A Comparison of Orphan Drugs Approved by the FDA and EMA, 1995-2022

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Background

Orphan drugs are used for the treatment, diagnosis, and prevention of rare diseases affecting less than 200,000 patients in the US and less than 5 in 10,000 people in the European Union. The US FDA and EMA have different regulations and policies that affect the availability in orphan drugs in the US and European Union (EU). The EU also requires that the condition is life-threatening or seriously debilitating, and that there is currently either no satisfactory alternative or that the new drug is of significant benefit to those affected by the condition.

This study assessed the differences and characteristics of orphan drugs authorized by FDA and EMA in 1995-2022.

Methods

We extracted the regulatory data for the orphan and non-orphan new molecular entities and new therapeutic biologics from the FDA and EMA websites from the implementation of the EU Orphan Regulation in 2000 to 2002. We classified the drugs using the WHO Anatomical Therapeutic Chemical Classification System. We use descriptive analysis to characterize the differences the orphan drugs authorized by each agency.

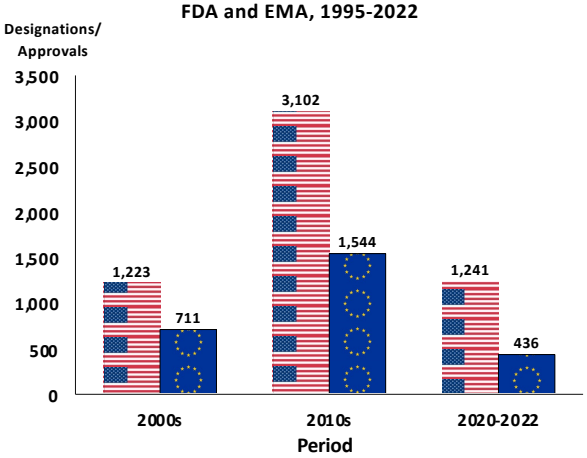
Results

The FDA granted orphan designation to 5,566 drugs and EMA to 2,691 drugs in the period from implementation of the EU orphan regulation in 2000 to 2022 (Figure 1). The average authorizations per year increased from 122 in the 2000s to 414 in 2020-2022 for the FDA, and from 71 in the 2000s to 145 in 2020-2022.

The FDA authorized 789 new drugs, of which 533 (67.6%) were also authorized by the EMA. The FDA granted orphan designation to 310 (39.3%) of those authorizations, while the EMA granted such designation to 106 (20.0%) authorizations.

Results

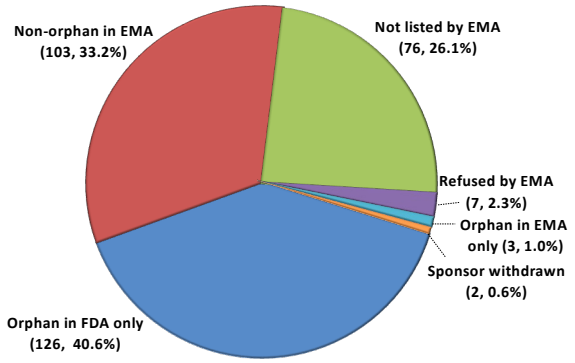
Figure 1. Orphan Designations FDA and EMA, 1995-2022



The EMA authorized with orphan designation 9.1% (n-=6) of the orphan drugs authorized by the FDA in the 2000s, 66 (40.7%) in the 2010s, and 34 (41.5%) in the 2020s. The EMA authorized with orphan designation 45 (56.8%) of the alimentary tract and metabolism, 8 (40.0%) of the blood and blood forming organs, and 21 (30.4%) of antineoplastic/immunomodulating orphan drugs authorized by the FDA.

Of the orphan drugs authorized by the FDA, 126 (40.6%) had orphan status in EMA, 103 (33.2%) had non-orphan status, 76 (26.1%) were not listed by EMA, and 7 (2.3%) were refused by EMA (Figure 2).

Figure 2. Orphan Designation and Approvals FDA and EMA, 1995-2022



Conclusions

The orphan designations granted by the FDA and EMA increased between the 2000s and 2020-2022. Orphan drugs accounted for 40% of FDA's new drug authorizations and 20% of EMA's new drug authorizations. The FDA granted orphan drug designation to more drugs than the EMA and authorized three times as many orphan drugs as the EMA.

The differences in approvals and designations are related to differences in orphan designation requirements and regulatory and sponsor companies' decisions.