ABSENCE OF PRICE-PREVALENCE CORRELATION IN ORPHAN DRUGS IN ITALY

Lanatti EP, Lidonnici D, Ronco V
MA Provider, Milano, Italy

Introduction: A disease is considered rare when it affects less than 5 people out of 10.000. Furthermore, this does not mean that the number of people affected by rare diseases is low, indeed, according to the Italian observatory of rare diseases, in Italy there are more than two millions of patients affected by a rare disease. Because of its low prevalence, drugs targeted to treat rare diseases require higher costs than usual, although this data has never been verified.

Objectives: The present study analyses the annual therapeutic costs of drugs indicated for rare diseases in Italy, in order to verify if a correlation between costs and pathologies prevalence exists or not.

Methods: We screened all orphan drugs available in Italy in terms of annual cost and prevalence of the pathology. Drugs with different annual costs or used for a pathology without available data of prevalence were excluded from the analysis. As source of prevalence data we chose the website Orphanet. Overall, 49 orphan drugs were considered. Drugs were analysed according to price (lower than 100.000 euro each year or higher than 20.000), according to therapeutic class (onco-hematology, enzymatic deficiencies, pulmonary diseases) and according to classes of prevalence.

Results: The analysis of the 49 drugs showed no correlation between annual therapeutic price and prevalence (Figure 1). In order to eliminate possible bias related to costs out of the scale, drugs with a therapeutic annual cost lower than 100.000 euro (n=38) and with therapeutic annual cost higher than 20.000 euro (n=9) were analysed separately. Both analysis confirmed that no price-prevalence correlation exists (data not shown). Even the analysis of orphan drugs sorted by therapeutic class (onco-hematology n=19, enzymatic deficiencies n=10, pulmonary diseases n=5) did not show a price–prevalence correlation (Figure 2). Similarly, no correlation was demonstrated classifying drugs into classes of prevalence (Figure 3).

Conclusions: In general, criteria to assign price to drugs are a strong matter of debate. Prevalence is generally considered one of the main criteria, thus to justify higher prices required by pharma companies marketing orphan drugs or indicated for low prevalence diseases. From the analysis performed, interestingly none of the subgroups considered showed any correlation between price and prevalence. According to this result, we concluded that prevalence data cannot be considered a major criteria adopted by AIFA in orphan drugs pricing.