National Ataxia Foundation
Connecting Ataxia families, researchers, clinicians and the community

January 15, 2021

Dear Food and Drug Administration and Reata Pharmaceuticals leadership,

Since our founding in 1957, the National Ataxia Foundation (NAF) has represented people affected by all forms of Ataxia. Our membership of almost 6,000 includes more than 700 people who either have Friedreich's Ataxia (FA) or are those who support them.

It is important for drug and biologic sponsors and the FDA to hear the voices of rare disease communities and to know that these patients and their family members do engage in understanding the data from clinical trials in making decisions about safety and efficacy. Patients and caregivers provide elucidative insight on the level of uncertainty and risk they are willing to bear, as they live with their disease every day. NAF was a partner of the Friedreich's Ataxia Research Alliance (FARA) in 2017 for the Externally-Led Patient Focused Drug Development Meeting (EL-PFDD) for FA. The Voice of the Patient Report that came from that meeting is a powerful summary of the devastating impact of FA. NAF held its own EL-PFDD meeting in 2020 for Polyglutamine Ataxias.

NAF has had the opportunity to review the results of the MOXIe studies, and as many of our stakeholders are individuals living with FA, we, like FARA, believe it is important to facilitate opportunities for direct patient engagement and incorporate the patient experience in the interpretation of results and decision making. It is based on these understandings and beliefs that NAF strongly supports the efforts of the Friedreich's Ataxia Research Alliance and FA Community Call to Action requesting Reata to submit a New Drug Application (NDA) on an urgent basis and FDA to exercise the flexibility granted by law and contained in FDA guidance in considering approval of an NDA for Omaveloxolone in FA based on the existing evidence from clinical trials.

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While we always respect the FDA's processes in approving drugs and biologics, it is important for the FDA to appreciate that time is imperative in their evaluations of data for rare diseases. Each day that passes for these patients is a day for additional functional losses to occur. For all diseases, and for rare diseases in particular, law and regulation allow for a collaborative process for review where patients and caregivers have an equal seat at the table to describe their lived experiences with their disease and with taking the drug or biologic under review.

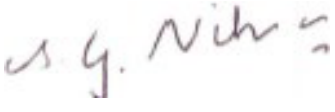
We believe that we are at an important inflection point in FA drug development with positive clinical results and believe that we need to achieve clarity on guidance regarding what level of evidence is necessary for approving new drugs for FA. This guidance could help inform similar decisions for related rare diseases, especially other inherited Ataxias, and/or could influence similar decisions by other regulatory bodies, ex-US, where individuals with Ataxia are living.

We thank Reata and the FDA for your urgent review of the attached letter, especially the patient testimonies, and request that you work together to provide access to Omaveloxolone for people with FA as soon as possible.

Sincerely,

Andrew Rosen
Executive Director

Samuel Kirton
President, Board of Directors



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