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Cystic Fibrosis Research News

Title:

Differential times of submission and approval of CFTR modulators for the treatment of cystic fibrosis in the United States and the European Union

Lay Title:

Differences between the United States and the European Union in the time of approval of CFTR modulators for the treatment of cystic fibrosis

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What was your research question?

The goal of this study was to analyze the differences in the time of approval of CFTR modulators between the United States (US) and the European Union (EU).

Why is this important?

Delayed initiation of CFTR modulators has been associated with severe clinical consequences for individuals with Cystic Fibrosis. So, analyzing the specific segments of time employed by the US and the EU systems in making such treatments available can make the Cystic Fibrosis community, regulators, and policymakers aware to address actions to close such a gap efficiently.

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What did you do?

By collecting publicly accessible data from the US Food and Drug Administration and the European Medicines Agency websites, we assessed the differences in the time of approval for the marketing of CFTR modulator products. For each product, we evaluated the time employed by each regulatory agency for granting the first approval and subsequent variations of indications, e.g., new eligible mutations or younger eligible population. We also assessed the request for marketing authorization submitted by the applicant and assessed how it impacted the whole process.

What did you find?

We found that applications for the marketing of CFTR modulators were generally first submitted in the US, where the evaluation by the US Food and Drug Administration was faster than the European Medicines Agency. Overall, CFTR modulators were approved 267 days earlier in the US than in the EU. Delays in submission to the European Medicines Agency referred to 30% of the final delay in approval in the EU compared to the US.

What does this mean and reasons for caution?

Our findings reflect what was already observed in other therapeutic areas, where both submissions and approvals for marketing first occurred in the US. Delays in approval in the EU mainly referred to the time employed by the sponsor to address scientific objections raised by the European Medicines Agency. This highlights a different culture in weighing data in the US and the EU. Caution is needed in interpreting such results to discriminate delays that can be reduced by streamlining administrative burdens, from the time needed for a comprehensive scientific evaluation, that relies on different regulatory and social backgrounds.

What's next?

The EU pharmaceutical legislation is under review. Resembling the FDA model, the reform aimed to improve early interactions between the European Medicines Agency and applicants. Such upfront discussions are expected to improve the whole assessment process, reducing the gap with the US.

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