FARA Friedreich's Ataxia Research Alliance

NEWS

June 16, 2011

Dear Friends,

The past quarter has been a busy and exciting time for the Friedreich's Ataxia Research Alliance (FARA) with some significant developments in our research and awareness programs. I'd like to take a moment, here, and share with you some of those developments.

World Orphan Drug Congress & Intellect Neuroscience
At the end of April, I participated in the second World Orphan Drug Congress. The Congress was held in downtown D.C. and assembled key representatives of many pharmaceutical companies from all over the world who are interested in advancing therapies for rare diseases like FA. Key rare-disease representatives of the NIH and FDA as well as a handful of rare-disease advocates also participated. I had the opportunity to meet with several representatives from different drug companies- some larger companies just starting to expand their rare disease program, some companies with specific interests in seeking treatments that would increase frataxin levels and others interested in improving mitochondrial function and oxidative metabolism.

It became abundantly clear to me that FA is of interest to these companies because of the maturity of FA research (the level of our understanding of the disease as well as the breadth and depth of treatment approaches being investigated), because of the collaborative nature of the FA research and patient community and because FARA has established key tools for drug screening and development as well as a strong infrastructure for conducting clinical trials. In sum, drug companies are coming to agree with our scientists who explain their commitment to FA research by saying, "We think we can get this one." These companies are beginning to see the mature FA science and clinical infrastructure FARA is supporting as the "low–hanging fruit" they are seeking among rare diseases. They see that FARA and the FA community will save them time and money in developing treatments and that those treatments are likely to provide insights and benefits in many other disorders.

A good illustration is the news from a company, Intellect Neuroscience, which recently filed an Orphan Drug Application for their compound in the treatment of FA. The compound, OX1, (indole-3-propionic acid) is a naturally occurring small molecule that appears to prevent oxidative stress by a combination of hydroxyl radical scavenging activity and metal chelation. The company has been developing the compound for 10 years and has completed Phase 1 safety studies. When they became interested in pursuing this compound in FA, FARA met with them to discuss how to plan and implement trials in FA. The company focused the discussions on the infrastructure FARA has built through the Collaborative Clinical Research Network (CCRN) in FA as well as the validated clinical outcome measures and natural history that are needed to design trials. These discussions made clear once again that the work we are doing together at the CCRN sites and with each of our clinical trials is making FA more and more appealing to drug companies. You can view the full press release from Intellect Neurosciences at: http://ir.stockpr.com/intellectns/press-releases/detail/503/intellect-neurosciences-files-orphan-drug-

application-in-the-united-states-for-its-clinical-candidate-ox1-for-the-treatment-of-friedreichs-ataxia .

International Friedreich's Ataxia Scientific Conference
In early May at the Institute of Genetics and Molecular and Cellular Biology in Strasbourg, France, FARA convened the world's largest assembly to date of Friedreich's ataxia scientists. The 200 conference participants included scientists from the world's leading research labs, government agencies, pharmaceutical industries, and representatives from multiple advocacy groups. Over the three-day meeting, they shared the latest insights and advances in basic, translational and clinical research in FA. FARA and the international FA scientific community are convinced that at conferences like this one, FA scientists share insights and nurture collaborative research essential to the next big breakthrough in FA. While in Strasbourg, we had the opportunity to interview some of our funded scientists about their work. You can view some of those interviews by clicking on the following links:

- Dr. Marcia Haigis from Harvard Medical School discusses her research in sirtuins: http://www.youtube.com/watch?v=oGBPiM70wa8.
- Dr. Michele Lufino from the University of Oxford discusses his gene therapy research: http://www.youtube.com/watch?v=dUrs1ZHLOxw

A summary of the conference will be in our next newsletter - The Advocate to be released this summer.

Edison Pharmaceuticals Announced the Study Results for A0001
Last week, Edison Pharmaceuticals announced the results of the Phase 2a Double Blind Placebo Controlled 28-day Clinical Trial of A0001 in FA. While the study failed to achieve the primary endpoint selected for the study -- glucose disposition index (a measure of the body's glucose handling), it did achieve its secondary endpoint by showing in both the high-dose and low-dose groups as compared to the placebo group that EPI-A0001 significantly improved neurological function as assessed by the Friedreich's Ataxia Rating Scale (FARS). FARA will continue working hand-in hand with Edison, the investigator team, and the patient and physician community to begin implementation of further EPI-A0001 prospective trials. We would like to thank all who participated in this study -- patients, families and investigators.

- To read FARA's press release regarding A0001, visit: http://www.curefa.org/_pdf/A0001DrugTrial.pdf
- To view Edison's press release, visit: http://www.edisonpharma.com/PressReleases/110610_Edison%20Pharmaceuticals%20Announces%20Results%20of%20EPI-A0001%20Phase%202A%20Double%20Blind%20Placebo%20Controlled%2028-day%20Clinical%20Trial%20in%20the%20Mitochondrial%20Disease%20Friedreichs%20Ataxia.pdf

EPI-743 Achieves Orphan Drug Status
Edison Pharmaceuticals, Inc. also announced that the United States Food and Drug Administration (FDA) has granted orphan drug designation to EPI-743 for treatment of inherited mitochondrial respiratory chain diseases, including FA. The company also announced that the FDA has approved EPI-743 for Expanded Use in FA and other mitochondrial disorders.

- To view FARA's press release, visit: http://www.curefa.org/_pdf/FDAOrphanDrugDesignationEPI-743.pdf

Army Wives Team Up with FARA to Raise Awareness for FA
The cast and crew of Lifetime's Army Wives recently lent their support to help raise awareness and promote giving to FA research. They filmed a campaign video on set and promoted the video to their fans through social media leading up to their season finale episode. The video can be viewed at: http://www.youtube.com/watch?v=wX-c4lWsP0I

As you can see from these updates, powerful momentum continues to mount in both FA research and awareness as we continue to advance all of the projects in our treatment pipeline. As a full time staff of five dedicated people, we are able to accomplish this only because of your support - - whether that be your contribution of time or financial resources. Thank you for your continued support of our collective goal to treat and cure FA.

Warm regards,

Ron

Ron Bartek, FARA President

About FARA
The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. Plese visit our website at www.curefa.org.