Poster Code: HPR65

PREVALENCE AS A *DRIVER* OF TREATMENT COST FOR DRUGS IN THE RARE DISEASE FIELD IN ITALY

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BACKGROUND AND OBJECTIVE

Since the low prevalence associated with a rare disease (5 per 10,000 people)[1] could be a key factor in the definition of reimbursed drug price, the aim of the study was **to investigate the existence of a correlation between the prevalence of rare diseases and related treatment costs for drugs reimbursed in Italy.**

MATERIALS AND METHODS

A database with **115 EMA-approved drugs/indications for rare diseases** was created and populated in Microsoft Excel. A total of 80 drugs/indications reimbursed in Italy between January 2016 and December 2021 were considered and analyzed. Additional inclusion/exclusion criteria for analysis sample selection, are shown in **Figure 1**. The treatment cost was estimated based on net prices and related dosing regimens for each drug included in the analysis. In the case of weight-dependent posology, assumptions for average patient characteristics were 70 kg weight and 1.72m^2 BSA. For chronic indications, a treatment duration of 365 days was assumed, whereas for non-chronic indications (less than 1 year) the duration was commensurate with the treatment cycle length. Data about prevalence were collected from the EMA website and integrated with data from Orphanet. A linear regression analysis was performed to investigate the correlation between prevalence and treatment costs. Subgroup analyses were performed for chronic and orphan indications, ultra-rare diseases (≤1/50,000)[2], innovative indications, and class H/A-PHT drugs.

RESULTS

For the 80 indications considered, a statistically significant inverse correlation was found between the treatment cost and the related prevalence (r=-0.3036). Each percentage point increase in prevalence corresponds to an average reduction of about €40,600 in the cost of treatment (X1= -40,671; CI95% [-69,435;-11,908]; p= 0.00617) **(Figure 2)**. An even stronger inverse correlation was found by analyzing chronic indications (n=57; r=-0.5011). In fact, a statistically significant inverse correlation emerged from this subgroup analysis: each percentage point increase in prevalence corresponds to an average reduction in treatment cost of €47,000 (X1= -47,205, CI95% [-69,228;-25,180]; p=0.00007) **(Figure 3)**. Also, the analyses conducted for orphan indications (n=61) and drugs in class H/A-PHT (n=67) result in an inverse correlation (respectively, r=-0.2888 and r=-0.3946): each percentage point increase in prevalence corresponds to an average reduction of about €42,800 in the treatment cost (X1= -42,866; CI95% [-79,881;-5,850]; p= 0.0239) **(Figure 4)** for the first, and an average reduction of about €38,000 (X1= -38,397; CI95% [-60,543;-16,252]; p= 0.0009) for the second. A non-significant correlation was observed for ultra-rare indications and innovative drugs.

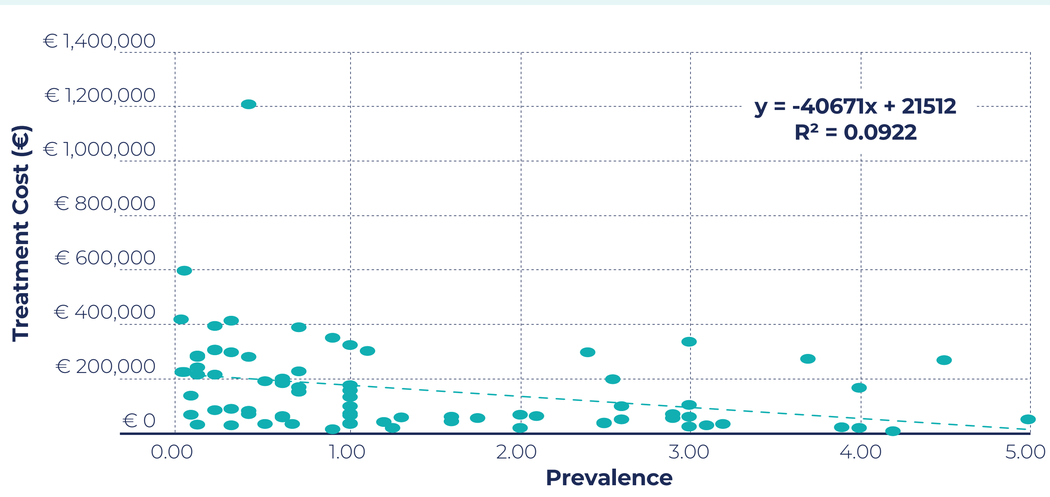

Figure 2. Overall results - Inverse correlation Treatment Cost vs. Prevalence

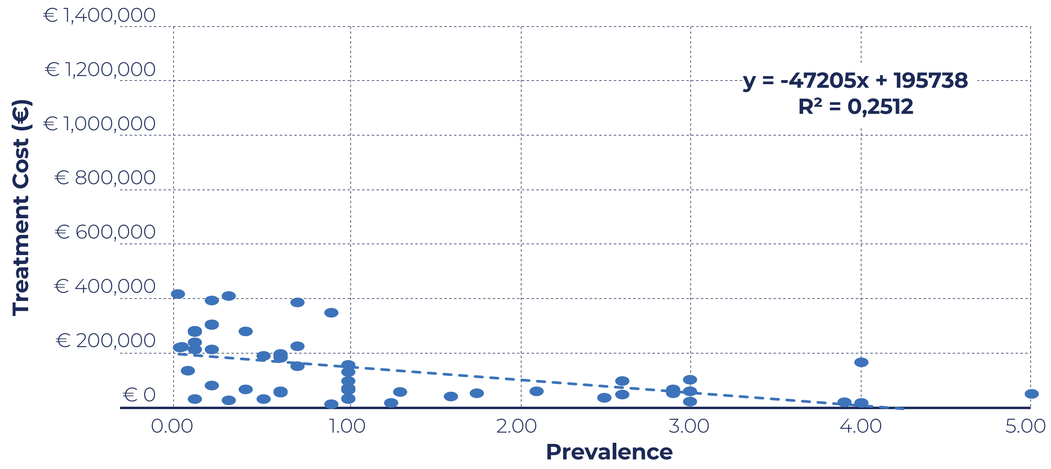

Figure 3. Chronic indications - Inverse correlation Treatment Cost vs. Prevalence

Figure 1. Sample selection flowchart.

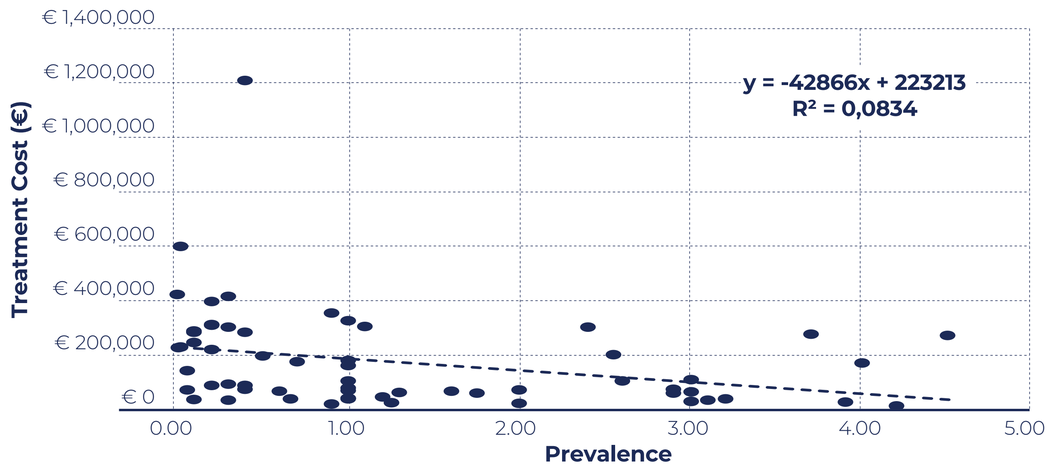

Figure 4. Orphan indications - Inverse correlation Treatment Cost vs. Prevalence

CONCLUSIONS

Although pricing outcomes come from a multifactorial process with several determinants, such as *unmet medical needs*, availability of therapeutic alternatives, added value, and P&R policies, **the results of this study show that epidemiology can be also considered one of the *drivers* in pricing decisions, at least in the field of rare diseases.** However, these results must be read considering some limitations such as the small sample size, especially for subgroup analyses, and the absence of specific prevalence data referring to the Italian context.

References:
1. *EUR Lex Regulation (No 141 2000 of the European Parliament and of the Council Available;* ***2.*** *Council, EUR Lex Regulation No 536 2014 of the European Parliament and of the Council.*

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