''(B) TOPICS.—The Secretary shall make available on the internet website of the Food and Drug Administration a report on the topics discussed at the meeting described in subparagraph (A) within 90 days of such meeting. Such topics shall include discussion of—

''(i) the rationale for, and potential barriers for patients created by, research clinical trial inclusion and exclusion criteria;

''(ii) how appropriate patient populations can benefit from the results of trials that employ alternative designs;

''(iii) barriers to participation in clinical trials, including—

''(I) information regarding any potential risks and benefits of participation;

''(II) regulatory, geographical, and socioeconomic barriers; and

''(III) the impact of exclusion criteria on the enrollment in clinical trials of particular populations, including infants and children, pregnant and lactating women, seniors, individuals with advanced disease, and individuals with comorbid conditions;

''(iv) clinical trial designs and methods, including expanded access trials, that increase enrollment of more diverse patient populations, when appropriate, while facilitating the collection of data to establish safe use and support substantial evidence of effectiveness, including data obtained from expanded access trials; and

''(v) how changes to clinical trial inclusion and exclusion criteria may impact the complexity and length of clinical trials, the data necessary to demonstrate safety and effectiveness, and potential approaches to mitigating those impacts.

''(2) REPORT.—Not later than 1 year after the Secretary issues the report under paragraph (1)(B), the Comptroller General of the United States shall report to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives on individual access to investigational drugs through the expanded access program under section 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb(b)). The report shall include—

''(A) a description of actions taken by manufacturers and distributors under section 561A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–0);

''(B) consideration of whether Form FDA 3926 and the guidance documents titled 'Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers' and 'Individual Patient Expanded Access Applications: Form FDA 3926', issued by the Food and Drug Administration in June 2016, have reduced application burden with respect to individuals and physicians seeking access to investigational new drugs pursuant to section 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and improved clarity for patients, physicians, and drug manufacturers about such process;

''(C) consideration of whether the guidance or regulations issued to implement section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) have improved access for individual patients to investigational drugs who do not qualify for clinical trials of such investigational drugs, and what barriers to such access remain;

''(D) an assessment of methods patients and health care providers use to engage with the Food and Drug Administration or drug sponsors on expanded access; and

''(E) an analysis of the Secretary's report under paragraph (1)(B).

''(3) GUIDANCE.—

''(A) IN GENERAL.—Not later than 1 year after the publication of the report under paragraph (1)(B), the Secretary, acting through the Commissioner of Food and Drugs, shall issue one or more draft guidances regarding eligibility criteria for clinical trials. Not later than 1 year after the public comment period on each such draft guidance ends, the Secretary shall issue a revised draft guidance or final guidance.

''(B) CONTENTS.—The guidance documents described in subparagraph (A) shall address methodological approaches that a manufacturer or sponsor of an investigation of a new drug may take to—

''(i) broaden eligibility criteria for clinical trials and expanded access trials, especially with respect to drugs for the treatment of serious and life-threatening conditions or diseases for which there is an unmet medical need;

''(ii) develop eligibility criteria for, and increase trial recruitment to, clinical trials so that enrollment in such trials more accurately reflects the patients most likely to receive the drug, as applicable and as appropriate, while establishing 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)(ii)(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

(ii) that is under investigation in a clinical trial that—

(I) is intended to form the primary basis of a claim of effectiveness in support of approval or licensure under section 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262]; and

(II) is the subject of an active investigational new drug application under section 355(i) of this title or section 351(a)(3) of the Public Health Service Act [42 U.S.C. 262(a)(3)], as applicable; and

(D) the active development or production of which is ongoing and has not been discontinued by the manufacturer or placed on clinical hold under section 355(i) of this title; and

(3) the term ''phase 1 trial'' means a phase 1 clinical investigation of a drug as described in section 312.21 of title 21, Code of Federal Regulations (or any successor regulations).

(b) Exemptions

Eligible investigational drugs provided to eligible patients in compliance with this section are exempt from sections 352(f), 353(b)(4), 355(a), and 355(i) of this title, section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)], and parts 50, 56, and 312 of title 21, Code of Federal Regulations (or any successor regulations), provided that the sponsor of such eligible investigational drug or any person who manufactures, distributes, prescribes, dispenses, introduces or delivers for introduction into interstate commerce, or provides to an eligible patient an eligible investigational drug pursuant to this section is in compliance with the applicable requirements set forth in sections 312.6, 312.7, and 312.8(d)(1) of title 21, Code of Federal Regulations (or any successor regulations) that apply to investigational drugs.

(c) Use of clinical outcomes

(1) In general

Notwithstanding any other provision of this chapter, the Public Health Service Act [42 U.S.C. 201 et seq.], or any other provision of Federal law, the Secretary may not use a clinical outcome associated with the use of an eligible investigational drug pursuant to this section to delay or adversely affect the review or approval of such drug under section 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262] unless—