

110TH CONGRESS
2^D SESSION

H. R. 6047

To amend the Federal Food, Drug, and Cosmetic Act to mandate early access by desperately ill patients to treatment use of new drugs under clinical investigation for a serious or immediately life-threatening disease condition for whom no comparable or satisfactory drug or other therapy is available.

IN THE HOUSE OF REPRESENTATIVES

MAY 14, 2008

Mr. JONES of North Carolina introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to mandate early access by desperately ill patients to treatment use of new drugs under clinical investigation for a serious or immediately life-threatening disease condition for whom no comparable or satisfactory drug or other therapy is available.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Early Access to Treat-
5 ment Act”.

1 **SEC. 2. FINDINGS.**

2 Congress finds the following:

3 (1) Section 506(a)(1) of the Federal Food,
4 Drug, and Cosmetic Act (21 U.S.C. 356(a)(1)) re-
5 quires the Secretary of Health and Human Services,
6 at the request of the sponsor of a new drug, to “fa-
7 cilitate the development and expedite the review of
8 such drug if it is intended for the treatment of a se-
9 rious or life-threatening condition and it dem-
10 onstrates the potential to address unmet medical
11 needs for such a condition”, designating such a drug
12 to be a “fast track product”.

13 (2) Section 506(b)(1) of such Act, however,
14 provides that the Secretary need not approve an ap-
15 plication for approval of a fast track product, even
16 though the Secretary has determined “that the prod-
17 uct has an effect on a clinical endpoint or on a sur-
18 rogate endpoint that is reasonably likely to predict
19 clinical benefit”.

20 (3) Section 312.34(a) of title 21, of the Code
21 of Federal Regulations, provides for treatment use
22 of a new drug, although such “drug . . . is not yet
23 approved for marketing,” but is “under clinical in-
24 vestigation for a serious or immediately life-threat-
25 ening disease condition in patients for whom no

1 comparable or satisfactory alternative drug or other
2 therapy is available”.

3 (4) Although the stated purpose of both current
4 law and regulations, as stated under section
5 312.34(a) of such title, is “to facilitate the avail-
6 ability of promising new drugs to desperately ill pa-
7 tients as early in the drug development process as
8 possible, before general marketing begins”, in prac-
9 tice applications for approval of promising therapies
10 intended to treat such desperately ill patients suf-
11 fering from serious and immediately life-threatening
12 diseases for whom no comparable or alternative drug
13 or other therapy is available have encountered un-
14 justified delays and denials.

15 (5) As a consequence of such delays and denials
16 of applications for such treatment use, desperately ill
17 patients suffering from serious and immediately life-
18 threatening disease conditions have been denied the
19 last, and perhaps the only, best chance for treatment
20 protocols that might have saved their lives.

21 (6) Applications for such treatment use ought
22 not be subject to the discretion of the Secretary, but
23 should be granted, in all cases, if such applications
24 meet the statutory criteria governing such applica-
25 tions.

1 (7) Applications for fast track product review
2 ought not be subject to the discretion of the Sec-
3 retary, but should be granted, in all cases, if such
4 applications meet the statutory criteria governing
5 such applications as set forth in section 506(b)(1) of
6 the Federal Food, Drug, and Cosmetic Act (21
7 U.S.C. 356(b)(1)).

8 **SEC. 3. TREATMENT USE APPROVAL SYSTEM.**

9 Section 505 of the Federal Food, Drug, and Cosmetic
10 Act (21 U.S.C. 355) is amended by adding at the end the
11 following new subsection:

12 “(v) TREATMENT USE OF AN INVESTIGATIONAL
13 DRUG.—

14 “(1) IN GENERAL.—A drug that is not ap-
15 proved for marketing, but is under clinical investiga-
16 tion for a serious or immediately life-threatening dis-
17 ease condition in patients for whom no comparable
18 or satisfactory alternative drug or other therapy is
19 available, shall be made available for treatment use
20 by an individual patient if—

21 “(A) the drug is intended to treat a serious
22 or immediately life-threatening disease condi-
23 tion;

24 “(B) there is no comparable or satisfactory
25 alternative drug or other therapy available to

1 treat that stage of the disease in the person for
2 whom the drug is intended;

3 “(C) the drug is under investigation in a
4 controlled clinical trial as an investigational new
5 drug under subsection (i) in effect for the trial
6 or all clinical trials have been completed; and

7 “(D) an application for treatment use has
8 been filed in writing with the Food and Drug
9 Administration by a licensed practitioner and
10 such application sets forth—

11 “(i) the specific intended use of the
12 drug;

13 “(ii) an explanation of the rationale
14 for use of the drug, including an expla-
15 nation as to why the use of the investiga-
16 tional drug is necessary in the treatment of
17 the individual patient;

18 “(iii) a treatment protocol, including
19 the method of the administration of the
20 drug and dosages;

21 “(iv) a statement of the qualifications
22 of the licensed practitioner to use the in-
23 vestigational drug for the intended treat-
24 ment use;

1 “(v) a statement of the licensed prac-
2 titioner’s familiarity with information of
3 the drug’s safety and effectiveness derived
4 from previous clinical and nonclinical expe-
5 rience with the drug; and

6 “(vi) a signed and notarized state-
7 ment of the individual patient, or guard-
8 ian, if any, setting forth in detail—

9 “(I) the information about the
10 drug’s safety and effectiveness;

11 “(II) risks disclosed to the pa-
12 tient; and

13 “(III) the patient’s informed con-
14 sent to the treatment protocol to be
15 administered by the licensed practi-
16 tioner.

17 “(2) PERMISSION FOR TREATMENT USE.—

18 “(A) DEADLINE.—Within 30 days of the
19 filing of an application for treatment use in
20 compliance with paragraph (1), the Secretary
21 shall grant permission to the sponsor of an in-
22 vestigational drug to furnish such drug to the
23 submitting licensed practitioner under such
24 terms as the sponsor, licensed practitioner, and
25 patient determine among themselves to be ap-

1 appropriate, including any agreement to waive li-
2 ability.

3 “(B) TREATMENT OF EMERGENCIES.—If
4 the need for an investigational drug arises in an
5 emergency situation that does not allow time
6 for submission of such an application for treat-
7 ment use in writing, such application may be
8 transmitted to the Secretary by telephone or by
9 other means of rapid communication, and shall
10 be acted on rapidly, but in no case later than
11 30 days after the date of receipt of the applica-
12 tion.

13 “(3) DEFINITIONS.—In paragraph (1)—

14 “(A) the term ‘serious’ means, with respect
15 to a disease or condition, a disease or condition
16 that causes major irreversible morbidity; and

17 “(B) the term ‘immediately life-threat-
18 ening’ means, with respect to a disease or con-
19 dition—

20 “(i) a disease or condition where the
21 likelihood of death is high unless the
22 course of the disease or condition is inter-
23 rupted; or

1 “(ii) a disease or condition with po-
2 tentially fatal outcomes, where the end
3 point of clinical trial analysis is survival.”.

4 **SEC. 4. REMOVING DISCRETION IN APPROVAL OF APPLICA-**
5 **TION FOR FAST TRACK PRODUCTS.**

6 Section 506(b)(1) of the Federal Food, Drug, and
7 Cosmetic Act (21 U.S.C. 356(b)(1)) is amended by strik-
8 ing “may” and inserting “shall”.

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