

Bob Casey

AMENDMENT NO. _____ Calendar No. _____

Purpose: To improve the treatment of rare diseases and conditions.

IN THE SENATE OF THE UNITED STATES—117th Cong., 2d Sess.

S. 4348

To amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user-fee programs for prescription drugs, medical devices, generic drugs, and biosimilar biological products, and for other purposes.

Referred to the Committee on _____ and
ordered to be printed

Ordered to lie on the table and to be printed

AMENDMENT intended to be proposed by Mr. CASEY

Viz:

1 At the end of section 508, insert the following:

2 (d) REVIEW PROCESS.—

3 (1) CONSULTATION WITH STAKEHOLDERS.—

4 Section 569(a)(1) of the Federal Food, Drug, and

5 Cosmetic Act (21 U.S.C. 360bbb-8(a)(1)) is amend-

6 ed—

7 (A) by striking “at a time” and inserting

8 “at any time”;

9 (B) by striking “Consistent with sections”

10 and inserting the following:

1 “(A) IN GENERAL.—Consistent with sec-
2 tions”; and

3 (C) by adding at the end the following:

4 “(B) CONSULTATION WITH PATIENTS AND
5 PATIENT GROUPS.—

6 “(i) IN GENERAL.—The Secretary
7 may, as appropriate, consult with patients
8 and relevant patient groups impacted by
9 the rare disease or condition, together with
10 at least one expert included on the list
11 under paragraph (2)(A) and selected by
12 such groups, as applicable, during meet-
13 ings between the Food and Drug Adminis-
14 tration and sponsors prior to the submis-
15 sion of an application for a new drug or bi-
16 ological product for a rare disease or con-
17 dition or a drug or biological product that
18 is genetically targeted.

19 “(ii) CONFLICTS OF INTEREST.—For
20 purposes of clause (i), to be eligible for
21 consultation pursuant to clause (i), pa-
22 tients and relevant patient groups may not
23 have any financial interest in the applica-
24 ble drug or biological product, and external
25 experts shall be in compliance with applica-

1 ble law, including section 208 of title 18,
2 United States Code.

3 “(C) CONSULTATION WITH DISPROPOR-
4 TIONATELY AFFECTED COMMUNITIES.—To the
5 extent an application for a new drug or biologi-
6 cal product relates to a rare disease or condi-
7 tion that disproportionately affects communities
8 of color or other historically underrepresented
9 and vulnerable populations, the Secretary is en-
10 couraged to consult with patients of that sub-
11 population, or one or more patient groups that
12 represent that subpopulation.”.

13 (2) REQUIRING APPROPRIATE EXPERT CON-
14 SULTATION.—Section 569(a)(2) of the Federal
15 Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb-
16 8(a)(2)) is amended—

17 (A) in subparagraph (A), by striking the
18 second sentence; and

19 (B) by striking subparagraph (B) and in-
20 serting the following:

21 “(B) CONSULTATION.—With respect to
22 any application under section 505 of this Act or
23 section 351 of the Public Health Service Act for
24 a drug designated under section 526 for a rare
25 disease or condition or a drug or biological

1 product that is genetically targeted, the Sec-
2 retary may, as appropriate, consult—

3 “(i) with an expert with respect to the
4 disease or condition referenced in the ap-
5 plication who appears on the list described
6 in subparagraph (A); or

7 “(ii) if no such expert is available, in-
8 cluding because of conflicts of interest,
9 with an expert on the list described in sub-
10 paragraph (A) in the science of small pop-
11 ulation studies.

12 “(C) AVAILABILITY AT MEETINGS.—In
13 connection with each drug product advisory
14 committee meeting concerning a drug or bio-
15 logical product for a rare disease or condition,
16 the Secretary may, as appropriate—

17 “(i) include—

18 “(I) an expert in the rare disease
19 or condition; or

20 “(II) if no such expert is avail-
21 able, including because of conflicts of
22 interest, an expert in the science of
23 small population studies; and

24 “(ii) invite at least one disease or con-
25 dition expert identified by the relevant pa-

1 tient groups to participate as a nonvoting
2 member of the advisory committee.”.

3 (3) ADDITIONAL TOPIC FOR CONSULTATION.—

4 Section 569(b) of the Federal Food, Drug, and Cos-
5 metic Act (21 U.S.C. 360bbb–8(b)) is amended—

6 (A) in paragraph (6), by striking “; and”
7 and inserting “;”;

8 (B) in paragraph (7), by striking the pe-
9 riod and inserting “; and”; and

10 (C) by adding at the end the following:

11 “(8) the science of small population studies.”.