# AMENDMENT TO THE AMENDMENT IN THE NATURE OF A SUBSTITUTE TO H.R. 7667 OFFERED BY MR. BUTTERFIELD OF NORTH CAROLINA

At the end of subtitle A of title VII, add the following:

**SEC. 713. RESEARCH INTO PEDIATRIC USES OF DRUGS; ADDITIONAL AUTHORITIES OF FOOD AND DRUG ADMINISTRATION REGARDING MOLECULARLY TARGETED CANCER DRUGS.**

(a) IN GENERAL.—

(1) ADDITIONAL ACTIVE INGREDIENT FOR APPLICATION DRUG; LIMITATION REGARDING NOVEL-COMBINATION APPLICATION DRUG.—Section 505B(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(3)) is amended—

(A) by redesignating subparagraphs (B) and (C) as subparagraphs (C) and (D), respectively; and

(B) by striking subparagraph (A) and inserting the following:

''(A) IN GENERAL.—For purposes of paragraph (1)(B), the investigation described in this

paragraph is (as determined by the Secretary) a molecularly targeted pediatric cancer investigation of—

''(i) the drug or biological product for which the application referred to in such paragraph is submitted; or

''(ii) such drug or biological product in combination with—

''(I) an active ingredient of a drug or biological product—

''(aa) for which an approved application under section 505(j) under this Act or under section 351(k) of the Public Health Service Act is in effect; and

''(bb) that is determined by the Secretary to be the standard of care for treating a pediatric cancer; or

''(II) an active ingredient of a drug or biological product—

''(aa) for which an approved application under section 505(b) of this Act or section 351(a) of the Public Health Service Act to

treat an adult cancer is in effect and is held by the same person submitting the application under paragraph (1)(B); and

''(bb) that is directed at a molecular target that the Secretary determines to be substantially relevant to the growth or progression of a pediatric cancer.

''(B) ADDITIONAL REQUIREMENTS.—

''(i) DESIGN OF INVESTIGATION.—A molecularly targeted pediatric cancer investigation referred to in subparagraph (A) shall be designed to yield clinically meaningful pediatric study data that is gathered using appropriate formulations for each age group for which the study is required, regarding dosing, safety, and preliminary efficacy to inform potential pediatric labeling.

''(ii) LIMITATION.—An investigation described in subparagraph (A)(ii) may be required only if the drug or biological product for which the application referred to in paragraph (1)(B) contains either—

"(I) a single new active ingredient; or

"(II) more than one active ingredient, if an application for the combination of active ingredients has not previously been approved but each active ingredient has been previously approved to treat an adult cancer.

"(iii) RESULTS OF ALREADY-COMPLETED PRECLINICAL STUDIES OF APPLICATION DRUG.—The Secretary may require that reports on an investigation required pursuant to paragraph (1)(B) include the results of all preclinical studies on which the decision to conduct such investigation was based.

"(iv) RULE OF CONSTRUCTION REGARDING INACTIVE INGREDIENTS.—With respect to a combination of active ingredients referred to in subparagraph (A)(ii), such subparagraph shall not be construed as addressing the use of inactive ingredients with such combination.".

(2) DETERMINATION OF APPLICABLE REQUIREMENTS.—Section 505B(e)(1) of the Federal Food,

Drug, and Cosmetic Act (21 U.S.C. 355c(e)(1)) is amended by adding at the end the following: ''The Secretary shall determine whether subparagraph (A) or (B) of subsection (a)(1) shall apply with respect to an application before the date on which the applicant is required to submit the initial pediatric study plan under paragraph (2)(A).''.

(3) CLARIFYING APPLICABILITY.—Section 505B(a)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(1)) is amended by adding at the end the following:

''(C) RULE OF CONSTRUCTION.—No application that is subject to the requirements of subparagraph (B) shall be subject to the requirements of subparagraph (A), and no application (or supplement to an application) that is subject to the requirements of subparagraph (A) shall be subject to the requirements of subparagraph (B).''.

(4) CONFORMING AMENDMENTS.—Section 505B(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)) is amended—

(A) in paragraph (3)(C), as redesignated by paragraph (1)(A) of this subsection, by striking ''investigations described in this para-

graph'' and inserting ''investigations referred to in subparagraph (A)''; and

(B) in paragraph (3)(D), as redesignated by paragraph (1)(A) of this subsection, by striking ''the assessments under paragraph (2)(B)'' and inserting ''the assessments required under paragraph (1)(A)''.

(b) GUIDANCE.—The Secretary shall—

(1) not later than 6 months after the date of enactment of this Act, issue draft guidance on the implementation of the requirements in subsection (a); and

(2) not later than 12 months after closing the comment period on such draft guidance, finalize such guidance.

(c) APPLICABILITY.—The amendments made by this section apply with respect to any application under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) and any application under section 351(a) of the Public Health Service Act (42 U.S.C. 262), that is submitted on or after the date that is 3 years after the date of enactment of this Act.

(d) REPORTS TO CONGRESS.—

(1) SECRETARY OF HEALTH AND HUMAN SERVICES.—Not later than 2 years after the date of en-

actment of this Act, the Secretary of Health and Human Services shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a report on the Secretary's efforts, in coordination with industry, to ensure implementation of the amendments made by subsection (a).

(2) GAO STUDY AND REPORT.—

(A) STUDY.—Not later than 2 years after the date of enactment of this Act, the Comptroller General of the United States shall conduct a study of the effectiveness of requiring assessments and investigations described in section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C.355c), as amended by subsection (a), in the development of drugs and biological products for pediatric cancer indications.

(B) FINDINGS.—Not later than 4 years after the date of enactment of this Act, the Comptroller General shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the

Senate a report containing the findings of the study conducted under subparagraph (A).