

# DRIVERS OF ORPHAN MEDICINAL PRODUCT VALUE, PRICE AND COSTS

## A PRACTICAL EXPLORATORY ANALYSIS

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### Context and objectives

- The extent to which Orphan Medicinal Product (OMP) pricing should/does reflect value and the extent to which 'value' is aligned between and across pharmaceutical companies and healthcare systems have been the subject of hot debate.
- Previous investigations on the relationship between value drivers and price do not paint a consistent picture<sup>1-4</sup>.
- Heterogeneity across products, value drivers or contexts included may explain this lack of consistency<sup>5</sup>.
- We performed an exploratory analysis of OMP prices and their driving attributes across 5 EU (UK, France, Italy, Spain, Germany) on a set of OMP selected to reduce variability: **high-cost** (>100k€ annual treatment cost), **ultrarare** (prevalence<15/100k), **non-oncology** and **reimbursed in the past 10 years**.

### Methods

- This **exploratory analysis includes 14 products**: eculizumab, velaglucerase alfa, C1 esterase inhibitor[human], ivacaftor, teduglutide, mercaptamine bitartrate, defibrotide, ataluren, elosufase alfa, eliglustat, sebelipase alfa, migalastat, ex-vivo stem cell gene and nusinersen.
- The **price at market introduction, price changes up to Q1 2018, yearly drug usage** (dosage and admins), **HAS population estimates** and selected **value drivers** (administration route – IV/oral/other; comparator included in HAS evaluation – yes/no; first in class – yes/no) were captured from publicly available evaluation files. From these, the **median price** (across 5EU), the **yearly cost** (median price x dosage/year) and **budget impact** (yearly cost x population size estimate) were calculated.

### Results

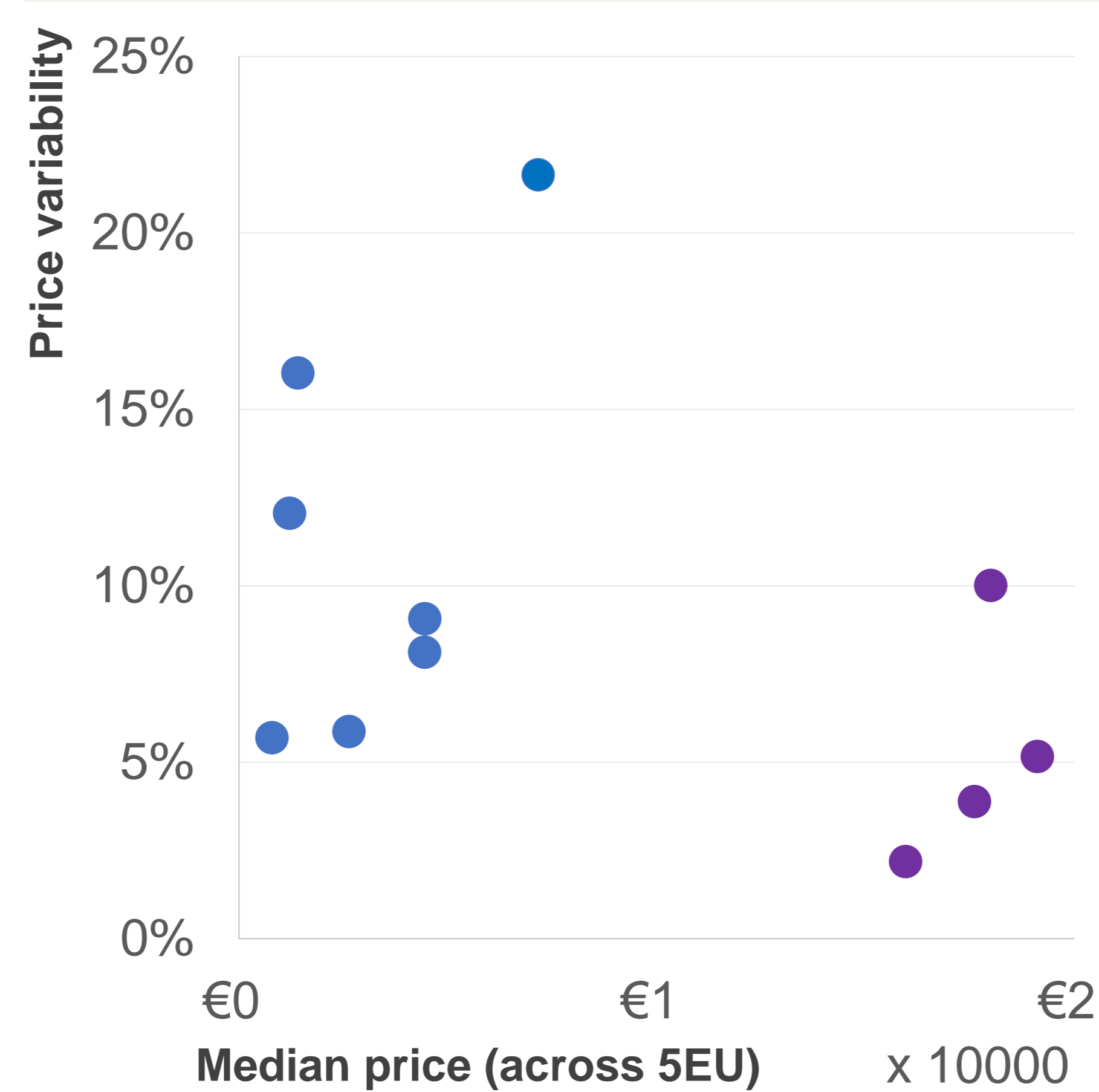
#### OMP price/cost is impacted by value

##### PRICE/COST PREMIUMS FOR INNOVATIVE PRODUCTS

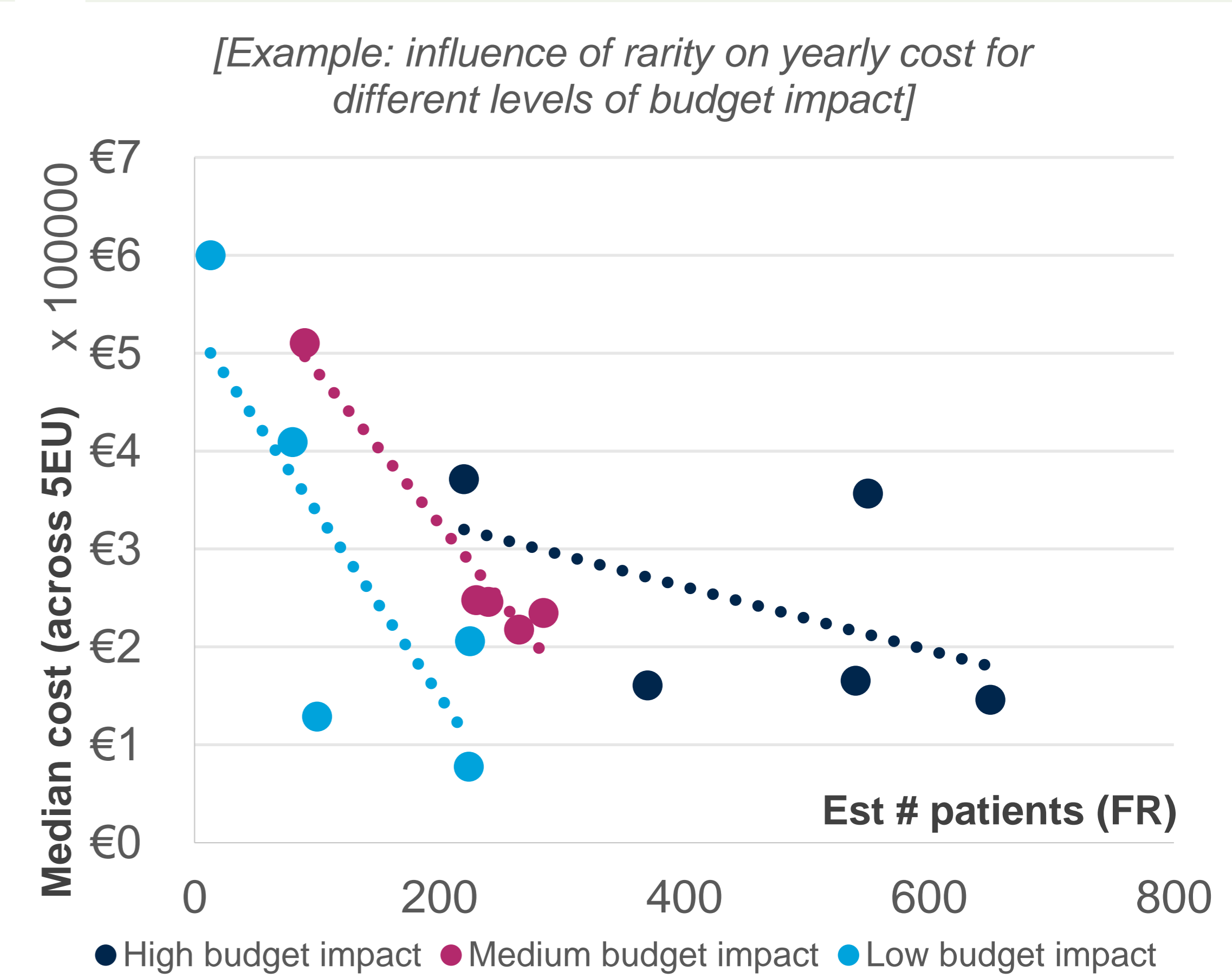
Value driver	Avg median price (excl. nusinersen)	Avg median cost per year
<b>Administration route</b>		
IV	€ 2 937	€ 318 173
Oral	€ 12 031	€ 262 988
<i>p-value (two-sided)</i>	0,06	0,52
<i>p-value (one-sided)</i>	0,03	
<b>Comparator</b>		
None	€ 7 607	€ 302 912
Aim and/or goal	€ 8 428	€ 196 483
<i>p-value (two-sided)</i>	0,8	0,11
<i>p-value (one-sided)</i>		0,06
<b>First in class</b>		
Yes	€ 8 743	€ 307 013
No	€ 10 233	€ 209 104
<i>p-value (two-sided)</i>	0,4	0,14
<i>p-value (one-sided)</i>	0,2	0,07

#### Price/cost elasticity is dependent on context

##### LOWER PRODUCT PRICE VARIABILITY (ACROSS 5EU) FOR HIGHER PRICED PRODUCTS

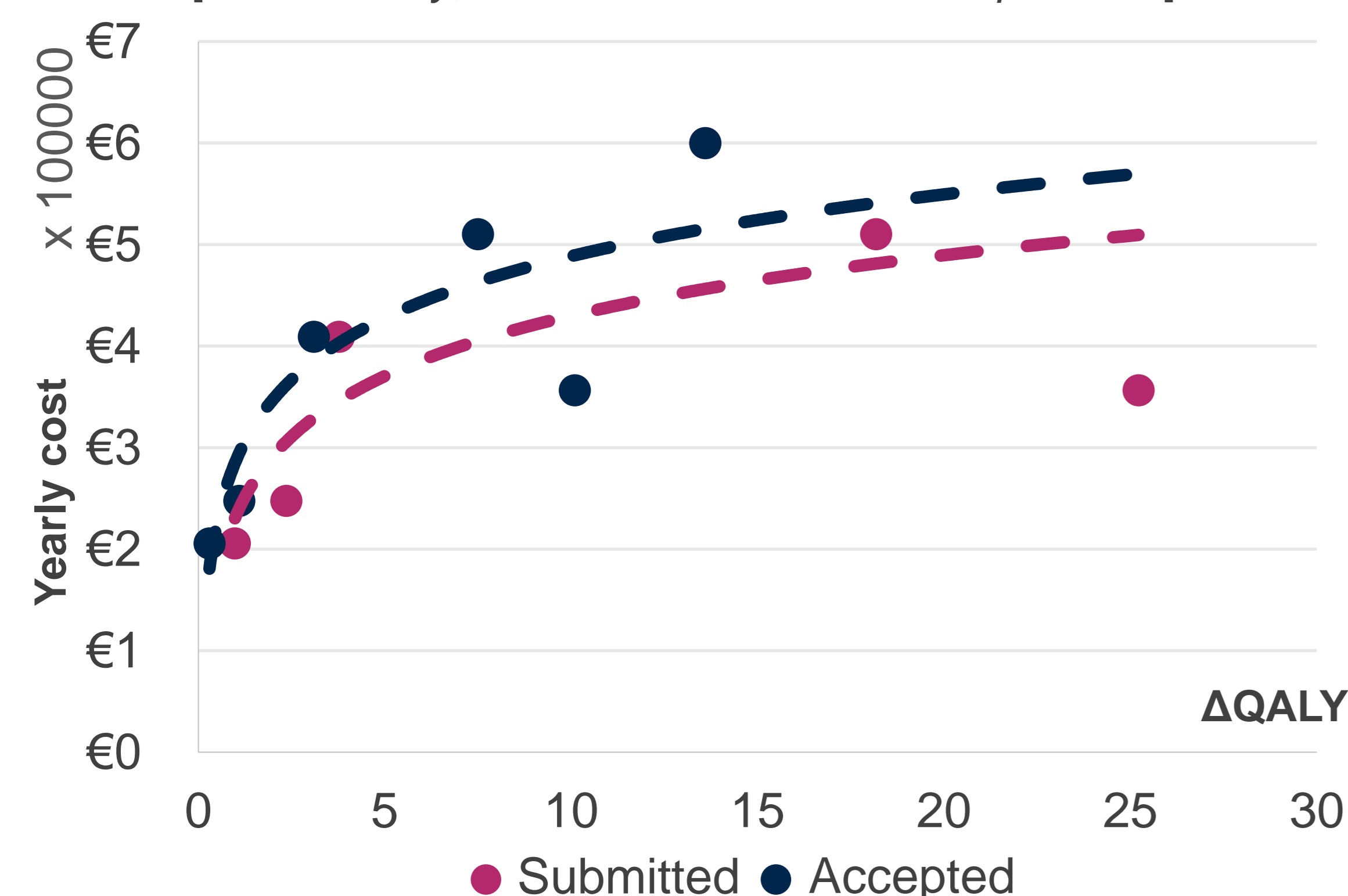


##### WILLINGNESS TO PAY (COST) ELASTICITY IS LOWER FOR HIGH BUDGET IMPACT PRODUCTS

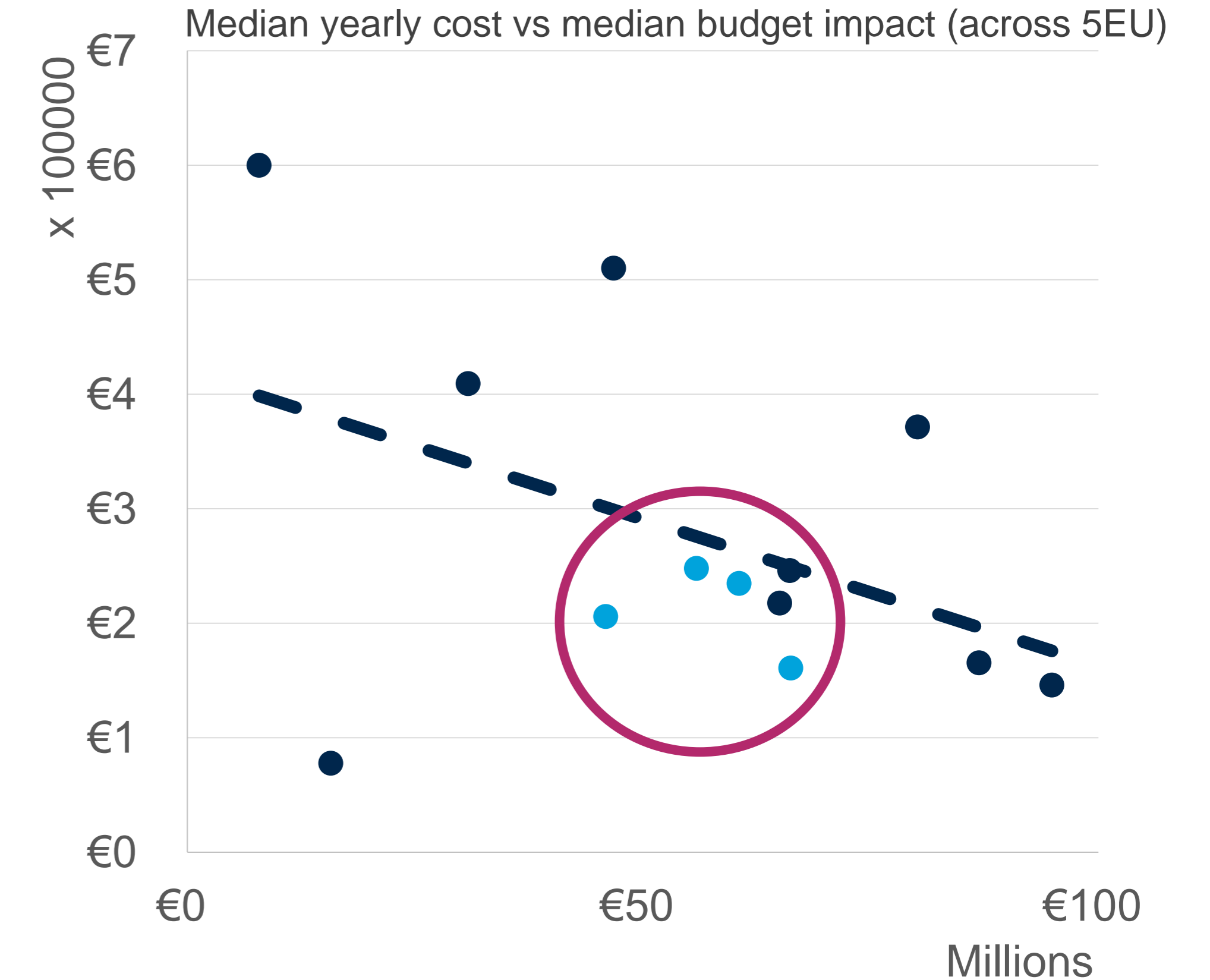
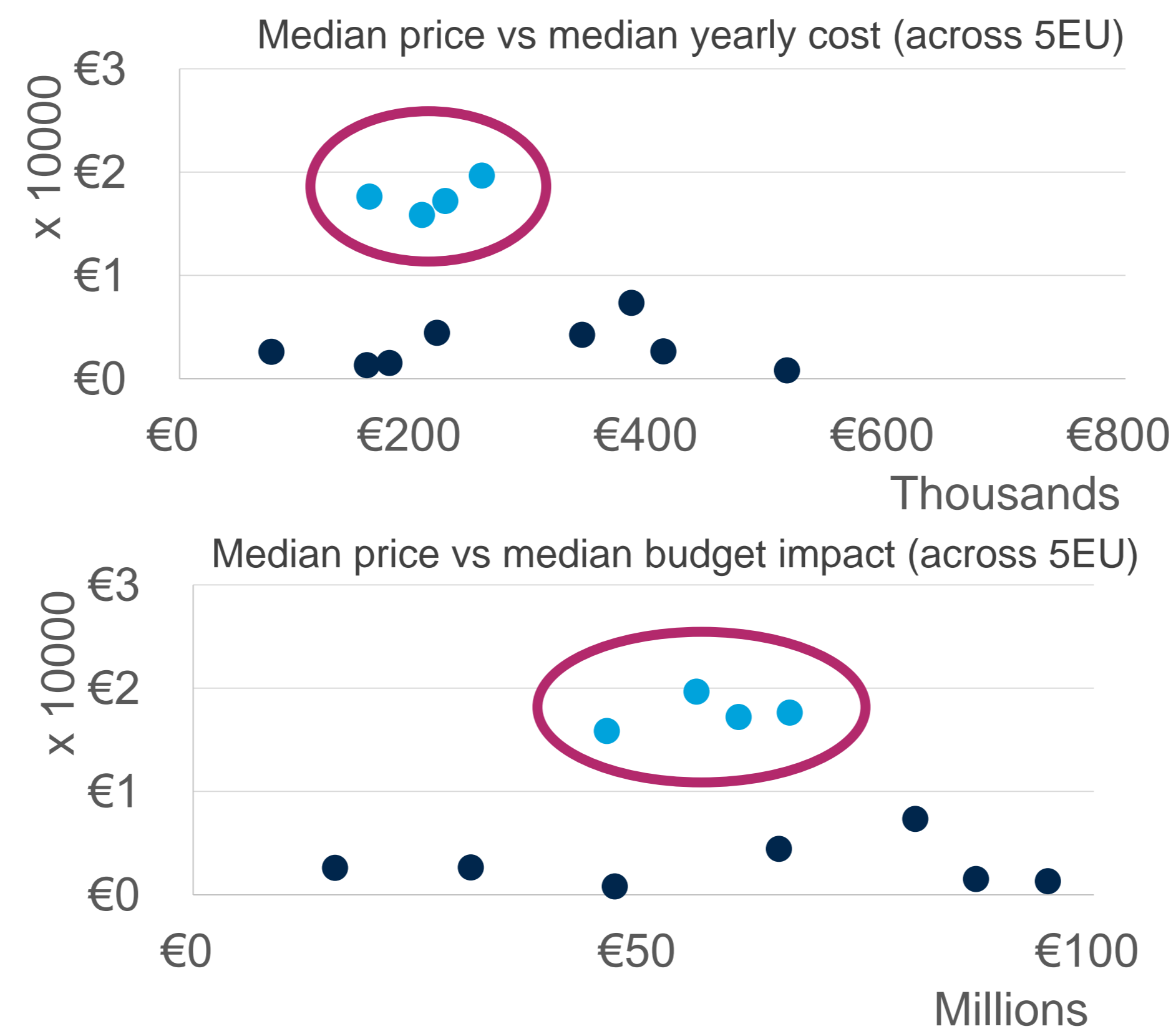


##### HIGHER YEARLY COSTS ARE ACCEPTED FOR PRODUCTS WITH HIGHER INCREMENTAL QALY

[UK data only, Data available in subset of products]



##### PRICE PREMIUMS (VS SIMILAR PRODUCTS IN TERMS OF YEARLY COST OR BUDGET IMPACT) APPEAR CAPPED BY COST VS BUDGET CONSIDERATIONS



- ### Conclusions
- Our study suggest OMP value drivers outside of prevalence, such as QALY impact and 'innovativeness' are implicitly and consistently taken into consideration
  - Financial considerations may limit willingness to accept price or cost premiums and therefore the feasibility of traditional "value-based pricing" methodologies. Value modulated pricing approaches such as setting modulated or explicit "value to pay" thresholds<sup>6</sup> or using a "value informed and affordable pricing" approach may present more appropriate approaches and warrant further investigation.

<sup>1</sup>Picavet et al., Orphanet J Rare Dis. 2014;9:62; <sup>2</sup>Schlender et al., J Comp Eff Res. 2014;3(4):399; <sup>3</sup>Schlender et al., J Mark Access Health Policy. 2016;4:10; <sup>4</sup>Simoens et al., Orphanet J Rare Dis. 2011;6:42; <sup>5</sup>Rollet et al., Orphanet J Rare Dis. 2013;8:109; <sup>6</sup>Berdud et al., OHE Research Paper 18/05