



April 13, 2015

Division of Dockets Management (HFA-305)
U.S. Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Docket No. FDA-2015-D-0268: Individual Patient Expanded Access Applications: Form FDA 3926; Draft Guidance for Industry

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the nearly 7,000 known rare diseases, NORD would like to thank the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's Draft Guidance titled, "Individual Patient Expanded Access Applications: Form FDA 3926: Draft Guidance for Industry".

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 enthusiastically support the creation of a unique form tailored specifically for individual patient expanded access requests. Currently physicians must use Form FDA 1571 to request access to investigational therapies. On line 102 of this draft guidance, the FDA recognizes Form FDA 1571 requires information from physicians that is not applicable to individual patient expanded access requests. The arduous process of filling out Form FDA 1571 has fueled calls for removing the FDA entirely from the expanded access process, something NORD does not support and believes could be very damaging.

Form FDA 3926 allows physicians to request access to investigational therapies using a simple two page application that requests information that is not overly burdensome. The Office of Management and Budget (OMB) estimates completing this form will take 45 minutes, a drastic improvement over the estimated 8 hours needed to complete Form FDA 1571 for individual patient expanded access requests.

We support the FDA's flexibility regarding emergency individual patient expanded access requests. As described in the Draft Guidance, physicians may proceed with treatment on an emergency basis by providing to the FDA a simple explanation of the necessity of the treatment, including by telephone. We agree that the FDA should permit physicians to forgo IRB approval in an emergency case as long as they receive IRB approval in the five days following the start of treatment. We thank the FDA for allowing physicians this necessary flexibility when treating patients with particularly time-sensitive cases.

While this Draft Guidance provides physicians with an easy to use, step-by-step process for completing Form FDA 3926, we believe the FDA could provide greater clarity on several steps within the completion process.

First, this Draft Guidance defines an “emergency situation” as a situation “that requires the patient to be treated before a written submission can be made”. This requires the physician to make a rather subjective assessment of whether or not a situation constitutes an “emergency” or not. We support the FDA allowing physicians the flexibility to determine if the situation is an emergency, but we believe the FDA could provide greater clarity to assist physicians in making this decision.

Second, greater clarity on when to use Form FDA 1571 instead of Form FDA 3926, particularly after the initial use of Form FDA 3926, would be beneficial. On page 4, starting on line 124, the FDA states, “Although FDA intends to accept draft Form FDA 3926, when finalized, for submitting a new expanded access IND for a single patient, the IND holder (physician) should use Form FDA 1571 for subsequent submissions to his/her IND”. We ask that the FDA clarify the requirement for when a physician should be using Form FDA 1571 instead of Form FDA 3926.

Finally, while the replacement of Form FDA 1571 with Form FDA 3926 will greatly improve the individual patient expanded access request process, there are still several potential roadblocks for patients to access investigational therapies within the current system. For example, approval of a single-patient IND requires the physician to be knowledgeable of which review division to contact and how to contact them.

It is our belief that a stronger, more visible central office that assists in expanded access requests would be beneficial for the rare disease community. A centralized Office of Patient Affairs would allow physicians to easily consult with the FDA on which review division to approach, and any other assistance needed with completing Form FDA 3926.

Overall, we believe the availability of Form FDA 3926 will greatly benefit the rare disease patient community seeking access to investigational therapies, and we support the FDA’s attempt to streamline and simplify the process.

We thank FDA for the opportunity to comment, and we look forward to working with FDA on improving the individual patient expanded access process. For questions regarding NORD or the above comments, please contact Paul Melmeyer, Assistant 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