

Conference on Clinical Research for Rare Diseases

September 21, 2010

Bethesda North Marriott, Bethesda, MD

Summary

The Rare Diseases Clinical Research Network (RDCRN) and the Clinical and Translational Science Awards (CTSA) programs co-sponsored the 2nd Conference on Clinical Research for Rare Diseases (CCRRD) which was held on September 21, 2010 in Bethesda, Maryland. The meeting was highly successful with approximately 270 meeting attendees, including trainees, junior and senior faculty, NIH program officials, and leaders of patient advocacy groups.

Among the conference speakers were investigators from the RDCRN and CTSA program, and representatives of the US FDA, the biomedical industry, and patient advocacy groups. The full-day program included a series of short lectures or panel discussion with ample time for questions. Topics covered included:

- Clinical trial design issues and options for study of rare diseases
- Creating, maintaining, and growing research networks in rare diseases
- The CTSA program and rare disease research
- Industry perspective of orphan disease drug development
- Study designs for rare diseases
- Conflict of interest and rare diseases
- Parent, advocate, researcher (the view from my parachute)
- Working with industry, FDA, and handling conflicts of interest
- Working with patient advocacy groups for rare diseases
- Career advice, working with patient advocacy groups, and working with the NIH

The conference ended with a dinner keynote address by Mr. John Crowley, former CEO of Novazyme Pharmaceuticals, Inc., and president and CEO of Amicus Therapeutics, Inc, whose account of his successful professional efforts to promote the development of a therapy to treat Pompe disease, which affects two of his children, was inspirational for all attendees.

The Conference poster session was held during the meeting, and 91 scientific posters were presented during the session. The posters were a mix of work submitted by trainees, fellows, junior faculty, and established investigators, and included RDCRN Consortium summaries.