The EFFECTIVENESS OF THE FDA PRIORITY REVIEW VOUCHER FOR NEGLECTED TROPICAL DISEASES AND RARE PEDIATRIC DISEASES

Wang A², Chowdhury C, MSc²
CBPartners, New York, NY, USA

Introduction

In the past, a lack of economic incentive for companies to develop new therapies for rare tropical and rare pediatric diseases led to a general paucity of innovation and subsequent commercialization within these areas of high unmet need. Between 1975 and 1985, less than 15 of new therapies for tropical diseases were launched. Between 1985 and 1995, this increased to 20 new therapies. However, after 1995, the number of new therapies approved dropped significantly and has, as of 2017, remained at approximate levels of 10 new tropical disease therapies per year. This decline can be partially attributed to the lack of incentive for industry to pursue the development of new treatments for tropical diseases. In 2006, the World Health Organization (WHO) estimated that seven of the world’s 20 most neglected diseases were tropical diseases. The isolation and devastation caused by these diseases, as well as spending among the world’s least able to pay for treatment, The Food and Drug Administration (FDA) created the Priority Review Voucher (PRV) program in 2007 to incentivize industry to develop new treatment options for these underserved disease areas. In 2017, the FDA decided to expand the PRV program to include rare pediatric diseases as well.

Companies who discover and develop a new therapy that successfully receives FDA approval for one of the eligible diseases are granted a PRV. The PRVs can be used to fast-track an additional therapy’s development for a different disease area, or can be sold to a different manufacturer for another development program, or sold to a different manufacturer for another drug in development. When applied to a New Drug Application (NDA) or Biologics License Application (BLA), the PRV is expected to expedite the FDA review process from 10 months to 6 months.

Objective

This study aimed to identify the incentives of the Priority Review Voucher program and the effectiveness of the program to encourage the development of new treatments.

Methods

A pragmatic literature review of research papers and government resources was completed to determine which diseases experienced an increase in treatment developments. Additionally, disease guidelines were analyzed to understand the effect of the new developments on treatment algorithms.

Results

- Of the newly approved therapies, four were indicated for a rare tropical disease and 10 received rare pediatric disease indications. 
- There has been some controversy surrounding treatments that are approved in other markets for the tropical disease arm; however, four rare tropical diseases (malaria, MDR-TB, leishmaniasis, and visceral leishmaniasis) now have at least one FDA-approved treatment option.
- The tropical disease arm of the PRV program has not experienced as much development in comparison to the pediatric disease arm; however, four rare tropical diseases (malaria, MDR-TB, leishmaniasis, and visceral leishmaniasis) now have at least one FDA-approved treatment option.
- Companies who discover and develop a new therapy that successfully receives FDA approval for one of the eligible diseases are granted a PRV.
- The FDA expressed concern that the PRV program affects the agency’s ability to prioritize public health needs, as well as being among the world’s least able to pay for treatment. The Food and Drug Administration (FDA) created the Priority Review Voucher (PRV) program in 2007 to incentivize industry to develop new treatment options for these underserved disease areas. In 2017, the FDA decided to expand the PRV program to include rare pediatric diseases as well.