FARA Friedreich's Ataxia Research Alliance

NEWS

FARA is Key Participant in Government Report on Accelerating Rare Disease Research
Institute of Medicine Report Calls for Integrated National Strategy

On Monday, October 04, 2010, the Institute of Medicine (IOM) released its report titled "Rare Diseases and Orphan Products: Accelerating Research and Development." To help in accelerating rare diseases research and product development, the National Institutes of Health (NIH), with support from the Food and Drug Administration (FDA), asked the IOM to examine the opportunities for and obstacles in developing drugs and medical devices for treating rare diseases. The IOM appointed a committee to prepare a report on these issues and called on a few rare disease advocacy organizations, including FARA, to provide information and perspective from their own experiences in grappling with these opportunities and obstacles.

The IOM Report is rich in recommendations for action FARA has long advocated. This is especially true in regards to the recommendations to increase FDA and NIH resources and capabilities for supporting and accelerating clinical and preclinical development of rare disease therapies and for using FDA and NIH grants to foster public-private partnerships bringing together in common cause government agencies, pharmaceutical companies, academic scientists and rare disease advocacy organizations such as FARA.

To guide policymakers in the Administration and Congress, the IOM Report sets forth the elements of an integrated national strategy to promote rare diseases research and product development. Among other recommendations, the IOM calls for NIH to work with industry, academia, and advocacy organizations to develop a comprehensive system of shared resources for discovery research on rare diseases and to facilitate communication and cooperation for such research. The IOM Report also calls for the Secretary of the Department of Health and Human Services to establish a national task force on accelerating rare diseases research and product development. We all know that the effort and investment needed to move further toward a collaborative, coordinated, open, and sustained approach to rare diseases will be substantial, but not making the investment will also be costly. FARA fully agrees with the IOM in saying that the potential benefits justify a renewed commitment to accelerating rare diseases research and product development.

FARA's Executive Director, Jen Farmer, made the FARA presentation to the IOM committee charged with preparing the Report – the Committee on Accelerating Rare Diseases Research and Orphan Product Development. FARA President Ron Bartek provided brief additional comments and served as a consultant and reviewer of the Report. The IOM Report refers to Friedreich's ataxia a number of times, cites Jen's

presentation to the Report Committee throughout the document and discusses FARA several times in terms of a model advocacy organization. For example, FARA is one of only six organizations used in Appendix F to illustrate successful approaches to rare disease research and drug development. FARA Spokesperson & Founder or Ride Ataxia, Kyle Bryant, is featured in a photo on the cover of the report. In other portions of the Report, FARA is credited with exemplary activities such as our successful participation in the NIH program to accelerate advancement of new drug discoveries into clinical trials (the NIH Rapid Access to Intervention Development – RAID-- that helped move A0001from Edison Pharmaceutical's lab into the current clinical trial), and our mouse model program with The Jackson Laboratory.

You can go to www.iom.edu/rarediseaseresearch and read a summary of the IOM report by clicking on "Report Brief" or see the entire report by clicking on "Read Report Online for Free." Jen's presentation to the IOM Committee, cited several times in the report, is at http://iom.edu/~/media/Files/Activity%20Files/Research/OrphanProductResearch/23-NOV-09/Farmer2.pdf.

About FARA
The Friedreich's Ataxia Research Alliance's (FARA) mission is to marshal and focus the resources and relationships needed to cure FA by raising funds for research, promoting public awareness, and aligning scientists, patients, clinicians, government agencies, pharmaceutical companies and other organizations dedicated to curing FA and related diseases. www.CureFA.org

Contact
Jennifer Farmer
Executive Director, Friedreich's Ataxia Research Alliance
(484) 875 3015
info@curefa.org