



May 9, 2017

The Honorable Lamar Alexander, Chairman
U.S. Senate Committee on Health, Education,
Labor, and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray, Ranking Member
U.S. Senate Committee on Health, Education,
Labor, and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the 30 million men, women, and children affected by one of the 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) fully supports the Enhanced Clinical Trial Design Act (S.1048), and supports its inclusion within the Food and Drug Administration Reauthorization Act (FDARA).

The Enhanced Clinical Trial Design Act would require FDA and the stakeholder community to examine clinical trial design and the methods used to determine inclusion/exclusion criteria. These criteria all too often exclude patient populations that could benefit from the treatment if approved, as well as individuals who could benefit from participation in the trial itself. We firmly believe clinical trial inclusion criteria should accurately reflect the population most likely to benefit from the therapy upon approval. Therefore, we support the public meeting, report, and guidance required within the bill in hopes they will improve current practices.

We also support the provision that allows FDA to streamline Institutional Review Board (IRB) evaluation of single-patient expanded access requests. IRB review is required for both emergency and non-emergency single patient expanded access requests, and IRB approval is required before non-emergency access can move forward. Currently, fully-convened IRBs are required to review these requests, often leading to unnecessary delays and barriers to access. Allowing a trained representative of an IRB to review these requests would expedite and streamline review, and facilitate accelerated access to investigational therapies.

Finally, greater sponsor clarity on expanded access policies for therapies using certain expedited review pathways will further improve transparency within the expanded access process.

We appreciate your time and attention. For questions regarding NORD or the above comments, please contact Paul Melmeyer, Director of Federal Policy, at pmelmeyer@rarediseases.org, or 202-545-3828.

Sincerely,

Peter L. Saltonstall
President and CEO