

PREVALENCE VS. TREATMENT COST: ANALYSIS OF DRUGS FOR RARE DISEASE IN ITALY

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Background

A disease is considered rare when it affects less than 5 people out of 10.000 in the EU¹, while in the USA when affects fewer than 200.000 people². Because of the low prevalence, drugs for rare diseases required considerable costs for R&D development, often resulting in high prices once on the market, in order to cover the high investment made by pharmaceutical companies.

Objectives

Focusing on rare diseases, the aim of the study was to assess possible correlation between disease prevalence and treatment cost in Italy.

Methods

Forty-four EMA-approved drugs indicated for rare diseases (46 first indications) reimbursed in Italy between August 2015 and March 2019 were tracked. Treatment cost was calculated based on net prices (mandatory temporary reductions² and confidential discounts included) and according to SmPC standard treatment schedules. Treatment duration was assumed 365 days for chronic indications and specific course duration was considered if shorter than one year. Assumptions for average patient characteristics were 70 kg weight and 1,7 m² BSA (if not differently indicated in the SmPC). Prevalence data were extracted from Italian setting³ (where available) or European sources⁴. The prevalence vs. treatment costs correlation was assessed through linear regression analysis. Subgroup analyses were performed for ultra-rare diseases ($\leq 10/100.000$) and innovative drugs (according to Italian criteria)⁵.

Results

For the forty-six indications analysed, prevalence ranged from 1 to 36/100.000, while treatment cost from 4.500€ to 594.000€ (mean 125.545€, median 61.333€). Overall results (Figure 1) show a statistically significant inverse correlation between prevalence and cost ($r = -0.36$; $p = 0.013$). Focusing on ultra-rare diseases (Figure 2) the inverse correlation was weaker, and results were not statistically significant ($r = -0.184$; $p = 0.358$). The subgroup analysis of innovative drugs (Figure 3) results in an even stronger inverse correlation ($n = 16$; $r = -0.50$; $p = 0.046$).

Figure 1. Annual treatment costs vs. prevalence – Overall sample.

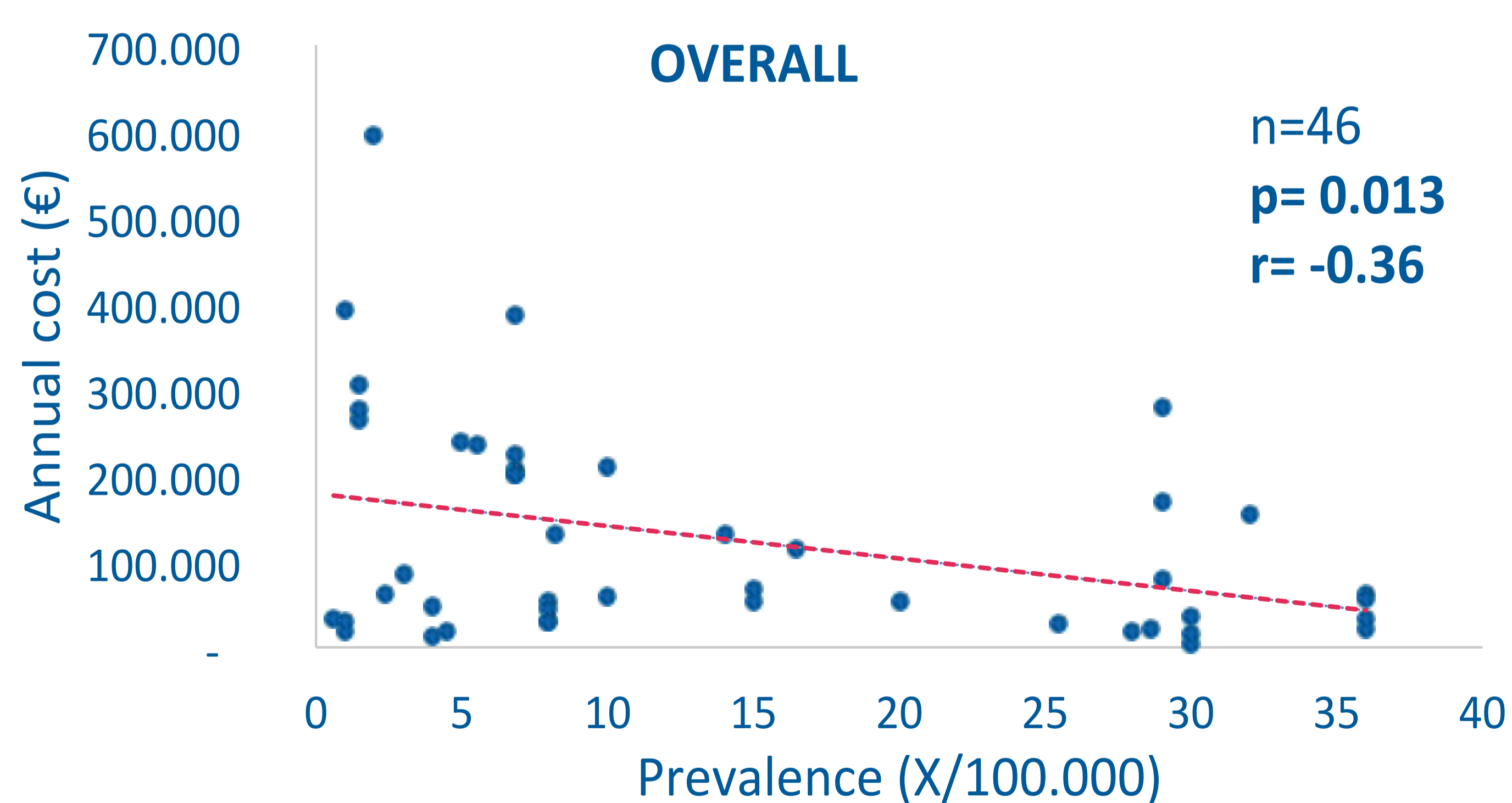


Figure 2. Annual treatment costs vs. prevalence – Indications for ultra-rare diseases (prevalence $\leq 10/100.000$).

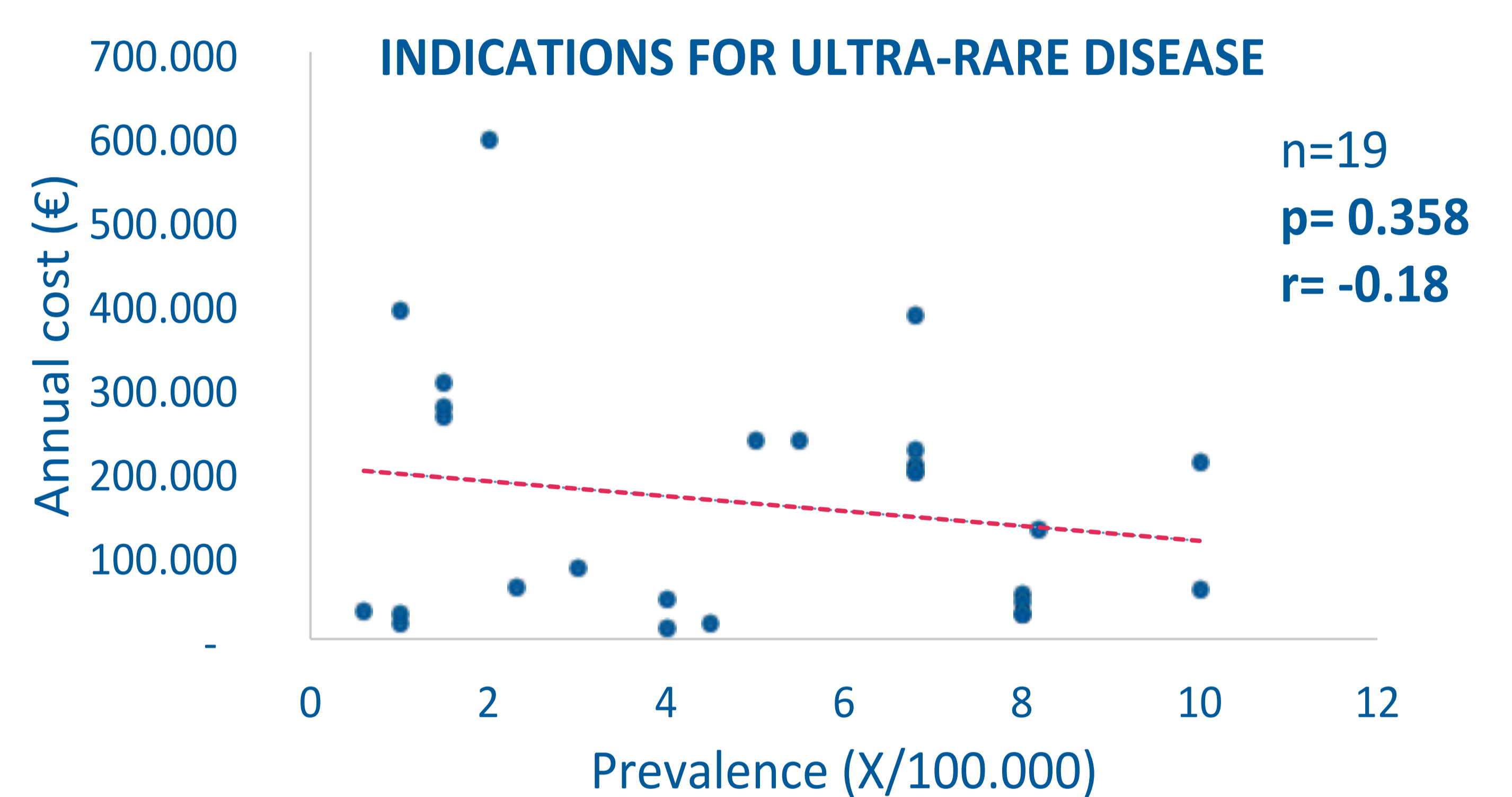
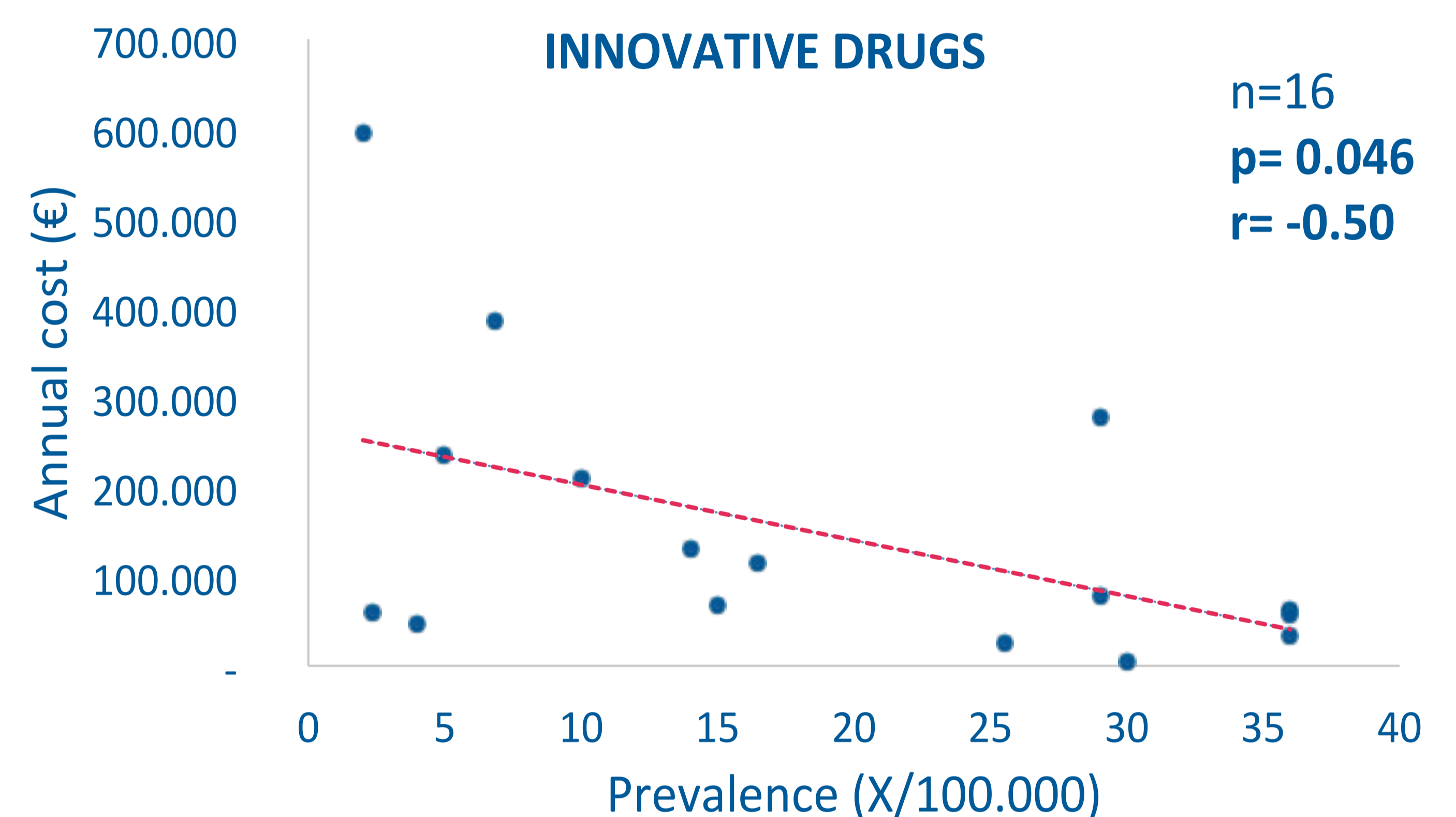


Figure 3. Annual treatment costs vs. prevalence – Innovative indications.



Conclusions

This analysis showed that although pricing outcomes derive from a multifactorial process with several determinants, such as unmet medical need, availability of therapeutic alternatives, added value, multiple indications and P&R policies, disease rarity can be also considered one of the drivers in pricing decisions, at least in the field of rare diseases. This was confirmed by innovative drugs subanalysis where AIFA innovative status criteria intrinsically consider therapeutic need, added value, quality of evidences. The overall results are coherent with previous similar studies⁷ performed for multiple countries including Italy, despite some methodological differences (use of ex-factory price and EU prevalence). Study limitations were small sample size (in particular for subgroup analyses), lack of details on applied MEAs (and their economic impact) and prevalence data not always referring to the specific Italian context.

References

- https://ec.europa.eu/health/non_communicable_diseases/rare_diseases_it
- <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>
- https://www.orpha.net/orphacom/cahiers/docs/GB/list_of_orphan_drugs_in_europe.pdf
- <https://www.osservatoriomalattierare.it/malattie-rare>
- <https://www.orpha.net/>
- <https://www.aifa.gov.it/>
- Medic et al. Do payers value rarity? An analysis of the relationship between disease rarity and orphan drug prices in Europe. J Mark Access Health Policy. 2017

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