AMENDMENT TO THE AMENDMENT IN THE NATURE OF A SUBSTITUTE TO H.R. 3433

OFFERED BY M__. _____

At the end of section 5, add the following (and make such conforming changes as may be necessary):

(b) GAO REPORT ON EFFECTIVENESS OF RARE PEDIATRIC DISEASE PRIORITY VOUCHER AWARDS IN INCENTIVIZING RARE PEDIATRIC DISEASE DRUG DEVELOPMENT.—

(1) GAO STUDY.—

(A) STUDY.—The Comptroller General of the United States shall conduct a study of the effectiveness of awarding rare pediatric disease priority vouchers under section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff), as amended by subsection (a), in the development of human drug products that treat or prevent rare pediatric diseases (as defined in such section 529).

(B) CONTENTS OF STUDY.—In conducting the study under subparagraph (A), the Comptroller General shall examine the following:

(i) The indications for each drug or biological product that—

(I) is the subject of a rare pediatric disease product application (as defined in section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff)) for which a priority review voucher was awarded; and

(II) was approved under section 505 of the Federal Food, Drug, and Cosmetic Act (42 U.S.C. 355) or licensed under section 351 of the Public Health Service Act (42 U.S.C. 262).

(ii) Whether, and to what extent, an unmet need related to the treatment or prevention of a rare pediatric disease was met through the approval or licensure of such a drug or biological product.

(iii) The size of the company to which a priority review voucher was awarded under section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff) for such a drug or biological product.

(iv) The value of such priority review voucher if transferred.

(v) Identification of each drug for which a priority review voucher awarded under such section 529 was used.

(vi) The size of the company using each priority review voucher awarded under such section 529.

(vii) The length of the period of time between the date on which a priority review voucher was awarded under such section 529 and the date on which it was used.

(viii) Whether, and to what extent, an unmet need related to the treatment or prevention of a rare pediatric disease was met through the approval under section 505 of the Federal Food, Drug, and Cosmetic Act (42 U.S.C. 355) or licensure under section 351 of the Public Health Service Act (42 U.S.C. 262) of a drug for which a priority review voucher was used.

(ix) Whether, and to what extent, companies were motivated by the availability of priority review vouchers under

section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff) to attempt to develop a drug for a rare pediatric disease.

(x) Whether, and to what extent, pediatric review vouchers awarded under such section were successful in stimulating development and expedited patient access to drug products for treatment or prevention of a rare pediatric disease that wouldn't otherwise take place without the incentive provided by such vouchers.

(xi) The impact of such priority review vouchers on the workload, review process, and public health prioritization efforts of the Food and Drug Administration.

(xii) Any other incentives in Federal law that exist for companies developing drugs or biological products described in clause (i).

(2) REPORT ON FINDINGS.—Not later than 5 years after the date of the enactment of this Act, the Comptroller General of the United States 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 paragraph (1).