

Safety Monitoring of Orphan Drugs Project: the SFDA Experience

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Objectives

Orphan drugs are developed to treat rare diseases. Some regulatory bodies established orphan drug designation with incentives to help pharmaceutical companies to develop orphan drugs. However, there is a lack of pre-marketing safety data for orphan drugs because limited number of patients involved, due to the rarity of the diseases. Considering that post-marketing long-term safety of orphan drugs still uncertain and the low utilization profile which make it challenging to monitor the safety of orphan drugs. The Saudi Food and Drug Authority (SFDA) initiated a project to monitor the safety of registered orphan drugs.

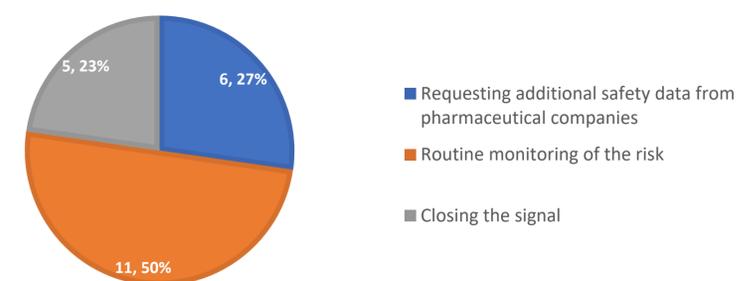
Methods

First, a list of SFDA registered orphan drugs from 2016 to 2022 were selected. Second, published reported adverse events retrieved from AdisInsight database. Third, labelness assessment was performed on the retrieved adverse drug event (ADEs) lists by crosschecking the label of U.S. Food and Drug Administration, European Medicines Agency label and local label information to exclude labeled ADEs. Fourth, a comprehensive drug safety review for the unlabeled ADEs was conducted using different evidence sources including unpublished clinical trials, literature, local and global spontaneous reports and Periodic Benefit-Risk Evaluation Reports.

Results

From 2016 to 2022, 35 orphan medications registered by SFDA, with a total of 1403 reported events on AdisInsight. The crosschecking resulted in requesting of local label update for 13 orphan drugs. Project yielded a total of 18 comprehensive drug safety reviews were performed for 22 potential safety signals. The safety review recommendations were requesting additional safety data from pharmaceutical companies (n=6 signals), routine monitoring of the risk (n=11 signals) and closing the signal (n=5 signals).

THE SAFETY REVIEW RECOMMENDATIONS



Conclusion

Safety Monitoring of Orphan Drugs Project in SFDA assisted in the identification and assessment of new potential signals with medications use. It is important raise the spontaneous reporting awareness associated with medicines especially orphan drugs.