

FOR IMMEDIATE
RELEASE
May 20, 2009

Contact: Tiphané Turpin
202-966-5557 x212
tturpin@geneticalliance.org

Genetic Alliance Issues Statement Applauding New NIH Therapeutics for Rare and Neglected Diseases Program

WASHINGTON – May 20, 2009 – Earlier today, the National Institutes of Health (NIH) announced the establishment of a new initiative called the Therapeutics for Rare and Neglected Diseases (TRND) Program, a federally mandated effort to encourage and speed the development of new drugs for rare and neglected diseases. Sharon Terry, president and chief executive officer of [Genetic Alliance](#), a nonprofit health advocacy organization, issued the following comments in response to the establishment of TRND:

“Today marks a leap forward in transforming the rare and neglected disease drug development pipeline. NIH is a crucial entity working in this space. In the United States, a rare disease is defined as a condition affecting fewer than 200,000 people, and diseases lacking substantial therapeutic development activity are considered neglected diseases. Many genetic diseases are both rare and neglected, and collectively there are more than 6,000 rare and neglected diseases affecting over 25 million Americans. Fewer than 200 of these diseases have any therapy available, and progress in this area is limited within the current system.

Clinical trial design is a unique challenge for rare and neglected diseases for reasons such as small patient populations and the need for global recruitment. Because of these challenges, it is critical to conduct effective trials from the onset, as subsequent opportunities may not be possible. Flexible, adaptive trial design and close interaction with the U.S. Food and Drug Administration will be especially important to consider in this context. In addition, engaging disease-specific groups in preparation for clinical trials to characterize the natural history of diseases and determine clinical trial endpoints is also essential and can begin right away through the application of existing tools, technologies and networks.

Genetic Alliance applauds the federal government for development of the TRND Program and views meaningful collaborations with external partners as a key element in the program. We have been collaborating with stakeholders in the health community including, disease-specific groups, umbrella organizations, and industry corporations while engaging in dialogue around regulatory and policy issues to bring about systems change in this area. This experience has shown us that collaborations through open and transparent processes are essential for change that will benefit health outcomes for all stakeholders.

We are excited for the potential the TRND Program holds and hope to collaborate with the program and its partners in system transformation.”

###

About Genetic Alliance

Genetic Alliance transforms health through genetics, promoting an environment of openness centered on the health of individuals, families, and communities. Genetic Alliance brings together diverse stakeholders that create novel partnerships in advocacy; integrates individual, family, and community perspectives to improve health systems; and revolutionizes access to information to enable translation of research into services and individualized decision making. For more information about Genetic Alliance, visit <http://www.geneticalliance.org>.