Basics of FDAAA
Section 1102---Priority Review Vouchers to Encourage Tropical Disease Medicines

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Introduction

- FDA Amendments Act (FDAAA) enacted on September 27, 2007
- FDAAA Section 1102 adds a new Section 524 to the FDCA
- Section 524 authorizes FDA to award priority review vouchers to encourage development of treatments for tropical diseases

How Does This Provision Work?

- Sponsor that gets approval for a drug to treat or prevent a listed tropical disease may get a “priority review voucher” that entitles them to a priority review for a subsequent 505(b)(1) or section 351 PHS Act application (Note: the voucher is not for any other application (e.g., 505b2 or 505j are not eligible).
- Voucher can be transferred (e.g., sold, bartered) to someone else
- Tropical disease product application must be for New Chemical Entity submitted and approved after FDAAA enactment
- Voucher not issued until 1 year from FDAAA enactment
- Sponsor must notify FDA of intent to use voucher and the date on which the sponsor intends to submit the application not later than 1 year before use
- FDA to establish and collect extra user fees based on average cost of priority reviews in previous fiscal year

Overview

- How does a sponsor get a priority review voucher?
- How does a sponsor use a voucher?

How does a sponsor get a priority review voucher?

- FDA will award a priority review voucher to the sponsor of a tropical disease product application upon approval of such tropical disease product application

What is a Tropical Disease Product Application?

- The term ‘tropical disease product application’ means an application that—
  - (A) is a human drug application as defined in section 735(1)—
    - (i) for prevention or treatment of a **tropical disease**; and
    - (ii) the Secretary deems eligible for **priority review**;
  - (B) is approved after the date of the enactment of the Food and Drug Administration Amendments Act of 2007, by the Secretary for use in the prevention or treatment of a tropical disease; and
  - (C) is for a human drug, no active ingredient (including any ester or salt of the active ingredient) of which has been approved in any other application under section 505(b)(1) or section 351 of the Public Health Service Act.

Which Diseases Qualify?

(A) Tuberculosis.
(B) Malaria.
(C) Blinding trachoma.
(D) Buruli Ulcer.
(E) Cholera.
(F) Dengue/
Dengue haemorrhagic fever.
(G) Dracunculiasis
(guinea-worm disease).
(H) Fascioliasis.
(I) Human African
trypanosomiasis.
(J) Leishmaniasis.
(K) Leprosy.
(L) Lymphatic filariasis.
(M) Onchocerciasis.
(N) Schistosomiasis.
(O) Soil transmitted helmithiasis.
(P) Yaws.
(Q) Any other infectious disease for which there is no significant market in developed nations and that disproportionately affects poor and marginalized populations, designated by regulation by the Secretary

Under What Conditions Would FDA Normally Designate a "Priority Review"?

An application for a drug product will receive a priority review if FDA determines that the product, if approved, would be a significant improvement, compared to marketed products, including non-drug products/therapies in the treatment, diagnosis, or prevention of a disease. See CDER's MAPP 6020.3

What is a “Human Drug Application”?

- The term "human drug application" means an application for approval of a new drug submitted under section 505(b) of the Federal Food, Drug, and Cosmetic Act (the Act) or licensure of a biologic product under section 351 of the Public Health Service Act.

- There are several exceptions - for example, applications for whole blood products or allergic extract products are not considered human drug applications.

- Please refer to section 735(1) of the Act for details.

What Are the Limitations?

Limitations

(A) No Award for Previously Submitted Applications

Sponsor may not receive a priority review voucher if the tropical disease product application was submitted prior to the date of FDAAA enactment

(B) One-Year Waiting Period

FDA will issue a priority review voucher to the sponsor of a tropical disease product no earlier than the date that is 1 year after the date of FDAAA enactment

How does a sponsor use a voucher?

- Sponsor that gets approval for a drug to treat or prevent a listed tropical disease and meets all other criteria would get a "priority review voucher" that entitles them to a priority review for any other application

What is a Priority Review?

- PRIORITY REVIEW.—The term ‘priority review’, with respect to a human drug application as defined in section 735(1), means review and act on 90% of the applications no later than 6 months after receipt, as described in the Manual of Policies and Procedures of the Food and Drug Administration and goals identified in the letters described in section 101(c) of the Food and Drug Administration Amendments Act of 2007.

Priority Review V*oucher*

- PRIORITY REVIEW VOUCHER.—The term ‘priority review voucher’ means a voucher issued by the Secretary to the sponsor of a tropical disease product application that entitles the holder of such voucher to priority review of a single human drug application submitted under section 505(b)(1) or section 351 of the Public Health Service Act after the date of approval of the tropical disease product application. (Section 524(a)(2) of the FDC Act).

Can a Voucher be Transferred to Another Sponsor?

- Transferability of Voucher: Sponsor that receives a priority review voucher under Section 1102 may transfer (including sell) the entitlement to such voucher to a sponsor of a human drug for which an application, under section 505(b)(1) or section 351 of the Public Health Service Act, will be submitted after the date of the approval of the tropical disease product application.

Using a Priority Review Voucher

Notification

- Sponsor must notify FDA of an intent to submit the human drug application with a priority review voucher
  - Notify FDA at least one year prior to submission of the human drug application that is the subject of a priority review voucher
  - Notification must include the date on which the sponsor intends to submit the application.
- Such notification shall be a legally binding commitment to pay for the “priority review user fee”

Priority Review Voucher User Fee

- Sponsor of a human drug application that is the subject of a priority review voucher shall pay FDA a priority-review voucher user fee in addition to any other user fee required to be submitted by the sponsor under PDUFA.
  - Amount of priority review user fee determined each fiscal year (FY) based on average cost incurred by FDA in the review of a human drug application subject to priority review in the previous fiscal year.
  - FDA shall establish the fee amount before the beginning of each FY
  - Payment: priority review user fee is due upon submission of the application for which the priority review voucher is used
    - The application will be considered incomplete if the priority fee and all other applicable user fees are not paid in accordance with FDA payment procedures
  - No waivers, exemptions, reductions or refunds

Frequently Asked Questions

Question: ***How do I find out if a certain disease will qualify as a tropical disease under Section 1102?***

The Agency encourages early interaction with potential sponsors and this should be included in early discussions with the appropriate review division. Such interactions could be done in the pre-IND phase

Question: ***Can the FDA provide a determination that an application will be eligible for a voucher prior to the point at which an application is approved for marketing (E.g., prior to NDA/BLA submission or during review of the application)?***

No. Such a determination cannot be made until the time at which the application is approved. A product that meets the criteria at the time of submission may not meet those same criteria by the time of the approval action and would thus not receive a priority review voucher.

This could occur if another product containing the same active ingredient is approved while that application is still under review.

Early communication with the Agency (e.g., pre-IND meetings, pre-NDA/BLA meetings) should provide the Sponsor with an understanding of the criteria that must be met at the time of approval.

Question: ***Are combination products eligible for priority vouchers?***

It depends. Combination products are eligible if the product, including all active ingredients, meets the criteria established in FDAAA. If, however, the product contains any active ingredient that has been previously approved, the application is not eligible for a priority voucher (see section 524(a)(4)(C) of the Act).

Question: ***If a sponsor submits a human drug application without a voucher, and FDA determines that the application is <u>not</u> a priority, what is the expected timeframe for review?***

If a human drug application is submitted without a voucher and does receive an FDA priority designation, the application will receive a "standard" review.

Under the goals referenced in FDAAA section 101(c), FDA commits to review and act on 90% of "standard" applications within 10 months of the date of receipt.

Question: ***Does this provision of FDAAA apply to Orphan Drug development?***

A drug that qualifies for a tropical disease product application may also qualify for incentives under provisions of the Orphan Drug Act.

Potential sponsors should contact: FDA Office of Orphan Products Development

More Information

- A more comprehensive set of Q and As will be posted to the FDA website Spring 2008
- FDA expects to issue guidance on Section 1102
- FDA anticipates publishing the priority user fee amount in the Federal Register along with the publication of other drug user fees for the coming fiscal year. The FR notice usually publishes in August.
- http://www.fda.gov/oc/initiatives/fdaaa/PL110-85.pdf