene Therapies](#).