



August 14, 2015

National Institutes of Health (NIH)
9000 Rockville Pike
Bethesda, MD 20892

Re: Request for Information (RFI): Inviting Comments and Suggestions on a Framework for the NIH-wide Strategic Plan

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, NORD would like to thank the National Institutes of Health for the invitation to comment on the Framework for the NIH-wide Strategic Plan.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

We would first like to congratulate and thank the NIH for the inclusion of "advancing research opportunities presented by rare diseases" as one of the NIH's priorities within its strategic plan. The rare disease patient community relies on the NIH to conduct or fund much of the essential basic and translational research on rare diseases. This research lays the groundwork for therapeutic development for neglected diseases with inadequate or no treatment. The following comments outline several ways to accelerate rare disease research.

Advancing rare disease research starts with adequately funding the NIH, a goal that has been sorely missed over the past ten years. NORD has long advocated that Congress allow NIH funding to keep pace with inflation, and recognize the critical importance of the NIH to the nation's health and economy by prioritizing NIH funding in each budget cycle.

Second, the Office of Rare Diseases Research (ORDR) plays an integral role in championing and coordinating rare disease research within the NIH. It also stimulates rare disease research outside of the NIH by providing logistical expertise. The ORDR coordinates the Rare Diseases Clinical Research Network (RDCRN) and serves as the central locus of rare disease data and research expertise for rare disease researchers around the world. The ORDR also houses the Genetic and Rare Diseases Information Center (GARD), the central hub for rare disease information. We request that any further development of the NIH strategic plan recognizes the importance of the ORDR to the rare disease patient community.

We also request that the National Center for Advancing Translational Sciences (NCATS) continues to play a key role throughout the NIH. NCATS works to translate the findings of basic research into treatments or cures for diseases. This is important for the rare disease community because developing novel therapies can cost upwards of \$1 billion, and 95 percent of therapies fail in the lab or in clinical trials without ever reaching the patient. NCATS works to improve and accelerate this process by filling the gaps that exist in the current framework, and by researching innovative research and drug development techniques. This includes clinical trial design as well as efficacious biomarker and endpoint identification.

Through its Therapeutics for Rare and Neglected Diseases (TRND) program, NCATS encourages collaborations aimed at stimulating drug discovery and development research among NIH and academic scientists, nonprofit organizations, and pharmaceutical and biotechnology companies working on rare and neglected diseases. NCATS is already achieving many of the goals set forth in the strategic plan framework, such as enhancing impact through partnerships, ensuring rigor and reproducibility, and more. For this reason, NCATS should be prominently featured throughout the NIH's Strategic Plan

Finally, the Undiagnosed Diseases Program (UDP) also plays a critical role in advancing rare diseases research as it is solving the medical mysteries that plague millions of undiagnosed patients. We request the strategic plan recognize the importance of the UDP in finding treatments and cures for the rare disease community.

We thank NIH for the opportunity to comment, and we look forward to working with the NIH as the strategic planning process moves forward. For questions regarding NORD or the above comments, please contact Paul Melmeyer, Associate Director of Public Policy, at pmelmeyer@rarediseases.org or (202) 588-5700, ext. 104.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in cursive script, appearing to read "Peter L. Saltonstall".

Peter L. Saltonstall
President and CEO