Introduction

At the time of this analysis, 14 new therapies across 13 disease areas were approved by the FDA and Of the newly approved therapies, four were indicated for a rare tropical disease and 10 received rare The introduction of VAXCHORATM provides the only cholera prevention vaccine for USA travelers More PRVs have been awarded for the development of rare pediatric diseases compared to rare tropical There has been some controversy surrounding treatments that are approved in other markets for the The FDA is one of the main critics of the PRV program; their primary concern is the strain that is placed on Under the 21st Century Cures initiative, the rare pediatric program was re-authorized and extended through In addition to the "novel" requirement that is applied across the PRV program, the FDA requires the issue the PRV if the product is not under patent protection is a point of dispute. The FDA expressed concern that the PRV program affects the agency's ability to prioritize public health

Objective

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

Results

Overview

- At the time of this analysis, 14 new therapies across 12 disease areas were approved by the FDA and received a Priority Review Voucher (PRV) (FIGURE 1)
- Of the newly approved therapies, four were indicated for a rare tropical disease and 10 received rare pediatric indications
- In order to be eligible for a PRV, NDAs/BAs must meet the pre-specified disease requirements and cannot be FDA-approved as orphan drugs (FIGURE 2)
- There has been some controversy surrounding treatments that are approved in other markets for the rare pediatric condition, but we note that this is a PRV under the criteria that is a "novel" in the USA

PRV INCENTIVE FOR RAREDISEASE-INNOVATION

1. FDA Priority Review Voucher Timeline

2. FDA Priority Review Voucher Requirements

RARE TROPICAL DISEASES

- The tropical disease arm of the PRV program has not experienced as much development in comparison to the pediatric disease arm. The introduction of VAXCHORATM and cholera now have at least one FDA-approved treatment option
- Based on CDC publication, VAXCHORATM and cholera (intranasal) are recommended as a first-line treatment option for V. Cholera infections in unvaccinated
- While M. lepra, L. (V.) braziliensis, L. (V.) panamensis, and L. (V.) guyanensis can be important in the USA
- The introduction of SIRTUROTM was a crucial addition for patients who are resistant to the two first-line treatment options for tuberculosis

FIGURE 1: FDA Priority Review Voucher Timeline

FIGURE 2: FDA Priority Review Voucher Requirements

Characteristics of Drug Applications for PRV Designation

- Eligibility with a potential to address unmet needs in pediatric or adult populations
- Eligibility with a potential to address unmet needs in at least 200,000 patients
- Eligibility with a potential to impact public health because it is an "orphan disease" or has an "orphan status" in 10 of the last 20 years

PRV INCENTIVE FOR RAREDISEASE-INNOVATION

PRV, ORPHAN DISEASE DRUGS APPROVALS

CONCLUSIONS

To date, the PRV program has rewarded the development of 14 new drugs - four for rare tropical diseases and 10 for rare pediatric diseases. The PRV program was initiated in 2007 and that, in many cases, over ten years were required to develop a new therapy. In the past, a perceived lack of economic incentive for companies to discover and develop new therapies for rare tropical and rare pediatric diseases led to a general paucity of innovation and subsequent commercialization of these areas with high unmet need. Between 1975 and 1989, less than 15% of new approved drugs were indicated for rare diseases based on the FAO criteria. From 1990 to 2015, the percentage of newly approved drugs indicated for rare diseases increased to 25% of all new approved drugs, as well as being among the world's lowest rate for development. The Food and Drug Administration (FDA) estimated that, in the last 10 years, the voucher was granted to 10 additional treatment options for these unmet disease areas in 2016. The FDA decided to expand the PRV program to include the rare tropical disease arm

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FIGURE 3: Orphan Disease Drug Approvals and Priority Review Vouchers Over Time