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# Guidance for Industry Information Program on Clinical Trials for Serious or Life-Threatening Diseases and Conditions

## *DRAFT GUIDANCE*

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For questions regarding this draft document, contact Terry Toigo, 301-827-4460.

**U.S. Department of Health and Human Services  
Food and Drug Administration  
Center for Drug Evaluation and Research (CDER)  
Center for Biologics Evaluation and Research (CBER)**

**January 2004**

**Procedural**

**Revision 1**

# Guidance for Industry Information Program on Clinical Trials for Serious or Life-Threatening Diseases and Conditions

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**U.S. Department of Health and Human Services  
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Procedural  
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*Contains Nonbinding Recommendations*

*Draft — Not for Implementation*

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1 **Guidance for Industry<sup>1</sup>**  
2 **Information Program on Clinical Trials for Serious or**  
3 **Life-Threatening Diseases and Conditions**  
4

5  
6 This draft guidance, when finalized, will represent the Food and Drug Administration's (FDA's) current  
7 thinking on this topic. It does not create or confer any rights for or on any person and does not operate to  
8 bind FDA or the public. You can use an alternative approach if the approach satisfies the requirements of  
9 the applicable statutes and regulations. If you want to discuss an alternative approach, contact the FDA  
10 staff responsible for implementing this guidance. If you cannot identify the appropriate FDA staff, call  
11 the appropriate number listed on the title page of this guidance.  
12

13  
14  
15 **I. INTRODUCTION**  
16

17 This guidance is intended to assist sponsors who will be submitting information to the Clinical  
18 Trials Data Bank. The data bank was established as required under section 113 of the Food and  
19 Drug Administration Modernization Act of 1997 (Modernization Act). This guidance updates  
20 and replaces the March 2002 guidance for industry of the same title to include assistance for  
21 sponsors who will be submitting information required by the Best Pharmaceuticals for Children  
22 Act (Public Law 107-109) (BCPA). Additional updates on procedural issues not related to the  
23 BCPA will be discussed in future revisions to this guidance.  
24

25 FDA's guidance documents, including this guidance, do not establish legally enforceable  
26 responsibilities. Instead, guidances describe the Agency's current thinking on a topic and should  
27 be viewed only as recommendations, unless specific regulatory or statutory requirements are  
28 cited. The use of the word *should* in Agency guidances means that something is suggested or  
29 recommended, but not required.  
30

31  
32 **II. BACKGROUND**  
33

34 Section 113 of the Modernization Act creates a public resource for information on studies of  
35 drugs, including biological drug products, to treat serious or life-threatening diseases and

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<sup>1</sup> This guidance has been prepared by the Implementation Team for section 113 of the Food and Drug Administration Modernization Act of 1997, including individuals from the Office of the Commissioner, the Center for Drug Evaluation and Research (CDER), the Center for Biologics Evaluation and Research (CBER), and the Center for Devices and Radiological Health (CDRH), at the Food and Drug Administration.

**Paperwork Reduction Act Public Burden Statement:** According to the Paperwork Reduction Act of 1995, a collection of information should display a valid OMB control number. The valid OMB control number for this information collection is 0910-0459 (expires 03/31/2004). The time to complete this information collection is estimated to average 284 hours per response, including the time to review instructions, search existing data resources, gather the data needed, and complete and review the information collection.

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36 conditions conducted under FDA's investigational new drug (IND) regulations (21 CFR part  
37 312). Section 113 of the Modernization Act, enacted November 21, 1997, amends section 402 of  
38 the Public Health Service Act (42 U.S.C. 282). It directs the Secretary of Health and Human  
39 Services, acting through the Director of the National Institutes of Health (NIH), to establish,  
40 maintain, and operate a data bank of information on clinical trials for drugs to treat serious or  
41 life-threatening diseases and conditions. The Clinical Trials Data Bank is intended to be a central  
42 resource, providing current information on clinical trials to individuals with serious or life-  
43 threatening diseases or conditions, to other members of the public, and to health care providers  
44 and researchers. Specifically, section 113 of the Modernization Act requires that the Clinical  
45 Trials Data Bank contain (1) information about Federally and privately funded clinical trials for  
46 experimental treatments (drug and biological products) for patients with serious or life-  
47 threatening diseases or conditions, (2) a description of the purpose of each experimental drug, (3)  
48 patient eligibility criteria, (4) a description of the location of clinical trial sites, and (5) a point of  
49 contact for patients wanting to enroll in the trial. Section 113 of the Modernization Act requires  
50 that information provided through the Clinical Trials Data Bank be in a form that can be readily  
51 understood by the public. 42 U.S.C. 282(j)(3)(A).

52  
53 The BPCA, signed by the President on January 4, 2002, requires a description of whether, and  
54 through what procedure, the manufacturer or sponsor of an IND will respond to requests for  
55 protocol exception, with appropriate safeguards, for single-patient and expanded access use of  
56 the investigational drug, particularly in children.

57  
58 The NIH, through its National Library of Medicine (NLM) and with input from the FDA and  
59 others, developed the Clinical Trials Data Bank. The first version of the Clinical Trials Data  
60 Bank was made available to the public on February 29, 2000, on the Internet.<sup>2</sup> At that time, the  
61 data bank included primarily NIH-sponsored trials.

62  
63 In response to the Modernization Act's requirements for a data bank, FDA made available two  
64 draft guidances and a final guidance. The first draft guidance provided recommendations for  
65 industry on the submission of protocol information to the Clinical Trials Data Bank.<sup>3</sup> It included  
66 information about the types of clinical trials for which submissions are required under section  
67 113 of the Modernization Act, as well as the content of those submissions.

68  
69 The second draft guidance addressed procedural issues, including how to submit required and  
70 voluntary protocol information to the Clinical Trials Data Bank, as well as issues related to  
71 submitting certification to the Secretary that disclosure of information for a particular protocol  
72 would substantially interfere with the timely enrollment of subjects in the clinical investigation<sup>4</sup>  
73 The second draft guidance also proposed a time frame for submitting the information. A final

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<sup>2</sup> See <http://clinicaltrials.gov>

<sup>3</sup> See <http://www.fda.gov/OHRMS/DOCKETS/98fr/001033gl.pdf>

<sup>4</sup> See 66 FR 35798 and <http://www.fda.gov/OHRMS/DOCKETS/98fr/001033gd.pdf>

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74 guidance, made available on March 18, 2002,<sup>5</sup> combined the two draft guidances into a single  
75 guidance.

76

77 This updated guidance includes new recommended procedures for submitting details, as required  
78 by the BPCA, about single-patient use and expanded access use.

79

80

### **81 III. REQUIREMENTS UNDER SECTION 113 OF THE MODERNIZATION ACT 82 FOR IND SPONSORS**

83

#### **84 A. What information must I submit to the Clinical Trials Data Bank?**

85

86 Section 113 of the Modernization Act requires you to submit information to the data bank about  
87 a clinical trial conducted under an investigational new drug (IND) application if it is for a drug to  
88 treat a serious or life-threatening disease or condition and it is a trial to test effectiveness (42  
89 U.S.C. 282(j)(3)(A)). If you wish, you can also provide information on trials not designed to  
90 assess effectiveness or for drugs to treat conditions not considered serious or life-threatening.

91

92 Section 113 of the Modernization Act requires that you submit a description of the purpose of  
93 each experimental drug, patient eligibility criteria for participation in the trial, a description of  
94 the location of clinical trial sites, and a point of contact for those wanting to enroll in the trial.  
95 Section 113 requires that the data bank provide this information in a form that can be readily  
96 understood by members of the public (42 U.S.C. 282(j)(3)(A)).

97

98 The BPCA amended 42 U.S.C. 282 (j)(3)(A) to require that you submit a description of whether,  
99 and through what procedure, you (the manufacturer or sponsor of a clinical investigation of a  
100 new drug) will respond to requests for protocol exception, with appropriate safeguards, for  
101 single-patient and expanded access use of the investigational drug, particularly in children.<sup>6</sup>

102

103 To ensure that information available through the Clinical Trial Data Bank is in a form that is  
104 readily understood, we have established four data elements, which are listed below. The data  
105 elements are made up of the following data fields: (1) descriptive information, (2) recruitment  
106 information, (3) location and contact information, and (4) administrative data. We have  
107 established the Protocol Registration System (PRS), a Web-based data processing program, to  
108 facilitate collection of this information for the data bank. The four data elements, which are listed  
109 below, as well as definitions applicable to the PRS, can be viewed at  
110 <http://prsinfo.clinicaltrials.gov/>.

111

#### **112 1. Descriptive Information**

113

114 Brief Title (in lay language)

115 Brief Summary (in lay language)

116 Study Design/Study Phase/Study Type

117 Condition or Disease

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<sup>5</sup> See 67 FR 12022 and [http://www.fda.gov/OHRMS/DOCKETS/98fr/00d-1033\\_gdl0003.pdf](http://www.fda.gov/OHRMS/DOCKETS/98fr/00d-1033_gdl0003.pdf)

<sup>6</sup> See 42 U.S.C. 282(j)(3)(A) at <http://www.fda.gov/opacom/laws/pharmkids/contents.html>.

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118 Intervention  
119 Single-patient/expanded access use  
120

### **2. Recruitment Information**

122 Study Status Information including  
123

- 124 • Overall Study Status (e.g., recruiting, no longer recruiting)
- 125 • Individual Site Status

126 Eligibility Criteria/Gender/Age  
127

### **3. Location and Contact Information**

129 Location of Trial  
130 Contact information (includes an option to list a central contact person for all trial sites)  
131  
132

### **4. Administrative Data**

134 Unique Protocol ID Number  
135 Study Sponsor  
136 Verification date  
137  
138

139 To verify the existence of an IND and to assist in administrative tracking, we ask that you also  
140 include in your submission the IND number and serial number and designate whether the IND is  
141 located in the Center for Drug Evaluation and Research (CDER) or the Center for Biologics  
142 Evaluation and Research (CBER). This administrative information is in a separate data field and  
143 will not be made public.  
144

#### **B. When should I begin submitting clinical trial information?**

146  
147 Section 113 of the Modernization Act requires that sponsors submit information no later than 21  
148 days after the trial is opened for enrollment<sup>7</sup> (42 U.S.C. 282(j)(3)). Section 113 does not specify  
149 when sponsors must submit information about clinical trials that are existing and ongoing. To  
150 provide a transitional period for sponsors of clinical trials that are currently ongoing and  
151 expected to continue enrolling patients for more than 45 days, we ask that you submit  
152 information within 45 days after this guidance is made available through the *Federal Register*.  
153 We encourage you to submit information through the PRS for inclusion in the data bank as soon  
154 as possible.<sup>8</sup>  
155  
156  
157  
158

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<sup>7</sup> Section 113 says "not later than 21 days after the approval of the protocol." Because the Agency does not approve protocols, we have interpreted this to mean within 21 days after the trial is open for enrollment.

<sup>8</sup> See <http://prsinfo.clinicaltrials.gov>.

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159           **C.     Can I submit my information at specified intervals rather than on a rolling**  
160                           **basis?**  
161

162       As discussed above, you must submit information about new protocols open for enrollment  
163       within 21 days after the trial is open for enrollment (42 U.S.C. 282(j)(3)), and we request that  
164       you submit information about existing ongoing trials within 45 days after this guidance is  
165       published. Supplemental information can be submitted at 30-day intervals. Such information  
166       includes amendments to the protocol with respect to one of the data elements, or interruptions,  
167       continuations, or completion of enrollment for a study. Protocol changes related to eligibility or  
168       status information, such as routine opening and closing of trial sites, can be made at 30-day  
169       intervals. FDA strongly encourages you to update information about trials that are unexpectedly  
170       closed (e.g., clinical hold) within 10 days after the closing or sooner if possible.  
171       To ensure that the information available through the data bank is timely and accurate, FDA also  
172       encourages you to review, verify, and update all active protocol records on a semi-annual basis,  
173       at a minimum.  
174

175           **D.     What is a trial for a serious or life-threatening disease or condition?**  
176

177       FDA has defined serious and life-threatening diseases and conditions in previous documents.  
178       Most recently, FDA discussed issues related to products intended to treat serious or life-  
179       threatening diseases and conditions in the guidance for industry on *Fast Track Drug*  
180       *Development Programs - Designation, Development, and Application Review* (November  
181       1998).<sup>9</sup> In that guidance, we stated that all conditions meeting the definition of life-threatening,  
182       as set forth at 21 CFR 312.81(a), would also be serious conditions. The term *life-threatening* is  
183       defined as (1) diseases or conditions where the likelihood of death is high unless the course of  
184       the disease is interrupted and (2) diseases or conditions with potentially fatal outcomes, where  
185       the endpoint of clinical trial analysis is survival (21 CFR 312.81(a)). All references in this  
186       document to serious diseases or conditions include life-threatening diseases and conditions.  
187

188       As FDA reiterated in the *Fast Track Guidance*, the seriousness of a disease is a matter of  
189       judgment, but generally is based on such factors as survival, day-to-day functioning, and the  
190       likelihood that the disease, if left untreated, will progress from a less severe condition to a more  
191       serious one. For example, acquired immunodeficiency syndrome (AIDS), all other stages of  
192       human immunodeficiency virus (HIV) infection, Alzheimer's disease, angina pectoris, heart  
193       failure, cancer, and many other diseases are clearly serious in their full manifestations.  
194       Furthermore, many chronic illnesses that are generally well managed by available therapy can  
195       have serious outcomes. For example, inflammatory bowel disease, asthma, rheumatoid arthritis,  
196       diabetes mellitus, systemic lupus erythematosus, depression, psychoses, and many other diseases  
197       can be serious in some or all of their phases or for certain populations.

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<sup>9</sup> CDER guidances are available at <http://www.fda.gov/cder/guidance/index.htm>.



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198 Any investigational drug that has received fast track designation would be considered a drug to  
199 treat a serious disease or condition.<sup>10</sup> Information on effectiveness trials for drugs that have  
200 received fast track designation would qualify for submission to the Clinical Trials Data Bank.

201

### **E. What is a trial to test effectiveness?**

202

203  
204 Not all trials carried out under 21 CFR part 312 are trials to test effectiveness. FDA considers all  
205 phase 2, phase 3, and phase 4 trials with efficacy endpoints as trials to test effectiveness.<sup>11</sup>

206

### **F. Which trials are provided to the public through the Clinical Trials Data Bank?**

207

208  
209  
210 Section 113 of the Modernization Act requires sponsors to submit information about clinical  
211 trials of experimental treatments for serious or life-threatening diseases and conditions when  
212 conducted under the IND regulations (42 U.S.C. 282(j)(3)(A)). Such information can be  
213 submitted at any time with the consent of the protocol sponsor, and must be submitted within 21  
214 days after a trial to test effectiveness begins. In addition, section 113 of the Modernization Act  
215 states that information on all treatment IND protocols and all Group C protocols<sup>12</sup> must be  
216 included in the Clinical Trials Data Bank.

217

218 There are situations in which there may be patients with the disease or condition for which the  
219 drug is being developed who are not adequately treated by existing therapy, who do not meet the  
220 eligibility criteria for enrollment, or who are otherwise unable to participate in a controlled  
221 clinical study. In these situations, you may have initiated an expanded access protocol or be  
222 willing to provide the drug to an individual patient through a single-patient IND or protocol  
223 exception.<sup>13</sup> The BPCA requires that you submit a description of whether, and through what  
224 procedure, you will respond to requests for protocol exception for single-patient and expanded  
225 access use of the investigational drug, particularly in children.

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<sup>10</sup> That a drug is intended to treat a serious or life-threatening disease or condition, however, does not mean that it fills an unmet medical need and qualifies for fast track designation under section 506 of the Food Drug and Cosmetic Act (21 U.S.C. 356).

<sup>11</sup> Listing a trial in the Clinical Trials Data Bank is not a guarantee that the trial design is considered adequate to support approval of a drug, nor does it reflect any judgment on the conduct, analysis, or outcome of the study.

<sup>12</sup> "Group C protocols" refers to investigational drugs designated by FDA for the treatment of specific cancers. These drugs have reproducible efficacy in one or more specific tumor types. Such a drug has altered or is likely to alter the pattern of treatment of disease and can be safely administered by properly trained physicians without specialized supportive care facilities. See National Cancer Institute Handbook for Investigators, Appendix XV, "Policy for Group C Drug Distribution," [http://ctep.info.nih.gov/HandbookText/Appendix\\_XV.htm#Proc\\_Mgmt\\_GrpC\\_Prot](http://ctep.info.nih.gov/HandbookText/Appendix_XV.htm#Proc_Mgmt_GrpC_Prot).

<sup>13</sup> There are a number of mechanisms FDA has used to provide access to promising investigational therapies. In addition to treatment INDs and treatment protocols, which are described in FDA regulations, expanded access mechanisms fall under a variety of terms, such as single patient INDs, emergency INDs, protocol exemptions, special exceptions, open label extensions, and parallel track. FDAMA has codified certain FDA regulations and practices regarding expanded patient access to experimental drugs. FDA is reviewing current regulations and practices to assure coordination with FDAMA.

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226  
227 For protocols not specifically mentioned above, sponsors should review each protocol submitted  
228 to an IND to determine if the protocol is for a serious disease or condition and if it is a trial to  
229 test effectiveness. If the protocol meets these criteria, the sponsor must submit information about  
230 the trial to the Clinical Trials Data Bank, ***unless*** the sponsor provides detailed certification to  
231 FDA that such a disclosure would substantially interfere with the timely enrollment of subjects in  
232 the investigation (42 U.S.C. 282(j)(3) and (j)(4)). Sponsors with questions on whether protocols  
233 meet the criteria for submission to the Clinical Trials Data Bank are encouraged to contact the  
234 appropriate review division for additional guidance.

### **G. Must I include information about foreign trial sites?**

235  
236  
237  
238 Yes, you must include information about foreign trials when those trials are conducted under an  
239 IND submitted to FDA and the trial meets the criteria for submission to the Clinical Trials Data  
240 Bank. Section 113 of the Modernization Act requires sponsors to submit information about  
241 specified clinical trials that are "under regulations promulgated pursuant to section 505(i) of the  
242 Federal Food, Drug, and Cosmetic Act," which are FDA's IND regulations (42 U.S.C. 282(j)(3)).  
243 Sponsors may voluntarily conduct a foreign trial under the IND regulations. Sponsors are not  
244 required to submit information to the Clinical Trials Data Bank when a foreign trial is not  
245 conducted under an IND.

## **IV. IMPLEMENTATION ISSUES**

### **A. How do I submit information to the Clinical Trials Data Bank?**

246  
247  
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250  
251 To facilitate the submission process, we have established the Web-based PRS at  
252 *ClinicalTrials.gov*. The system allows for entry of required and voluntary information about  
253 clinical trials. You or your designee can initiate submission of clinical trial information to  
254 *ClinicalTrials.gov* by completing a registration form at <http://prsinfo.clinicaltrials.gov/>.  
255 After you have entered the data, the PRS generates a receipt for use by sponsors. An electronic  
256 copy of the receipt will be sent to the FDA.

### **B. What information about trial sites must be included?**

257  
258  
259  
260 Section 113 of the Modernization Act requires sponsors to submit a description of the location of  
261 trial sites and a point of contact. To ensure an adequate description, we recommend that you  
262 provide for each individual trial site the full name of the organization, city, state, postal code, and  
263 country where the protocol is being conducted; and a central contact name and phone number.  
264 You can also provide the names and phone numbers of individual site contacts.

### **C. How long does it take for information to be made available on ClinicalTrials.gov?**

265  
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269  
270 Studies will be made available to the public through [ClinicalTrials.gov](http://ClinicalTrials.gov) within 2 to 5 days after  
271 submission by the sponsor.

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### **D. How long will information about studies remain available through ClinicalTrials.gov?**

NLM intends to maintain the Data Bank as a long-term registry of clinical trials. Therefore, in addition to information about open trials, information about closed trials will also be available through *ClinicalTrials.gov*, even after accrual and analysis are completed and the product is approved.

### **E. Can information be transferred from a sponsor computer to the PRS?**

Yes. Information can be transferred according to the format specified by the PRS. The PRS has a mechanism for uploading and downloading XML-formatted protocol records. Instructions for transferring information are provided at <http://prsinfo.clinicaltrials.gov/>

### **F. Can intermediaries acting on behalf of a sponsor submit data?**

Yes. For example, in some cases a sponsor might want to contract with an information management company to serve as an intermediary in preparing data for inclusion in *ClinicalTrials.gov*. The information management company, when authorized by the sponsor, could act on behalf of the sponsor for this purpose.

### **G. Can sponsors designate multiple individuals to be data providers?**

Yes. When sponsors register to become a PRS data provider, they will be given information, including instructions, for creating additional users for their accounts. A sponsor can control access to the account by designating users and administrators for the account.

### **H. What happens to the information submitted to the Clinical Trials Data Bank?**

Except for the IND number, serial number, and FDA center designation, all information submitted through the PRS is made available to the public at <http://clinicaltrials.gov>.

### **I. Can I submit other information to the Clinical Trials Data Bank?**

Yes. PRS is designed to permit you to submit more detailed information about a protocol. Additional data fields (e.g., projected enrollment) and their definitions are included in the PRS. You also can submit protocol information about other clinical trials under IND, including trials for a disease or condition that is not serious or any trial that is not designed to test effectiveness. Finally, you can submit information about results of a trial. This information, which, according to the structure of the Clinical Trials Data Bank, is to come from the published literature, should be linked by including the unique MEDLINE identifier for citations of publications. You can use the *link* section provided to allow pointers to Web pages directly relevant to the protocol. If you link to other Web pages from your entries, you should ensure that the links do not misbrand your products, for example, by promoting the products before the product or an

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318 indication is approved. (See 21 U.S.C. 321(n), 331(a)(b)(c)(d), 352(a)(n)  
319 <http://www.fda.gov/opacom/laws/fdcact/fdcact1.htm>.) When inputting links to other web pages,  
320 the database will instruct you that the links should be directly relevant to the protocol, and that  
321 you should not link to sites whose primary goal is to advertise or sell commercial products or  
322 services.

323

### **J. Should I continue submitting information to the ACTIS and PDQ databases?**

324

325  
326 No. All information for AIDS and cancer protocols that meet the requirements of section 113 of  
327 the Modernization Act must now be submitted to [ClinicalTrials.gov](http://ClinicalTrials.gov) through the PRS. Data from  
328 the current AIDS Clinical Trials Information System (ACTIS) and Physician's Data Query  
329 (PDQ) databases are included in [ClinicalTrials.gov](http://ClinicalTrials.gov). Information from the Rare Diseases and  
330 National Institute of Aging Databases is also included in [ClinicalTrials.gov](http://ClinicalTrials.gov).

331

### **K. Are there exemptions for submitting clinical trials information?**

332

333  
334 Information about an investigation will not be included in the data bank if you provide a detailed  
335 certification to the Secretary of Health and Human Services that disclosure of such information  
336 would substantially interfere with timely enrollment of subjects in the clinical trial and the  
337 Secretary does not disagree. If there is disagreement, the Secretary will provide a detailed written  
338 determination that such disclosure would not substantially interfere with such enrollment (42  
339 U.S.C. 282(j)(4)).

340

341 FDA has not identified specific instances when disclosure of information would substantially  
342 interfere with enrollment of subjects in a clinical investigation. We solicited comments on this  
343 topic for the purpose of including a listing of acceptable reasons for certification in the final  
344 guidance. We received no comments. Therefore, if you identify a specific instance when  
345 disclosure of information would interfere with enrollment of subjects in a clinical investigation,  
346 FDA will consider your request on a case-by-case-basis.

347

348 All requests for exemption should be forwarded to Director, Office of Special Health Issues,  
349 Office of Communications and Constituent Relations, Office of the Commissioner, HF-12, 5600  
350 Fishers Lane Rockville, MD 20857, or by email at [113trials@oc.fda.gov](mailto:113trials@oc.fda.gov), or by fax at 301-443-  
351 4555.

352

### **L. Is Institutional Review Board preapproval of the protocol listing required?**

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355 No. Section 113 of the Modernization Act does not require prior IRB approval when submitting  
356 this information to the Clinical Trials Data Bank. Current FDA guidance recommends that IRB  
357 review of listings need not occur when, as here, the system format limits the information  
358 provided to basic information, such as title, purpose of the study, protocol  
359 summary, basic eligibility criteria, study site locations, and how to contact the site for further  
360 information.<sup>14</sup>

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<sup>14</sup> The 1998 update of *Information Sheets: Guidance for Institutional Review Boards and Clinical Investigators* provides guidance on IRB review and approval of listings of clinical trials on the Internet. See <http://www.fda.gov/oc/ohrt/irbs/toc4.html#recruiting>.

## ***Contains Nonbinding Recommendations***

*Draft — Not for Implementation*

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### **M. Will FDA monitor compliance?**

A copy of the protocol listing in [ClinicalTrials.gov](http://ClinicalTrials.gov) will be sent to the FDA. FDA's Office of Special Health Issues initiated a pilot educational program in 2002 that included a component to evaluate compliance. The primary objective of the pilot program is to educate sponsors about the existence of the guidance document and the availability of the online PRS data entry tool. The secondary objective of the pilot program is to evaluate the success of the educational initiative. The pilot program will measure the number of protocols (voluntary and required) made available through the [ClinicalTrials.gov](http://ClinicalTrials.gov) database. Data from the completed project will help senior FDA officials assess the need for further efforts to facilitate or perhaps compel participation in [ClinicalTrials.gov](http://ClinicalTrials.gov).

### **N. What information about protocol exceptions, single-patient use, and expanded access protocols must I include?**

There are situations in which there may be patients with the disease or condition for which the drug is being developed who are not adequately treated by existing therapy, who do not meet the eligibility criteria for enrollment, or who are otherwise unable to participate in a controlled clinical study. In such a situation, you may wish to provide the drug to a patient through a protocol exception/exemption, single patient IND, or expanded access protocol.

The BPCA amended Section 113 of the Modernization Act to require that you submit, in addition to the information already included in the Clinical Trials Data Bank, a description of whether and through what procedure you will respond to requests for protocol exception, with appropriate safeguards, for single-patient and expanded access use of the investigational drug, particularly in children.

The PRS includes a mechanism for providing information about protocol exceptions, single-patient INDs, and expanded access protocols. In order to comply with the BPCA amendment to section 113 of the Modernization Act, we suggest that you address the following two questions and provide a brief description as described below. This information is required for each new protocol that is listed in the data bank; we encourage you also to provide this information for protocols currently open to enrollment.

- Is this investigational drug available for use in adults through a protocol exception, single-patient IND, or expanded access protocol?  
Yes No
- Is this investigational drug available for use in children through a protocol exception, single-patient IND, or expanded access protocol?  
Yes No
- Brief description of the procedure for responding to requests for expanded access, including contact number and/or email address.