AMENDMENT NO.________ Calendar No.____

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


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:

   (d) REVIEW PROCESS.—

2. (1) CONSULTATION WITH STAKEHOLDERS.—


4. (A) by striking “at a time” and inserting “at any time”;

5. (B) by striking “Consistent with sections” and inserting the following:
“(A) IN GENERAL.—Consistent with sections”; and

(C) by adding at the end the following:

“(B) CONSULTATION WITH PATIENTS AND

PATIENT GROUPS.—

“(i) IN GENERAL.—The Secretary may, as appropriate, consult with patients and relevant patient groups impacted by the rare disease or condition, together with at least one expert included on the list under paragraph (2)(A) and selected by such groups, as applicable, during meetings between the Food and Drug Administration and sponsors prior to the submission of an application for a new drug or biological product for a rare disease or condition or a drug or biological product that is genetically targeted.

“(ii) CONFLICTS OF INTEREST.—For purposes of clause (i), to be eligible for consultation pursuant to clause (i), patients and relevant patient groups may not have any financial interest in the applicable drug or biological product, and external experts shall be in compliance with applica-
ble law, including section 208 of title 18,
United States Code.

“(C) Consultation with disproportionately affected communities.—To the extent an application for a new drug or biological product relates to a rare disease or condition that disproportionately affects communities of color or other historically underrepresented and vulnerable populations, the Secretary is encouraged to consult with patients of that subpopulation, or one or more patient groups that represent that subpopulation.”.

(2) Requiring appropriate expert consultation.—Section 569(a)(2) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–8(a)(2)) is amended—

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

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

“(B) Consultation.—With respect to any application under section 505 of this Act or section 351 of the Public Health Service Act for a drug designated under section 526 for a rare disease or condition or a drug or biological
product that is genetically targeted, the Secretary may, as appropriate, consult—

“(i) with an expert with respect to the disease or condition referenced in the application who appears on the list described in subparagraph (A); or

“(ii) if no such expert is available, including because of conflicts of interest, with an expert on the list described in subparagraph (A) in the science of small population studies.

“(C) AVAILABILITY AT MEETINGS.—In connection with each drug product advisory committee meeting concerning a drug or biological product for a rare disease or condition, the Secretary may, as appropriate—

“(i) include—

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

“(II) if no such expert is available, including because of conflicts of interest, an expert in the science of small population studies; and

“(ii) invite at least one disease or condition expert identified by the relevant pa-
tient groups to participate as a nonvoting
member of the advisory committee.”.

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

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

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

(C) by adding at the end the following:

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