



The National Organization for Rare Disorders (NORD) is pleased to present its recommendations to the NIH transition team.

NORD is a unique federation of voluntary health organizations dedicated to: helping people with rare or “orphan” diseases; and assisting the organizations that serve them. A rare or “orphan” disease affects fewer than 200,000 people in the United States. There are more than 6,000 rare disorders that, taken together, affect approximately 30 million Americans. For 25 years, NORD has served as the primary non-governmental clearinghouse for information on rare disorders. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service. (www.rarediseases.org)

NORD strongly supports increased funding for biomedical research. We urge the incoming Administration to state publicly its commitment to advancing medical research. Further, NORD urges the appointment of NIH leadership that supports increased research into rare diseases and understands that such research often advances the scientific understanding of diseases affecting larger patient populations.

Specific recommendations with regard to NIH are as follows:

- 1. Increase funding to the NIH Office of Rare Diseases (ORD) to allow the expansion of the Rare Diseases Clinical Research Network.** This is a consortium of NIH Institutes that helps provide internal coordination in the pursuit of better understanding of rare diseases. Increased funding for this important initiative will enhance the ability of NIH institutes to conduct rare disease research more efficiently
- 2. Increase funding for the Undiagnosed Diseases Program.** Some patients with rare diseases can wait years for a definitive diagnosis. In a study conducted by NORD a few years ago, one in six rare-disease patients remained undiagnosed for six years or longer. The Undiagnosed Diseases Program is a relatively new NIH



initiative that helps patients with mysterious conditions that have long eluded diagnosis. While seemingly focused on rare diseases, it has already advanced medical knowledge about rare and common diseases. The program is trans-NIH in scope. It is organized by the National Human Genome Research Institute (NHGRI), the NIH Office of Rare Diseases (ORD) and the NIH Clinical Center. Increased funding will enable more patients to be diagnosed earlier and gain the benefit of treatment and be identified for enrollment in clinical trials, when appropriate..

3. Increase emphasis on international cooperation in research on rare diseases. For rare diseases research, cooperation is increasingly important because our patient populations are small and geographically dispersed, and funding for such research is best coordinated for maximum impact.

NORD is committed to working closely with the new Administration to advance innovation in research and access to new treatments for the 30 million rare disease patients that we represent.