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Value of IRB in Expanded Access

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In a recent review of publicly available institutional review board (IRB) policies pertaining to expanded access, Ms Folkers and Dr Bateman-House found that 92.5% contained procedures for nonemergency or emergency uses under FDA's expanded access regulations.[1] The authors conclude that IRB policies varied widely and were difficult to understand. Further, and more important, they questioned the value of an IRB review in these cases.

We disagree that an IRB review, an FDA requirement, is not warranted in expanded access situations. The IRB is the sole entity that has *only* the patient's interests as its focus. Its value in expanded access lies in reviewing and approving the consent process and document. The IRB determines that the consent document is written for treatment, not research; for a patient, not a subject; clearly states the risks and that there might be unknown risks; and clearly states that the investigational product might not be efficacious. In these dire situations, some argue that desperate patients will accept any treatment for hope; however, patients can, and do make, informed decisions about their future. Many weigh the potential benefits and risks carefully, based on the informed consent process.

Under the FDA expanded access pathway, manufacturers providing access to investigational products for treatment use carefully review single patient requests. FDA further determines that the patient has a serious or life-threatening disease or condition, and that the patient cannot access an already approved therapy or gain access through a clinical trial. The current approval process involving the manufacturer, FDA, and the IRB provides the best protection of the patient's interests and welfare.

A federal right-to-try law went into effect in late May, which is troubling because it significantly reduces patient protection by eliminating the IRB review requirement and specifies no criteria for appropriate informed consent when single patients obtain expanded access through the new right-to-try pathway.[2]

We also point out that IRBs normally do not charge for review of expanded access requests for single patients. We are aware of at least one independent IRB, WIRB, that provides reviews at no cost to the patient or treating physician.[3]

The authors view the variability in IRB procedures as negative. We offer a different view. The federal regulations give IRBs flexibility to establish procedures that are potentially more appropriate for them as long as the IRB members understand them. Many IRBs review hundreds and even thousands of new research protocols, continuing reviews, modifications to existing research annually—whereas they might review fewer than 10 expanded access requests in a year. Some IRBs use the flexibility in the federal regulations to establish rapid review boards, convene meetings by conference call or assign the request to the next IRB meeting when there are weekly meetings to handle these requests quickly. Expanded access requests should be handled outside the normal IRB process and begin with the treating physician alerting the IRB that the request is coming for review.

Finally, FDA announced in October 2017 that the treating physician may request a waiver for the convened IRB to review a single-patient expanded-access request. Instead, the IRB chair or a designee may conduct the review.[4] This change addresses the concern that IRB review is not timely. While it would help IRBs for FDA to provide more guidance on IRB review responsibilities for expanded access, our challenge today is to ensure that IRBs are aware of the most recent FDA guidance to improve IRB efficiency, and provide added protections for patients considering using investigational products.

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