

Docket No. 2006N-AF14
RIN 0910-AF14

Huntington's Disease Drug Works
www.hddrugworks.org

Huntington's Disease Advocacy Center
www.hdac.org

Huntington's Disease Lighthouse
www.hdlighthouse.org

The listed advocacy organizations for Huntington's patients have been disappointed by expanded access amendments that cover so little new ground that it is literally hard to find what was amended. Even more frustrating is that while the FDA describes several shortcomings of present policies, it fails to address any of the problems identified.

Present policies discriminate against patients outside of academic centers. While in theory an individual physician can obtain an investigational drug for a desiring patient; in practice, the process remains so labor intensive that very few individual physicians can afford the time and expense. *FDA can address this problem by providing incentives to sponsors willing to submit an umbrella treatment IND protocol.*

Present policies primarily benefit patients with cancer or AIDS more than those belonging to other disease groups. *The FDA can address this problem by working on early access programs with other institutes as closely as it does with National Cancer Institute and Office for AIDS Research.*

The amendments do nothing to promote *meaningful change* for "token" expanded access programs because meaningful change can occur only with industry support. While we realize that the FDA has no authority to compel support from the pharmaceutical industry, there are ways to provide encouragement. *The FDA can address this by offering financial incentives to industry, such as extending periods of exclusivity, as has been successful in clinical trials for children, or expediting drug review.*

The FDA is proposing a change in policy for large-group expanded access programs that will require more intensive reporting of adverse and therapeutic results. This model, that has been successful for NCI "Group C" programs supplies greater safety and efficacy data *and* creates wider access. While a good idea in theory, this change in policy that entails greater sponsor expense will *discourage* industry support of large group access unless incentives are offered.

Unfortunately these amendments do little more than provide lip service for improved expanded access to patients who lack other alternatives. Until there is willingness to take action by providing adequate incentives to industry, the words add up to “nothing new”. These FDA amendments do nothing to restore the public’s trust in improved expanded access.

C. Baseline for the Analysis

During the period 1997 through 2005, FDA received an average of 2,046.6 INDs per year. Of this number, on average, approximately 659, or 32.2 percent ($0.322 = 659 / 2,046.6$) were individual patient or emergency INDs. In addition, FDA received approximately 4.6 treatment IND or treatment protocol submissions per year during this time period. Thus, treatment IND or treatment protocol submissions represent about 0.2 percent ($0.022 = 4.6 / 2,046.6$) of all

FDA invites comments on these topics: (1) Whether the proposed collection of information is necessary for proper performance of FDA’s functions, including whether the information will have practical utility; (2) the accuracy of FDA’s estimate of the burden of the proposed collection of information, including the validity of the methodology and assumptions used; (3) ways to enhance the quality, utility, and clarity of the information to be collected; and (4) ways to minimize the burden of the collection of information on respondents, including through the use of automated collection techniques and other forms of information technology, when appropriate.

Proposed § 312.310(b)(1) states that if the drug is the subject of an existing IND, the expanded access submission may be made by a commercial sponsor or by a licensed physician. Proposed § 312.310(b)(2) states that a sponsor may satisfy the submission requirements by amending its existing IND to include an individual patient expanded access protocol. Proposed § 312.310(b)(3) states that a licensed physician may satisfy the submission requirements by obtaining a right of reference to pertinent information in the IND and providing any other required information not contained in the IND (usually only the information specific to the individual patient).

This proposal to amend FDA regulations for early access to investigational drugs is very little more than written acknowledgement of agency shortcomings. What is more striking is that this document contains nothing new to address the problems identified. They've again failed to comply with the congressional order they quoted: to ensure that "opportunities to participate in expanded access programs are available to every individual with life threatening or serious debilitating illness for which there is not effective therapy".

The FDA acknowledges that expanded access to investigational drugs occurs almost exclusively for patients who are cared for in academic centers. This document offers no plan to correct this inequity. In practice, expanded access is not available to individual patients and physicians who desire access, because the process is too labor intensive and expensive for those who lack the administrative support of academic centers.

The FDA acknowledges that expanded access to investigational drugs has mostly been limited to cancer and AIDS patients. This document does not further discuss this problem; nor does it make recommendations that might improve this situation.

By far, the most significant problem: the lack of industry support. The FDA cannot "compel" industrial companies to sponsor early access proposals as part of drug approval processes. The strongest language used in this document is that they "may request" that companies consider expanded access programs if public demand is high enough. While the FDA acknowledges the absolute importance of industry as step one of the process, it gives the industry no support. If the FDA is really serious about expanded access programs in more than a token way, they must step up to the bar, and offer real incentives to companies who run early access programs.

Broader ACCESS

We believe the larger issue is not FDA regulation, or even lack of effective FDA support for existing programs. The paramount issue for early access programs is industry support. Sponsoring companies will best respond to incentives at FDA level.

