Calendar No. 425

114TH CONGRESS
2D SESSION

S. 185

To create a limited population pathway for approval of certain antibacterial drugs.

IN THE SENATE OF THE UNITED STATES

JANUARY 16, 2015

Mr. HATCH (for himself, Mr. BENNET, Ms. AYOTTE, Mr. ISAKSON, Mr. KIRK, Mr. CARPER, and Mr. BLUMENTHAL) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

APRIL 18, 2016

Reported by Mr. ALEXANDER, with an amendment

[Strike out all after the enacting clause and insert the part printed in italic]

A BILL

To create a limited population pathway for approval of certain antibacterial drugs.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

~~**SECTION 1. SHORT TITLE.**~~

~~This Act may be cited as the "Promise for Antibiotics and Therapeutics for Health Act" or the "PATH Act".~~

SEC. 2. LIMITED POPULATION PATHWAY FOR ANTIBACTERIAL DRUGS.

Section 506 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356) is amended—

(1) by transferring subsection (e) so that it appears before subsection (f); and

(2) by adding at the end the following:

''(g) LIMITED POPULATION PATHWAY FOR ANTIBACTERIAL DRUGS.—

''(1) IN GENERAL.—The Secretary shall establish a program under which the Secretary may, at the request of a sponsor, approve an antibacterial drug, alone or in combination with one or more drugs, as a limited population antibacterial drug, upon a determination that such drug is intended to treat a serious or life-threatening disease, condition, or infection and address an unmet medical need for such disease, condition, or infection within an identifiable limited population.

''(2) LIMITED POPULATION PATHWAY.—

''(A) IN GENERAL.—The sponsor of an antibacterial drug that the Secretary determines to be eligible for approval as a limited population antibacterial drug shall be required to demonstrate the safety and effectiveness of such drug, as required under section 505(d) or

section 351(a) of the Public Health Service Act, for the intended use of the drug. The Secretary shall determine the safety and effectiveness of an antibacterial drug under the limited population pathway for antibacterial drugs in accordance with subparagraph (B). An antibacterial drug shall be eligible for approval under the limited population pathway only upon the request of the sponsor.

"(B) CONSIDERATIONS.—

"(i) BENEFIT-RISK PROFILE.—The Secretary's determination of safety and effectiveness of a limited population antibacterial drug shall reflect the benefit-risk profile of the drug in the intended limited population, taking into account the severity, rarity, or prevalence of the infection the drug is intended to treat and the availability or lack of alternative treatment for such infection. Approval of a drug under the limited population antibacterial drug pathway shall not be denied due to a lack of evidence to fully establish a favorable benefit-risk profile in a population that is

broader than the intended limited population.

"(ii) TYPES OF EVIDENCE.—In determining whether to approve a drug under the limited population pathway, the Secretary—

"(I) shall rely on sufficient evidence, which may include traditional endpoints, alternate endpoints, or a combination of traditional and alternate endpoints, and, as appropriate, small clinical data sets; and

"(II) may rely on supplemental data, including preclinical evidence, pharmacologic or pathophysiologic evidence, nonclinical susceptibility, pharmacokinetic data, and other such confirmatory evidence as the Secretary determines appropriate.

"(3) REQUIREMENTS.—With respect to a drug approved through the limited population pathway, the Secretary shall require—

"(A) the labeling of such antibacterial drug, such as through a logo or other means, to indicate that the drug has been approved for

use only in a limited population and that the safety and efficacy of the drug has been demonstrated only with respect to such limited population; and

"(B) the sponsor to submit copies of all promotional materials related to the limited population antibacterial drug, at least 30 days prior to dissemination of the materials.

"(4) OTHER PROGRAMS.—A sponsor of a drug that seeks approval of a drug through the limited population pathway for antibacterial drugs may also seek approval of such drug under subsections (a), (b), and (c), and sections 505E and 524.

"(5) GUIDANCE.—Not later than 18 months after the date of enactment of the Promise for Antibiotics and Therapeutics for Health Act, the Secretary shall issue draft guidance describing criteria, processes, and other general considerations for demonstrating the safety and effectiveness of limited population antibacterial drugs and how the pathway can be expanded to other therapeutic areas in addition to antibacterial infections. The Secretary may approve antibacterial drugs through such limited population pathway prior to issuing guidance under this paragraph.

''(6) POSTAPPROVAL MONITORING PROGRAMS FOR ANTIBACTERIAL DRUGS.—The Secretary, in consultation with the Commissioner and other relevant heads of agencies, shall conduct postapproval monitoring programs to study how antibacterial drugs approved through the pathway under this subsection are used and to monitor changes in bacterial resistance to drugs, including drugs approved under this pathway.

''(7) ADVICE.—The Secretary shall provide prompt advice to the sponsor of a drug for which the sponsor seeks approval through the limited population pathway for antibacterial drugs to enable the sponsor to plan a development program to obtain the necessary data for approval of such drug through the limited population pathway for antibacterial drugs and to conduct any additional studies that would be required to gain approval of such drug for use in a broader population.

''(8) TERMINATION OF LIMITATIONS.—If, after approval of a drug through the limited population pathway for antibacterial drugs, the Secretary approves a broader indication for such drug for which the sponsor applies under section 505(b) or section 351 of the Public Health Service Act, the Secretary

may remove any postmarketing conditions, including requirements with respect to labeling and review of promotional materials under paragraph (3) and postapproval monitoring under paragraph (6), applicable to the approval of the drug through the limited population pathway for antibacterial drugs.

"(9) RULES OF CONSTRUCTION.—

"(A) STANDARDS OF EVIDENCE AND AUTHORITY OF SECRETARY.—Nothing in this subsection shall be construed to alter the standards of evidence applicable to the review and approval of a drug under this Act or the Public Health Service Act, or to modify or limit the authority of the Secretary to approve or monitor drugs pursuant to this Act or the Public Health Service Act as authorized prior to the date of enactment of the Promise for Antibiotics and Therapeutics for Health Act.

"(B) PRESCRIBING AUTHORITY.—Nothing in this subsection shall be construed to restrict the prescribing of antibiotics or other products, including drugs approved under the limited population pathway, by health care professionals, or to limit the practice of health care.

~~"(10) EXPANSION OF PATHWAY.—Beginning on October 1, 2016, the limited population pathway for antibiotic drugs may be expanded to apply to approval of other drugs intended to treat a serious or life-threatening illness. The approval of such drugs shall be subject to the considerations and requirements described in this subsection, unless the Secretary delivers a report to Congress prior to that date explaining why such pathway should not be used for other therapeutic areas in addition to antibacterial infections.".~~

SECTION 1. SHORT TITLE.

This Act may be cited as the "Promise for Antibiotics and Therapeutics for Health Act" or the "PATH Act".

SEC. 2. ANTIBACTERIAL RESISTANCE MONITORING.

Section 319E of the Public Health Service Act (42 U.S.C. 247d-5) is amended—

(1) by redesignating subsections (f) and (g) as subsections (k) and (l), respectively; and

(2) by inserting after subsection (e), the following:

"(f) MONITORING AT FEDERAL HEALTH CARE FACILITIES.—The Secretary shall encourage reporting on aggregate antibacterial drug use and bacterial resistance to antibacterial drugs and the implementation of antibiotic stew-

ardship programs by health care facilities of the Department of Defense, the Department of Veterans Affairs, and the Indian Health Service and shall provide technical assistance to the Secretary of Defense and the Secretary of Veterans Affairs, as appropriate and upon request.

"(g) REPORT ON ANTIBACTERIAL RESISTANCE IN HUMANS AND USE OF ANTIBACTERIAL DRUGS.—Not later than 1 year after the date of enactment of this subsection, and annually thereafter, the Secretary shall prepare and make publically available data and information concerning—

"(1) aggregate national and regional trends of bacterial resistance in humans to antibacterial drugs, including those approved under section 506(g) of the Federal Food, Drug, and Cosmetic Act;

"(2) antibacterial stewardship, which may include summaries of State efforts to address bacterial resistance in humans to antibacterial drugs and antibacterial stewardship; and

"(3) coordination between the Director of the Centers for Disease Control and Prevention and the Commissioner of Food and Drugs with respect to the monitoring of—

"(A) any applicable resistance under paragraph (1); and

"(B) drugs approved under section 506(g) of the Federal Food, Drug, and Cosmetic Act.

"(h) INFORMATION RELATED TO ANTIBIOTIC STEWARDSHIP PROGRAMS.—The Secretary shall, as appropriate, disseminate guidance, educational materials, or other appropriate materials related to the development and implementation of evidence-based antibiotic stewardship programs or practices at health care facilities, such as nursing homes and other long-term care facilities, ambulatory surgical centers, dialysis centers, and community and rural hospitals.

"(i) SUPPORTING STATE-BASED ACTIVITIES TO COMBAT ANTIBACTERIAL RESISTANCE.—The Secretary shall continue to work with State and local public health departments on statewide or regional programs related to antibacterial resistance. Such efforts may include activities to related to—

"(1) identifying patterns of bacterial resistance in humans to antibacterial drugs;

"(2) preventing the spread of bacterial infections that are resistant to antibacterial drugs; and

"(3) promoting antibiotic stewardship.

"(j) ANTIBACTERIAL RESISTANCE AND STEWARDSHIP ACTIVITIES.—

"(1) IN GENERAL.—For the purposes of supporting stewardship activities, examining changes in bacterial resistance, and evaluating the effectiveness of section 506(g) of the Federal Food, Drug, and Cosmetic Act, the Secretary shall—

"(A) provide a mechanism for facilities to report data related to their antimicrobial stewardship activities (including analyzing the outcomes of such activities); and

"(B) evaluate—

"(i) antimicrobial resistance data using a standardized approach; and

"(ii) trends in the utilization of drugs approved under such section 506(g) with respect to patient populations.

"(2) USE OF SYSTEMS.—The Secretary shall use available systems, including the National Healthcare Safety Network or other systems identified by the Secretary, to fulfill the requirements or conduct activities under this section.

"(3) AVAILABILITY OF DATA.—The Secretary shall make the data collected pursuant to this subsection public. Nothing in this subsection shall be construed as authorizing the Secretary to disclose any information that is a trade secret or confidential infor-

mation subject to section 552(b)(4) of title 5, United States Code, or section 1905 of title 18, United States Code.".

SEC. 3. LIMITED POPULATION PATHWAY FOR ANTIBACTERIAL DRUGS.

Section 506 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356) is amended—

(1) by transferring subsection (e) so that it appears before subsection (f); and

(2) by adding at the end the following:

"(g) LIMITED POPULATION PATHWAY FOR ANTIBACTERIAL DRUGS.—

"(1) IN GENERAL.—The Secretary may approve an antibacterial drug, alone or in combination with one or more other drugs, as a limited population drug pursuant to this subsection only if—

"(A) the drug is intended to treat a serious or life-threatening infection in a limited population of patients with unmet needs;

"(B) the standards for approval under section 505(c) and (d), or the standards for licensure under section 351 of the Public Health Service Act, as applicable, are met; and

"(C) the Secretary receives a written request from the sponsor to approve the drug as a limited population drug pursuant to this subsection.

"(2) BENEFIT-RISK CONSIDERATION.—The Secretary's determination of safety and effectiveness of a limited population antibacterial drug shall reflect the benefit-risk profile of the drug in the intended limited population, taking into account the severity, rarity, or prevalence of the infection the drug is intended to treat and the availability or lack of alternative treatment in such limited population. Such drug may be approved under this subsection notwithstanding a lack of evidence to fully establish a favorable benefit-risk profile in a population that is broader than the intended limited population.

"(3) ADDITIONAL REQUIREMENTS.—A drug approved under this subsection shall be subject to the requirements of this paragraph, in addition to any other applicable requirements of this Act:

"(A) LABELING.—To indicate that the safety and effectiveness of a drug approved under this subsection has been demonstrated only with respect to a limited population—

"(i) all labeling and advertising of an antibacterial drug approved under this sub-

section shall contain the statement 'Limited Population' in a prominent manner and adjacent to, and not more prominent than—

"(I) the proprietary name of such drug, if any; or

"(II) if there is no proprietary name, the established name of the drug, if any, as defined in section 503(e)(3), or for drugs which are biological products, the proper name, as defined by regulation; and

"(ii) the prescribing information for such antibacterial drug required by section 201.57 of title 21, Code of Federal Regulations (or any successor regulation) shall also include the following statement: 'This drug is indicated for use in a limited and specific population of patients.'.

"(B) PROMOTIONAL MATERIAL.—The sponsor of an antibacterial drug subject to this subsection shall submit to the Secretary copies of all promotional materials related to such drug at least 30 calendar days prior to dissemination of the materials.

"(4) OTHER PROGRAMS.—A sponsor of a drug that seeks approval of a drug under this subsection for antibacterial drugs may also seek designation or approval, as applicable, of such drug under other applicable sections or subsections of this Act of the Public Health Service Act.

"(5) GUIDANCE.—Not later than 18 months after the date of enactment of the Promise for Antibiotics and Therapeutics for Health Act, the Secretary shall issue draft guidance describing criteria, processes, and other general considerations for demonstrating the safety and effectiveness of limited population antibacterial drugs. The Secretary shall publish final guidance within 18 months of the close of the public comment period on such draft guidance. The Secretary may approve antibacterial drugs under this subsection prior to issuing guidance under this paragraph.

"(6) ADVICE.—The Secretary shall provide prompt advice to the sponsor of a drug for which the sponsor seeks approval under this subsection for antibacterial drugs to enable the sponsor to plan a development program to obtain the necessary data for approval of such drug under this subsection for antibacterial drugs and to conduct any additional studies

that would be required to gain approval of such drug for use in a broader population.

"(7) TERMINATION OF LIMITATIONS.—If, after approval of a drug under this subsection, the Secretary approves a broader indication for such drug for which the sponsor applies under section 505(b) or section 351(a) of the Public Health Service Act, the Secretary may remove any postmarketing conditions, including requirements with respect to labeling and review of promotional materials under paragraph (3), applicable to the approval of the drug under this subsection.

"(8) RULES OF CONSTRUCTION.—Nothing in this subsection shall be construed to alter the authority of the Secretary to approve drugs pursuant to this Act and section 351 of the Public Health Service Act, including the standards of evidence, and applicable conditions, for approval under such Acts, the standards of approval of a drug under this Act or the Public Health Service Act, or to alter the authority of the Secretary to monitor drugs pursuant to this Act or the Public Health Service Act.

"(9) REPORTING AND ACCOUNTABILITY.—

"(A) BIANNUAL REPORTING.—The Secretary shall report to Congress not less often than once

every 2 years on the number of requests for approval, and the number of approvals, of an antibacterial drug under this subsection.

"(B) GAO REPORT.—Not later than December 2021, the Comptroller General of the United States shall report on the coordination of activities required under section 319E of the Public Health Service Act, a review of such activities, and the extent to which the use of the pathway established under this subsection has streamlined premarket approval for antibacterial drugs for limited populations, if such pathway has functioned as intended, if such pathway has helped provide for safe and effective treatment for patients, if such premarket approval would be appropriate for other categories of drugs, and if the authorities under this subsection have affected antibiotic resistance.".

SEC. 4. PRESCRIBING AUTHORITY.

Nothing in this Act, or an amendment made by this Act, shall be construed to restrict the prescribing of antibacterial drugs or other products, including drugs approved under section 506(g) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356(g)), by health care professionals, or to limit the practice of health care.

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