

## 21 USC §355c. Research into pediatric uses for drugs and biological products

### (a) New drugs and biological products

#### (1) In general

##### (A) General requirements

Except with respect to an application for which subparagraph (B) applies, a person that submits, on or after September 27, 2007, an application (or supplement to an application) for a drug-

(i) under [section 355 of this title](#) for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration; or

(ii) under [section 262 of title 42](#) for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration, shall submit with the application the assessments described in paragraph (2).

##### (B) Certain molecularly targeted cancer indications

A person that submits, on or after the date that is 3 years after August 18, 2017, an original application for a new active ingredient under [section 355 of this title](#) or [section 262 of title 42](#), shall submit with the application reports on the investigation described in paragraph (3) if the drug or biological product that is the subject of the application is-

(i) intended for the treatment of an adult cancer; and

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

**(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).

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#### (3) Molecularly targeted pediatric cancer investigation

##### (A) In general

~~With respect to a drug or biological product described in paragraph (1)(B), the investigation described in this paragraph is a molecularly targeted pediatric cancer investigation, which shall be designed to yield clinically meaningful pediatric study data, 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.~~

**(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.

**(B-C) Extrapolation of data**

Paragraph (2)(B) shall apply to investigations described in this paragraph to the same extent and in the same manner as paragraph (2)(B) applies with respect to the assessments required under paragraph (1)(A).

**(C-D) Deferrals and waivers**

Deferrals and waivers under paragraphs (4) and (5) shall apply to investigations described in this paragraph to the same extent and in the same manner as such deferrals and waivers apply with respect to the assessments under paragraph (2)(B).