

Andrea Sloan CURE Act Section-by-Section

SECTION 1. SHOT TITLE.

The Andrea Sloan Compassionate Use Reform and Enhancement Act, or the Andrea Sloan CURE Act.

SEC. 2. EXPANDED ACCESS POLICY AS CONDITION OF EXPEDITED APPROVAL.

This section requires the sponsor of a covered breakthrough drug to submit to the Secretary of the Department of Health and Human Services (HHS) and make publicly available the sponsor's expanded access policy if the sponsor accepts expanded access requests. The policy shall include—

- (A) A single point of contact who receives and processes such requests;
- (B) Procedures for making such requests;
- (C) The minimum criteria for the sponsor's consideration or approval of such requests; and
- (D) The amount of time the sponsor anticipates will be necessary to make a decision on such requests.

This section also defines "covered breakthrough drug" as a drug—

- (A) That is designated as a breakthrough therapy or fast track product or is approved under accelerated approval;
- (B) That is a qualified infectious disease product; or
- (C) The sponsor of which is awarded a priority review voucher.

SEC. 3. NOTIFICATION OF SUBMITTERS OF COMPASSIONATE USE REQUESTS.

This section requires the manufacturer or distributor of a drug to provide a person (or their physician) with a written notice if that person's request for expanded access is denied. The notice must be provided within 5 days after the date of such a denial, and include an explanation for the denial.

SEC. 4. GAO QUALITATIVE ANALYSIS ON INDIVIDUAL PATIENT ACCESS TO UNAPPROVED THERAPIES AND DIAGNOSTICS.

This section requires the Government Accountability Office (GAO), not later than 180 days after the date of enactment of the *Andrea Sloan CURE Act*, to submit to Congress a report containing an analysis of individual patient requests for expanded access and recommendations for improving such access. In preparing the report, the GAO shall conduct a qualitative analysis of the following:

- (1) Whether there are any identifiable patterns in expanded access requests, such as the types of indications for which such request are sought or the reasons for the denial of such requests.
- (2) What the primary barriers are to drug sponsors granting requests for individual patient expanded access.
- (3) How the HHS Secretary evaluates the safety and efficacy data submitted in connection with expanded access requests.
- (4) The amount of time that—
 - (A) A physician typically takes to complete the paperwork necessary to make a request for expanded access;
 - (B) The drug sponsor takes to process such a request and to issue a decision with respect to such a request; and
 - (C) The HHS Secretary takes to process such a request and to issue a decision with respect to such a request.
- (5) How regulations, guidance, policies, or practices may be modified, streamlined, expanded, or discontinued to reduce or prevent delays in approving such requests.
- (6) The number of requests that—
 - (A) Were approved by drug sponsors and the Food and Drug Administration (FDA);
 - (B) Were approved by drug sponsors but denied by the FDA; and

- (C) Were denied by drug sponsors.
- (7) How to encourage drug sponsors to grant requests for expanded access, including for emergency use, intermediate-size patient populations, and large patient populations.
- (8) Whether and to what extent adverse events reported to the HHS Secretary as a result of expanded access use affected the development or approval of any drug or device.

SEC. 5. EXPANDED ACCESS TASK FORCE

This section establishes an Expanded Access Task Force at HHS to explore mechanisms for improving the access individual patients have to investigational drugs. The Task Force shall be established within 90 days after the GAO submits the report to Congress required in Sec. 4 of this bill. There will be not more than 9 voting members of the Task Force appointed as follows:

- (A) One member to serve as chairman, appointed by the Speaker of the House.
- (B) One representative from HHS, appointed by the HHS Secretary.
- (C) Four representatives appointed by the House Majority Leader, in consultation with the House Minority Leader and the Chairman and Ranking Member of the Energy and Commerce Committee, including—
 - i. One representative of a biopharmaceutical company of less than 250 full-time employees;
 - ii. One representative of the rare disease patient community;
 - iii. One representative of the health care provider community; and
 - iv. One bioethicist.
- (D) Three representatives appointed by the Senate Majority Leader, in consultation with the Senate Minority Leader and the Chairman and Ranking Member of the Health, Education, Labor, and Pensions Committee, including—
 - i. One representative of a biopharmaceutical company;
 - ii. One representative of the patient community; and
 - iii. One representative of the health care payor community.

This section also explains that Task Force members shall serve without pay, and that the function of the Task Force is to comprehensively evaluate the access individual patients have to investigational drugs, taking into account—

- (1) The unique challenges faced by children with likely fatal diseases and no comparable or satisfactory treatment options;
- (2) Possible incentives for biopharmaceutical companies and providers to approve expanded access requests;
- (3) How the FDA interprets and takes into consideration adverse event data that is reported from use of unapproved drugs;
- (4) Ways to streamline and standardize the process for submitting expanded access requests; and
- (5) The costs incurred by biopharmaceutical companies for providing patients with access to unapproved drugs.

Within 180 days after the Task Force is convened, the Task Force shall transmit to the House Energy and Commerce Committee and the Senate Health, Education, Labor, and Pensions Committee an electronic report describing the Task Force's recommendations for improving individual patient expanded access. The Task Force shall terminate after submitting their report.

SEC. 6. FINALIZING DRAFT GUIDANCE ON EXPANDED ACCESS

This sections requires HHS to finalize its May 2013 draft guidance on expanded access within 180 days after the Expanded Access Task Force submits its report to Congress required in Sec. 5 of this bill, and to clearly define in such guidance how the FDA interprets and uses adverse drug event data that is reported from use of unapproved drugs.