Outreach and Research

The Center for Rare and Neglected Diseases at the University of Notre Dame works with patient services organizations, clinicians, and other stakeholders to raise awareness about rare diseases. Ty Quandt who has Niemann Pick Type C disease (pictured above) enjoyed visiting Notre Dame and our laboratory mouse models to develop new methods to diagnose and treat neurological, lysosomal diseases.

Prof. Shaun Lee
Chaperones

Dr. Lee who received the prestigious NIH Innovator Award in 2011 is a biologist. He collaborates with Dr. Tony Serianni, a medicinal chemist and together they work to find drugs that improve function of poorly active proteins caused by genetic mutations that result in rare diseases. This is why these drugs are called ‘chaperones’. The approach is important in treating many rare diseases.

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Mrs. Eva Luise Koehler, the former First Lady of the Federal Republic of Germany, is Patroness of the German Alliance for Rare Diseases (ACHSE). She and her husband Dr. Horst Koehler, (former President of Germany) also a leading advocate for rare diseases, visited the campus in late September. During her visit with CRND, Mrs. Koehler eloquently spoke about the challenges facing the European rare disease coalition ACHSE and suggested forming a closer tie with CRND. She was interested to learn about CRND’s project to make rare diseases “non-rare” through a medical record repository of rare diseases (see “Developing Health Networks in Rare and Neglected Diseases”). CRND will engage ACHSE to strengthen our ties.