Macedonian pharmaceutical bulletin, 68 (Suppl 1) 207 - 208 (2022)
Online ISSN 1857 - 8969
*DOI:*10.33320/maced.pharm.bull.2022.68.03.099
Short communication

Regulatory Approaches of EMA and FDA for accelerated approval of marketing-authorisation application

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Introduction

The development of a new medicine is a long-term and complex process, covering a period from 10 to 15 years. It includes a large number of research studies for discovery of new active substances, pharmaceutical development studies, preclinical testing and clinical trials for confirming safety and efficacy, as well as thorough analytical testing for confirming the quality of the medicine. The obtained data are used for generation of comprehensive documentation that is submitted to the health regulatory authorities in the procedure for obtaining marketing authorization. The process of evaluation of the submitted documentation aimed for assessing the positive benefit/risk profile, may cover a period of several months, and may delay the timely access of the medicine to the patients (Johnson et al., 2011).

Taking into account the above mentioned, the regulatory authorities in the European Union (EU) and the United States of America (USA) have developed different mechanisms to accelerate the development of medicines and the process of approval of medicines, intended for the treatment of serious diseases, medicines with unmet medical needs as well as medicines that represent innovation medicines (obtained with the most current technology based on molecular genetics and immunology) and are of major interest to public health (Warreth, 2020).

The aim of this study was to conduct comparative analysis of the regulatory approaches applied for the accelerated procedures for marketing authorization, implemented by European Medicines Agency (EMA) and Food and drug administration (FDA).

Regulatory approaches of EMA for acceleration of the development of medicines and the process of approval of medicines

Pharmaceutical legislation in EU defines several approaches aimed to support early access to new medicines, which are qualified to apply for the centralized procedure, in order to meet public health medical needs:
Prime, as a support scheme and *Accelerated assessment* as a regulatory tool, are aimed for accelerating the development/assessment of new medicines representing therapeutic innovation and are of a major interest form the public health point of view. *Prime* is designed to enable early scientific and regulatory support for the development of new medicines that have a potential for fulfilment of the unmet medical need. The support is based on reinforced interaction and early dialogue between the EMA and the manufacturers, in order to optimize the development plans and to shorten the timeframe needed for evaluation of the newly developed medicines, enabling timely access of the medicines to the patients.
Accelerated assessment reduces the time for evaluation of the marketing authorisation application from 210 days (standard procedure) to 150 days. Applicants need to apply for this procedure 6-7 months before marketing authorisation application.
Conditional marketing authorization is a regulatory tool for early access of the medicines that are intended to cure the debilitating or life-threatening diseases, including orphan medicines and medicines for emergency situations. The conditional marketing authorization is granted for a period of one year the applicant is committed to fulfil all of the obligations in the defined timeframes.

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Compassionate use is a regulatory tool for early access to the unauthorised medicines for treatment of chronically, serious or life threatening diseases, without satisfactory treatment authorised in EU. Application for authorization for compassionate use cannot be submitted by the applicants directly to EMA. It should be forwarded by the national competent authorities (Council of Europe, 2004, European Medicines Agency, 2022).

Regulatory approaches of FDA for acceleration of the development of medicines and the process of approval of medicines

The Food and Drug Administration (FDA) of the United States has developed several mechanisms to accelerate development and approval of medicines, including:

Priority Review is a procedure for designation of the application for an authorization of a medicine aimed to treat serious condition and has a potential to provide a significant improvement in safety or effectiveness. The timeframe for review and evaluation of the submitted documentation is shorter (6 months) compared to standard review (10 months).

Breakthrough Therapy represents designation of a medicine that is intended, alone or in combination with one or more other medicines, to treat a serious or life-threatening disease or condition, providing that preliminary clinical evidence indicates that the medicine has a potential for significant improvement compared to existing therapies. The clinical evidence needed to support breakthrough designation is preliminary (based on one or more clinically significant endpoints).

Accelerated Approval is a procedure for accelerated assessment of the submitted documentation, which can be applied for a medicine intended to treat a serious or life-threatening disease or condition. Assessment is based on a determination that the product has an effect on a surrogate or intermediate endpoint that is reasonably likely to predict clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

Fast Track is intended to facilitate development and accelerate review of medicines for treatment of the serious and life-threatening conditions or has the potential to address unmet medical needs (Congress of USA, 2009, Food and Drug Administration, 2014).

Comparative analysis of the accelerated procedures in EU and USA

The results from comparative analysis, indicate on significant differences in the processes of marketing authorization, requirements for qualification, as well as characteristics (method of administration, dosage form, strength, dosage and indications) of the pharmaceutical products, which are subject of accelerated approval programs of regulatory agencies in the US and the EU. The differences in the procedures for accelerated approval, partially reflect the different regulatory frameworks, policies, and the markets of the pharmaceutical sector (Alqahtani et al., 2015).

Conclusion

The various tools for accelerated marketing authorization of a medicine support the process of evaluation of the documentation submitted in the marketing authorization process and reduce the approval time. Enhanced regulatory support provides higher degree of success of applications for marketing authorization.

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