

safe use and supporting findings of substantial evidence of effectiveness; and

“(iii) use the criteria described in clauses (i) and (ii) in a manner that is appropriate for drugs intended for the treatment of rare diseases or conditions.

“(b) IMPROVING INSTITUTIONAL REVIEW BOARD REVIEW OF SINGLE PATIENT EXPANDED ACCESS PROTOCOL.—Not later than 1 year after the date of enactment of this Act [Aug. 18, 2017], the Secretary, acting through the Commissioner of Food and Drugs, shall issue guidance or regulations, or revise existing guidance or regulations, to streamline the institutional review board review of individual patient expanded access protocols submitted under [section] 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb(b)). To facilitate the use of expanded access protocols, any guidance or regulations so issued or revised may include a description of the process for any person acting through a physician licensed in accordance with State law to request that an institutional review board chair (or designated member of the institutional review board) review a single patient expanded access protocol submitted under such section 561(b) for a drug. The Secretary shall update any relevant forms associated with individual patient expanded access requests under such section 561(b) as necessary.”

§ 360bbb-0. Expanded access policy required for investigational drugs

(a) In general

The manufacturer or distributor of one or more investigational drugs for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions shall make available the policy of the manufacturer or distributor on evaluating and responding to requests submitted under section 360bbb(b) of this title for provision of such a drug.

(b) Public availability of expanded access policy

The policies under subsection (a) shall be made public and readily available, such as by posting such policies on a publicly available Internet website. Such policies may be generally applicable to all investigational drugs of such manufacturer or distributor.

(c) Content of policy

A policy described in subsection (a) shall include—

- (1) contact information for the manufacturer or distributor to facilitate communication about requests described in subsection (a);
- (2) procedures for making such requests;
- (3) the general criteria the manufacturer or distributor will use to evaluate such requests for individual patients, and for responses to such requests;
- (4) the length of time the manufacturer or distributor anticipates will be necessary to acknowledge receipt of such requests; and
- (5) a hyperlink or other reference to the clinical trial record containing information about the expanded access for such drug that is required under section 282(j)(2)(A)(i)(II)(gg) of title 42.

(d) No guarantee of access

The posting of policies by manufacturers and distributors under subsection (a) shall not serve as a guarantee of access to any specific investigational drug by any individual patient.

(e) Revised policy

Nothing in this section shall prevent a manufacturer or distributor from revising a policy required under this section at any time.

(f) Application

This section shall apply to a manufacturer or distributor with respect to an investigational drug beginning on the earlier of—

- (1) the first initiation of a phase 2 or phase 3 study (as such terms are defined in section 312.21(b) and (c) of title 21, Code of Federal Regulations (or any successor regulations)) with respect to such investigational drug; or
- (2) as applicable, 15 days after the drug receives a designation as a breakthrough therapy, fast track product, or regenerative advanced therapy under subsection (a), (b), or (g), respectively, of section 356 of this title.

(June 25, 1938, ch. 675, §561A, as added Pub. L. 114–255, div. A, title III, §3032, Dec. 13, 2016, 130 Stat. 1100; amended Pub. L. 115–52, title VI, §610(c), Aug. 18, 2017, 131 Stat. 1053.)

Editorial Notes

AMENDMENTS

2017—Subsec. (f). Pub. L. 115–52 substituted “earlier” for “later” in introductory provisions, added par. (2), redesignated former par. (2) as (1), and struck out former par. (1) which read as follows: “the date that is 60 calendar days after December 13, 2016; or”.

§ 360bbb-0a. Investigational drugs for use by eligible patients

(a) Definitions

For purposes of this section—

(1) the term “eligible patient” means a patient—

(A) who has been diagnosed with a life-threatening disease or condition (as defined in section 312.81 of title 21, Code of Federal Regulations (or any successor regulations));

(B) who has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician, who—

- (i) is in good standing with the physician’s licensing organization or board; and
- (ii) will not be compensated directly by the manufacturer for so certifying; and

(C) who has provided to the treating physician written informed consent regarding the eligible investigational drug, or, as applicable, on whose behalf a legally authorized representative of the patient has provided such consent;

(2) the term “eligible investigational drug” means an investigational drug (as such term is used in section 360bbb of this title)—

(A) for which a Phase 1 clinical trial has been completed;

(B) that has not been approved or licensed for any use under section 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262];

(C)(i) for which an application has been filed under section 355(b) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)]; or