

## Introduction

List prices often do not reflect prices health systems pay for technologies. Confidential discounts in the form of rebates or managed entry agreements make it challenging to estimate net prices.

Orphan drugs (ODs) are often associated with high costs and complex net pricing mechanisms, many of which are not made transparent in value assessment and pricing frameworks.

This research aims to determine factors driving confidential discounts and estimated net prices for non-oncological ODs in the EU4 and US.

The exclusion of oncology in this research stems from the notion that most oncology drugs are launched in specific patient subgroups, with limited disease heterogeneity to properly inform discount estimates.

## Methodology

Two payer experts from each market (US, Germany, France, Italy, and Spain) were interviewed to understand the factors driving confidential discounts, as well as confidential discount estimates for non-oncological ODs.

Non-oncological ODs granted marketing authorization by the European Commission from January 2017 to June 2023 were then identified using the European Medicines Agency database.

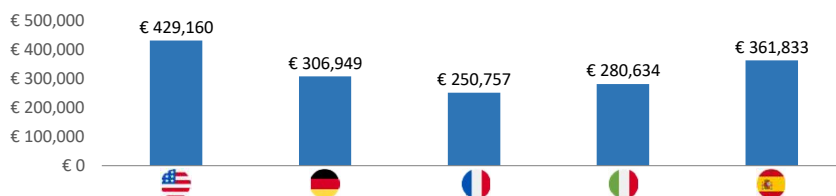
To compare list prices, ODs were then filtered based on availability of annual list prices in all scope markets, using the NAVLIN® database.

Confidential discount estimates from payers were then applied to list prices to reach estimated net prices.

## Results

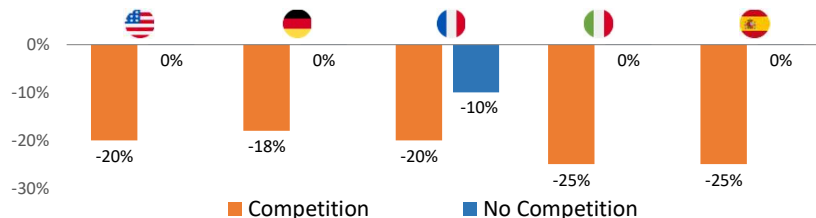
Of 68 identified ODs, 17 had visible list prices across all scope countries. Average annual list prices ranged from €250,757 in France to €429,160 in the US.

**Figure 1: Average List Prices of ODs (n=17) in the US and EU4**



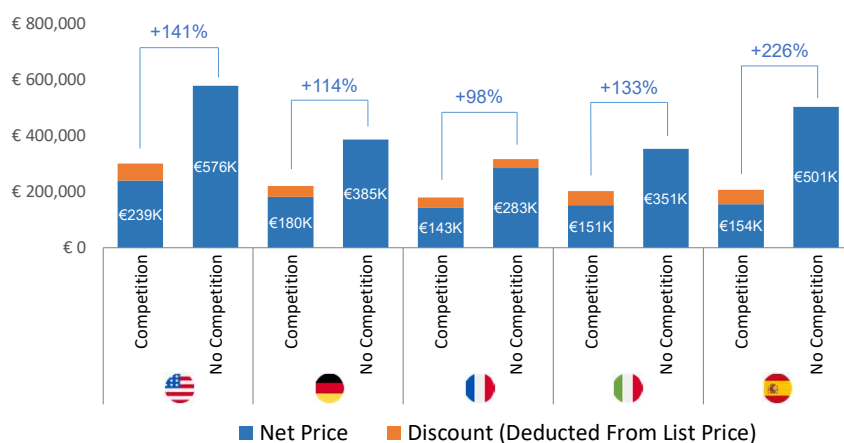
All payers identified competition as the key factor driving confidential discounts. Other factors such as clinical differentiation and budget impact were mentioned as less influential factors driving confidential discounts in France, Italy, and Spain. Discounts with vs without competition were -20% vs 0% in the US, -18% vs 0% in Germany, -20% vs -10% in France, -25% vs 0% in Italy, and -25% vs 0% in Spain.

**Figure 2: Discount Levels (%) for ODs With vs Without Competition**



Application of these confidential discounts to formal list prices resulted in higher net prices for ODs that launched without competition compared to those that launched with competition (98%-226% higher).

**Figure 3: Average Discounts and Net Prices of ODs (n=17) With vs Without Competition**



## Conclusion

ODs launching with competition have lower net prices compared to ODs without competition, driven by lower list prices and higher discount levels.

Competition gives payers increased leverage to negotiate confidential discounts for ODs across the EU4 and US. A more nuanced understanding of OD net price setting can help strategic planning for both health systems and manufacturers.

## References

- 1) European Medicines Agency (2023), Table Of All Orphan Designations, Accessed on: 30/06/23
- 2) NAVLIN (2023), NAVLIN Price Database, Accessed on 30/06/23