

Today, Saturday, May 18, 2013 is Friedreich's Ataxia Awareness Day, which is recognized each year on the third Saturday in May. Beginning a dozen years ago, various members of the U.S. Congress and various state legislatures have proclaimed and marked the third Saturday in May as the day on which we annually recognize the increasing importance of building awareness and understanding of Friedreich's ataxia and committing ourselves to treating and curing this disease.

Friedreich's ataxia is a rare, life-shortening neuromuscular disorder that is usually diagnosed in childhood. It is one of the 7,000 rare diseases that afflict a total of about 30 million Americans. Friedreich's ataxia causes muscle weakness and loss of coordination in the arms and legs; impairment of vision, hearing and speech; scoliosis, diabetes; and a life-threatening heart condition. Most patients need a wheelchair full-time by their twenties. There is currently no treatment for this devastating disorder.

In those early years when Friedreich's Ataxia Awareness Day was first proclaimed, far fewer people were aware of the disorder, much less was known about it and hope was hard to come by. Now, although there is still no treatment available at this time, Friedreich's ataxia patients and families have more and more reason for real hope and even confidence. An extraordinary explosion of research findings followed the identification of the Friedreich's ataxia gene in 1996 and, since that discovery, research scientists have learned a great deal about the disorder. We now know what defects in the gene cause the disease, what protein the gene produces, what that protein is supposed to accomplish, and why a shortage of the protein results in the cell death that leads to the disease symptoms. Scientific investigators are increasingly optimistic that they are drawing very close to developing effective treatments.

In the United States and around the world, clinical trials in Friedreich's ataxia are being conducted on drugs that hold real promise. Intensifying collaboration among scientists and medical care professionals, patient-advocacy organizations, government agencies and the pharmaceutical industry provides powerful evidence of the tremendous progress being made and the growing hope, confidence and determination to conquer Friedreich's ataxia. There is a growing conviction that treatments can and will be developed for this disease and that the resulting insights will be broadly applicable across a wide range of rare and common neurological disorders.

As in each of the last dozen years, multiple events will be held in communities across our country this month to increase public awareness of Friedreich's ataxia and to raise the funds needed to support the research that is so promising of effective treatments for this disorder. We ask you to join the Friedreich's Ataxia Research Alliance (FARA), Friedreich's ataxia patients and families across the country and around the world in recognizing May 18, 2013, as Friedreich's Ataxia Awareness Day and to show our concern for all those families affected by this disorder and to express our support and encouragement for their efforts to achieve treatments and a cure.

Thank you very much.