Introduction

- Time to approval for new medicines is under constant scrutiny, by patients seeking quicker approval.
- More flexible regulatory approaches have been formalized in ICH jurisdictions, providing options to accelerate the regulatory review process, particularly in response to unmet medical needs.
- CIRS carries out an annual study to monitor regulatory performance to identify the influence of factors such as the use of expedited pathways on new medicines’ approval time.

Definitions

- Expedited (NASs) are defined as: serious conditions, orphan designations, serious or life-threatening conditions and rare diseases.
- New active substance (NAS): A chemical, biological, biotechnology or radiopharmaceutical substance that has not been previously available for therapeutic use in humans and is destined to be made available as a prescription only medicine, to be used for the cure, alleviation, treatment, prevention or in vivo diagnosis of diseases in humans.
- Approval time: Time calculated from the date of submission to the date of approval by the agency. This time includes agency and company time.

Objectives

To review approvals of new active substances (NASs) by US FDA, EU EMA, and Japanese PMDA between 2005-2014 in order to evaluate the use by companies of the different review pathways and special designations available and the role they play in expediting the approval of new medicines.

Methodology

- Data for 825 NASs approved by ICH countries in 2005-2014 were collected from the public domain and for each NAS, the submission and approval dates, expedited pathways and orphan designations were identified to characterise the relationship between approval time and review type.

Results

- Figure 1a: NAS approval numbers achieved a record high in 2014, PMDA approved the greatest number of NASs.
- Figure 1b: NAS median approval times converged within ICH between 2005-2014; FDA was the fastest in 2014.
- Figure 2a: FDA and PMDA expedited approvals were much faster compared to EMA.
- Figure 2b: Expedited review median approval times were similar across the ICH agencies in 2014.

Conclusions

- Expedited pathways and special designations played an important role in FDA and PMDA in accelerating the approval of innovative medicines over the last decade, thereby enabling treatments for diseases with little or no effective medicines, including orphan diseases, to be made available to patients in a timely manner.
- The proportion of expedited reviews was consistent for both parts of the decade for FDA, but PMDA doubled its number of expedited NASs during this time. This relates mainly to a change in process at PMDA, enabling the use of an expedited process for new medicines to meet the needs of Japanese patients.
- However, although Japan historically had the longest review times, the median regulatory approval times in Europe were the slowest amongst the three countries. This is partly due to a lack of wide use of expedited pathways.
- Although the number of EMA expedited NASs increased only slightly in 2010-2014 compared with 2005-2009, 2010-2014 expedited approval times were considerably faster compared with standard and in 2014 were very similar to expedited speed at both FDA and PMDA.
- The scarce use of expedited pathways in the EU suggests that either the criteria for an accelerated approval are much stricter for EMA than FDA or PMDA or that certain aspects of the process limit its use by companies or its designation by the agency.
- Indeed a perception survey carried out by Liberti and colleagues showed that 87% of respondents believe that the EMA pathway either does not meet the goal or needs improvements to expedite reviews of important new medicines.
- Based on the role expedited reviews play in both USA and Japan, wider use of the EMA accelerated pathway or the creation of new facilitated routes may be needed to ensure the timely availability of important new medicines, including orphan NASs, to patients in Europe.

Bibliography


Data Source

CIRS Regulatory Review Times database

Survey

Explore a database of scientific evidence relating to regulatory science with the purpose of advancing regulatory science and policy and in promoting decision-making in health and pharmacoeconomic areas.

Contact

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