Federal Food, Drug, and Cosmetic Act

CHAPTER V--DRUGS AND DEVICES

SUBCHAPTER E--GENERAL PROVISIONS RELATING TO DRUGS AND DEVICES

SEC. 561. [21 U.S.C. 360bbb] EXPANDED ACCESS TO UNAPPROVED THERAPIES AND DIAGNOSTICS.

(a) EMERGENCY SITUATIONS.—The Secretary may, under appropriate conditions determined by the Secretary, authorize the shipment of investigational drugs or investigational devices for the diagnosis, monitoring, or treatment of a serious disease or condition in emergency situations.

(b) INDIVIDUAL PATIENT ACCESS TO INVESTIGATIONAL PRODUCTS INTENDED FOR SERIOUS DISEASES.—Any person, acting through a physician licensed in accordance with State law, may request from a manufacturer or distributor, and any manufacturer or distributor may, after complying with the provisions of this subsection, provide to such physician an investigational drug or investigational device for the diagnosis, monitoring, or treatment of a serious disease or condition if—

(1) the licensed physician determines that the person has no comparable or satisfactory alternative therapy available to diagnose, monitor, or treat the disease or condition involved, and that the probable risk to the person from the investigational drug or investigational device is not greater than the probable risk from the disease or condition;

(2) the Secretary determines that there is sufficient evidence of safety and effectiveness to support the use of the investigational drug or investigational device in the case described in paragraph (1);

(3) the Secretary determines that provision of the investigational drug or investigational device will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval; and

(4) the sponsor, or clinical investigator, of the investigational drug or investigational device submits to the Secretary a clinical protocol consistent with the provisions of section 505(i) or 520(g), including any regulations promulgated under section 505(i) or 520(g), describing the use of the investigational drug or investigational device in a single patient or a small group of patients.

(c) TREATMENT INVESTIGATIONAL NEW DRUG APPLICATIONS AND TREATMENT INVESTIGATIONAL DEVICE EXEMPTIONS.—Upon submission by a sponsor or a physician of a protocol intended to provide widespread access to an investigational drug or investigational device for eligible patients (referred to in this subsection as an ‘‘expanded access protocol’’), the Secretary shall permit such

investigational drug or investigational device to be made available for expanded access under a treatment investigational new drug application or treatment investigational device exemption if the Secretary determines that—

(1) under the treatment investigational new drug application or treatment investigational device exemption, the investigational drug or investigational device is intended for use in the diagnosis, monitoring, or treatment of a serious or immediately life-threatening disease or condition;

(2) there is no comparable or satisfactory alternative therapy available to diagnose, monitor, or treat that stage of disease or condition in the population of patients to which the investigational drug or investigational device is intended to be administered;

(3)(A) the investigational drug or investigational device is under investigation in a controlled clinical trial for the use described in paragraph (1) under an investigational drug application in effect under section 505(i) or investigational device exemption in effect under section 520(g); or

(B) all clinical trials necessary for approval of that use of the investigational drug or investigational device have been completed;

(4) the sponsor of the controlled clinical trials is actively pursuing marketing approval of the investigational drug or investigational device for the use described in paragraph (1) with due diligence;

(5) in the case of an investigational drug or investigational device described in paragraph (3)(A), the provision of the investigational drug or investigational device will not interfere with the enrollment of patients in ongoing clinical investigations under section 505(i) or 520(g);

(6) in the case of serious diseases, there is sufficient evidence of safety and effectiveness to support the use described in paragraph (1); and

(7) in the case of immediately life-threatening diseases, the available scientific evidence, taken as a whole, provides a reasonable basis to conclude that the investigational drug or investigational device may be effective for its intended use and would not expose patients to an unreasonable and significant risk of illness or injury.

A protocol submitted under this subsection shall be subject to the provisions of section 505(i) or 520(g), including regulations promulgated under section 505(i) or 520(g). The Secretary may inform national, State, and local medical associations and societies, voluntary health associations, and other appropriate persons about the availability of an investigational drug or investigational device under expanded access protocols submitted under this subsection. The information provided by the Secretary, in accordance with the preceding sentence, shall be the same type of information that is required by section 402(j)(3) of the Public Health Service Act.

(d) TERMINATION.—The Secretary may, at any time, with respect to a sponsor, physician, manufacturer, or distributor described in this section, terminate expanded access provided under this section for an investigational drug or investigational device if the requirements under this section are no longer met.

(e) DEFINITIONS.—In this section, the terms ‘‘investigational drug’’, ‘‘investigational device’’, ‘‘treatment investigational new drug application’’, and ‘‘treatment investigational device exemption’’ shall have the meanings given the terms in regulations prescribed by the Secretary.

SEC. 562. [21 U.S.C. 360bbb–1] **DISPUTE RESOLUTION.**

If, regarding an obligation concerning drugs or devices under this Act or section 351 of the Public Health Service Act, there is a scientific controversy between the Secretary and a person who is a sponsor, applicant, or manufacturer and no specific provision of the Act involved, including a regulation promulgated under such Act, provides a right of review of the matter in controversy, the Secretary shall, by regulation, establish a procedure under which such sponsor, applicant, or manufacturer may request a review of such controversy, including a review by an appropriate scientific advisory panel described in section 505(n) or an advisory committee described in section 515(g)(2)(B). Any such review shall take place in a timely manner. The Secretary shall promulgate such regulations within 1 year after the date of the enactment of the Food and Drug Administration Modernization Act of 1997.

SEC. 563. [21 U.S.C. 360bbb–2] **CLASSIFICATION OF PRODUCTS.**

(a) REQUEST.—A person who submits an application or submission (including a petition, notification, and any other similar form of request) under this Act for a product, may submit a request to the Secretary respecting the classification of the product as a drug, biological product, device, or a combination product subject to section 503(g) or respecting the component of the Food and Drug Administration that will regulate the product. In submitting the request, the person shall recommend a classification for the product, or a component to regulate the product, as appropriate.

(b) STATEMENT.—Not later than 60 days after the receipt of the request described in subsection (a), the Secretary shall determine the classification of the product under subsection (a), or the component of the Food and Drug Administration that will regulate the product, and shall provide to the person a written statement that identifies such classification or such component, and the reasons for such determination. The Secretary may not modify such statement except with the written consent of the person, or for public health reasons based on scientific evidence.

(c) INACTION OF SECRETARY.—If the Secretary does not provide the statement within the 60-day period described in subsection (b), the recommendation made by the person under subsection (a) shall be considered to be a final determination by the Secretary of such classification of the product, or the component of the Food and Drug Administration that will regulate the product, as applicable, and may not be modified by the Secretary except with the written consent of the person, or for public health reasons based on scientific evidence.

SEC. 564. [21 USC 360bbb-3]- **AUTHORIZATION FOR MEDICAL PRODUCTS FOR USE IN EMERGENCIES.**

(a) In General.--(1) Emergency uses.--Notwithstanding sections 505, 510(k), and 515 of this Act and section 351 of the Public Health Service Act, and subject to the provisions of this section, the Secretary may authorize the introduction into interstate commerce, during the effective period of a declaration under subsection (b), of a drug, device, or biological product intended for use in an actual or potential emergency (referred to in this section as an "emergency use").

(2) Approval status of product.--An authorization under paragraph (1) may authorize an emergency use of a product that--

(A) is not approved, licensed, or cleared for commercial distribution under a provision of law referred to in such paragraph (referred to in this section as an "unapproved product"); or

(B) is approved, licensed, or cleared under such a provision, but which use is not under such provision an approved, licensed, or cleared use of the product (referred to in this section as an "unapproved use of an approved product").

(3) Relation to other uses.--An emergency use authorized under paragraph (1) for a product is in addition to any other use that is authorized for the product under a provision of law referred to in such paragraph.

(4) Definitions.--For purposes of this section:

(A) The term "biological product" has the meaning given such term in section 351 of the Public Health Service Act.

(B) The term "emergency use" has the meaning indicated for such term in paragraph (1).

(C) The term "product" means a drug, device, or biological product.

(D) The term "unapproved product" has the meaning indicated for such term in paragraph (2)(A).

(E) The term "unapproved use of an approved product" has the meaning indicated for such term in paragraph (2)(B).

(b) DECLARATION OF EMERGENCY.—

(1) IN GENERAL.—The Secretary may declare an emergency justifying the authorization under this subsection for a product on the basis of—

(A) a determination by the Secretary of Homeland Security that there is a domestic emergency, or a significant potential for a domestic emergency, involving a heightened risk of attack with a specified biological, chemical, radiological, or nuclear agent or agents;

(B) a determination by the Secretary of Defense that there is a military emergency, or a significant potential for a military emergency, involving a heightened risk to United States military forces of attack with a specified biological, chemical, radiological, or nuclear agent or agents; or

(C) a determination by the Secretary of a public health emergency under section 319 of the Public Health Service Act that affects, or has a significant potential to affect, national security, and that involves a specified biological, chemical, radiological, or nuclear agent or agents, or a specified disease or condition that may be attributable to such agent or agents.

(2) Termination of declaration.--

(A) In general.--A declaration under this subsection shall terminate upon the earlier of--

(i) a determination by the Secretary, in consultation with the Secretary of Defense, that the circumstances described in paragraph (1) have ceased to exist; or

(ii) the expiration of the one-year period beginning on the date on which the declaration is made.

(B) Renewal.--Notwithstanding subparagraph (A), the Secretary may renew a declaration under this subsection, and this paragraph shall apply to any such renewal.

(C) Disposition of product.--If an authorization under this section with respect to an unapproved product ceases to be effective as a result of a termination under subparagraph (A) of this paragraph, the Secretary shall consult with the manufacturer of such product with respect to the appropriate disposition of the product.

(3) Advance notice of termination.--The Secretary shall provide advance notice that a declaration under this subsection will be terminated. The period of advance notice shall be a period reasonably determined to provide--

(A) in the case of an unapproved product, a sufficient period for disposition of the product, including the return of such product (except such quantities of product as are necessary to provide for continued use consistent with subsection (f)(2)) to the manufacturer (in the case of a manufacturer that chooses to have such product returned); and

(B) in the case of an unapproved use of an approved product, a sufficient period for the disposition of any labeling, or any information under subsection (e)(2)(B)(ii), as the case may be, that was provided with respect to the emergency use involved.

(4) Publication.--The Secretary shall promptly publish in the Federal Register each declaration, determination, advance notice of termination, and renewal under this subsection.

(c) Criteria for Issuance of Authorization.--The Secretary may issue an authorization under this section with respect to the emergency use of a product only if, after consultation with the Director of the National Institutes of Health and the Director of the Centers for Disease Control and Prevention (to the extent feasible and appropriate given the circumstances of the emergency involved), the Secretary concludes--

(1) that an agent specified in a declaration under subsection (b) can cause a serious or life-threatening disease or condition;

(2) that, based on the totality of scientific evidence available to the Secretary, including data from adequate and well-controlled clinical trials, if available, it is reasonable to believe that--

(A) the product may be effective in diagnosing, treating, or preventing--

(i) such disease or condition; or

(ii) a serious or life-threatening disease or condition caused by a product authorized under this section, approved or cleared under this Act, or licensed under section 351 of the Public Health Service Act, for diagnosing, treating, or preventing such a disease or condition caused by such an agent; and

(B) the known and potential benefits of the product, when used to diagnose, prevent, or treat such disease or condition, outweigh the known and potential risks of the product;

(3) that there is no adequate, approved, and available alternative to the product for diagnosing, preventing, or treating such disease or condition; and

(4) that such other criteria as the Secretary may by regulation prescribe are satisfied.

(d) Scope of Authorization.--An authorization of a product under this section shall state--
(1) each disease or condition that the product may be used to diagnose, prevent, or treat within the scope of the authorization;

(2) the Secretary's conclusions, made under subsection (c)(2)(B), that the known and potential benefits of the product, when used to diagnose, prevent, or treat such disease or condition, outweigh the known and potential risks of the product; and

(3) the Secretary's conclusions, made under subsection (c), concerning the safety and potential effectiveness of the product in diagnosing, preventing, or treating such diseases or conditions, including an assessment of the available scientific evidence.

(e) Conditions of Authorization.—

(1) Unapproved product.--

(A) Required conditions.--With respect to the emergency use of an unapproved product, the Secretary, to the extent practicable given the circumstances of the emergency, shall, for a person who carries out any activity for which the authorization is issued, establish such conditions on an authorization under this section as the Secretary finds necessary or appropriate to protect the public health, including the following:

(i) Appropriate conditions designed to ensure that health care professionals administering the product are informed--

(I) that the Secretary has authorized the emergency use of the product;

(II) of the significant known and potential benefits and risks of the emergency use of the product, and of the extent to which such benefits and risks are unknown; and

(III) of the alternatives to the product that are available, and of their benefits and risks.

(ii) Appropriate conditions designed to ensure that individuals to whom the product is administered are informed--

(I) that the Secretary has authorized the emergency use of the product;

(II) of the significant known and potential benefits and risks of such use, and of the extent to which such benefits and risks are unknown; and

(III) of the option to accept or refuse administration of the product, of the consequences, if any, of refusing administration of the product, and of the alternatives to the product that are available and of their benefits and risks.

(iii) Appropriate conditions for the monitoring and reporting of adverse events associated with the emergency use of the product.

(iv) For manufacturers of the product, appropriate conditions concerning recordkeeping and reporting, including records access by the Secretary, with respect to the emergency use of the product.

(B) Authority for additional conditions.--With respect to the emergency use of an unapproved product, the Secretary may, for a person who carries out any activity for which the authorization is issued, establish such conditions on an authorization under this section as the Secretary finds necessary or appropriate to protect the public health, including the following:

(i) Appropriate conditions on which entities may distribute the product with respect to the emergency use of the product (including limitation to distribution by government entities), and on how distribution is to be performed.

(ii) Appropriate conditions on who may administer the product with respect to the emergency use of the product, and on the categories of individuals to whom, and the circumstances under which, the product may be administered with respect to such use.

(iii) Appropriate conditions with respect to the collection and analysis of information, during the period when the authorization is in effect, concerning the safety and effectiveness of the product with respect to the emergency use of such product.

(iv) For persons other than manufacturers of the product, appropriate conditions concerning recordkeeping and reporting, including records access by the Secretary, with respect to the emergency use of the product.

(2) Unapproved use.--With respect to the emergency use of a product that is an unapproved use of an approved product:

(A) For a manufacturer of the product who carries out any activity for which the authorization is issued, the Secretary shall, to the extent practicable given the circumstances of the emergency, establish conditions described in clauses (i) and (ii) of paragraph (1)(A), and may establish conditions described in clauses (iii) and (iv) of such paragraph.

(B)(i) If the authorization under this section regarding the emergency use authorizes a change in the labeling of the product, but the manufacturer of the product chooses not to make such change, such authorization may not authorize distributors of the product or any other person to alter or obscure the labeling provided by the manufacturer.

(ii) In the circumstances described in clause (i), for a person who does not manufacture the product and who chooses to act under this clause, an authorization under this section regarding the emergency use shall, to the extent practicable given the circumstances of the emergency, authorize such person to provide appropriate information with respect to such product in addition to the labeling provided by the manufacturer, subject to

compliance with clause (i). While the authorization under this section is effective, such additional information shall not be considered labeling for purposes of section 502.

(C) The Secretary may establish with respect to the distribution and administration of the product for the unapproved use conditions no more restrictive than those established by the Secretary with respect to the distribution and administration of the product for the approved use.

(3) Good manufacturing practice.--With respect to the emergency use of a product for which an authorization under this section is issued (whether an unapproved product or an unapproved use of an approved product), the Secretary may waive or limit, to the extent appropriate given the circumstances of the emergency, requirements regarding current good manufacturing practice otherwise applicable to the manufacture, processing, packing, or holding of products subject to regulation under this Act, including such requirements established under section 501.

(4) Advertising.--The Secretary may establish conditions on advertisements and other promotional descriptive printed matter that relate to the emergency use of a product for which an authorization under this section is issued (whether an unapproved product or an unapproved use of an approved product), including, as appropriate--

(A) with respect to drugs and biological products, requirements applicable to prescription drugs pursuant to section 502(n); or

(B) with respect to devices, requirements applicable to restricted devices pursuant to section 502(r).

(f) Duration of Authorization.--

(1) In general.--Except as provided in paragraph (2), an authorization under this section shall be effective until the earlier of the termination of the declaration under subsection (b) or a revocation under subsection (g).

(2) Continued use after end of effective period.--Notwithstanding the termination of the declaration under subsection (b) or a revocation under subsection (g), an authorization shall continue to be effective to provide for continued use of an unapproved product with respect to a patient to whom it was administered during the period described by paragraph (1), to the extent found necessary by such patient's attending physician.

(g) Revocation of Authorization.--

(1) Review.--The Secretary shall periodically review the circumstances and the appropriateness of an authorization under this section.

(2) Revocation.--The Secretary may revoke an authorization under this section if the criteria under subsection (c) for issuance of such authorization are no longer met or other circumstances make such revocation appropriate to protect the public health or safety.

(h) Publication; Confidential Information.--

(1) Publication.--The Secretary shall promptly publish in the Federal Register a notice of each authorization, and each termination or revocation of an authorization under this section, and an explanation of the reasons therefore (which may include a summary of data or information that has been submitted to the Secretary in an application under section 505(i) or section 520(g), even if such summary may indirectly reveal the existence of such application).

(2) Confidential information.--Nothing in this section alters or amends section 1905 of title 18, United States Code, or section 552(b)(4) of title 5 of such Code.

(i) Actions Committed to Agency Discretion.--Actions under the authority of this section by the Secretary or by the Secretary of Defense are committed to agency discretion.

(j) Rules of Construction.--The following applies with respect to this section:

(1) Nothing in this section impairs the authority of the President as Commander in Chief of the Armed Forces of the United States under article II, section 2 of the United States Constitution.

(2) Nothing in this section impairs the authority of the Secretary of Defense with respect to the Department of Defense, including the armed forces, under other provisions of Federal law.

(3) Nothing in this section (including any exercise of authority by a manufacturer under subsection (e)(2)) impairs the authority of the United States to use or manage quantities of a product that are owned or controlled by the United States (including quantities in the stockpile maintained under section 319F-2 of the Public Health Service Act).

(k) Relation to Other Provisions.--If a product is the subject of an authorization under this section, the use of such product within the scope of the authorization shall not be considered to constitute a clinical investigation for purposes of section 505(i), section 520(g), or any other provision of this Act or section 351 of the Public Health Service Act.

(l) Option to Carry Out Authorized Activities.--Nothing in this section provides the Secretary any authority to require any person to carry out any activity that becomes lawful pursuant to an authorization under this section, and no person is required to inform the Secretary that the person will not be carrying out such activity, except that a manufacturer of a sole-source unapproved product authorized for emergency use shall report to the Secretary within a reasonable period of time after the issuance by the Secretary of such authorization if such manufacturer does not intend to carry out any

activity under the authorization. This section only has legal effect on a person who carries out an activity for which an authorization under this section is issued. This section does not modify or affect activities carried out pursuant to other provisions of this Act or section 351 of the Public Health Service Act. Nothing in this subsection may be construed as restricting the Secretary from imposing conditions on persons who carry out any activity pursuant to an authorization under this section.