

# ORPHAN DRUG MARKET ANALYSIS IN TURKEY

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## INTRODUCTION

- Rare diseases are defined as life-threatening, deteriorating or serious chronic conditions affecting a relatively small number of patients.
- Orphan drugs, specifically designed for treatment of rare diseases are of special importance in all healthcare systems.
- Drugs approved for an orphan indication in EMA, also play significant role in the Turkish pharmaceutical market.

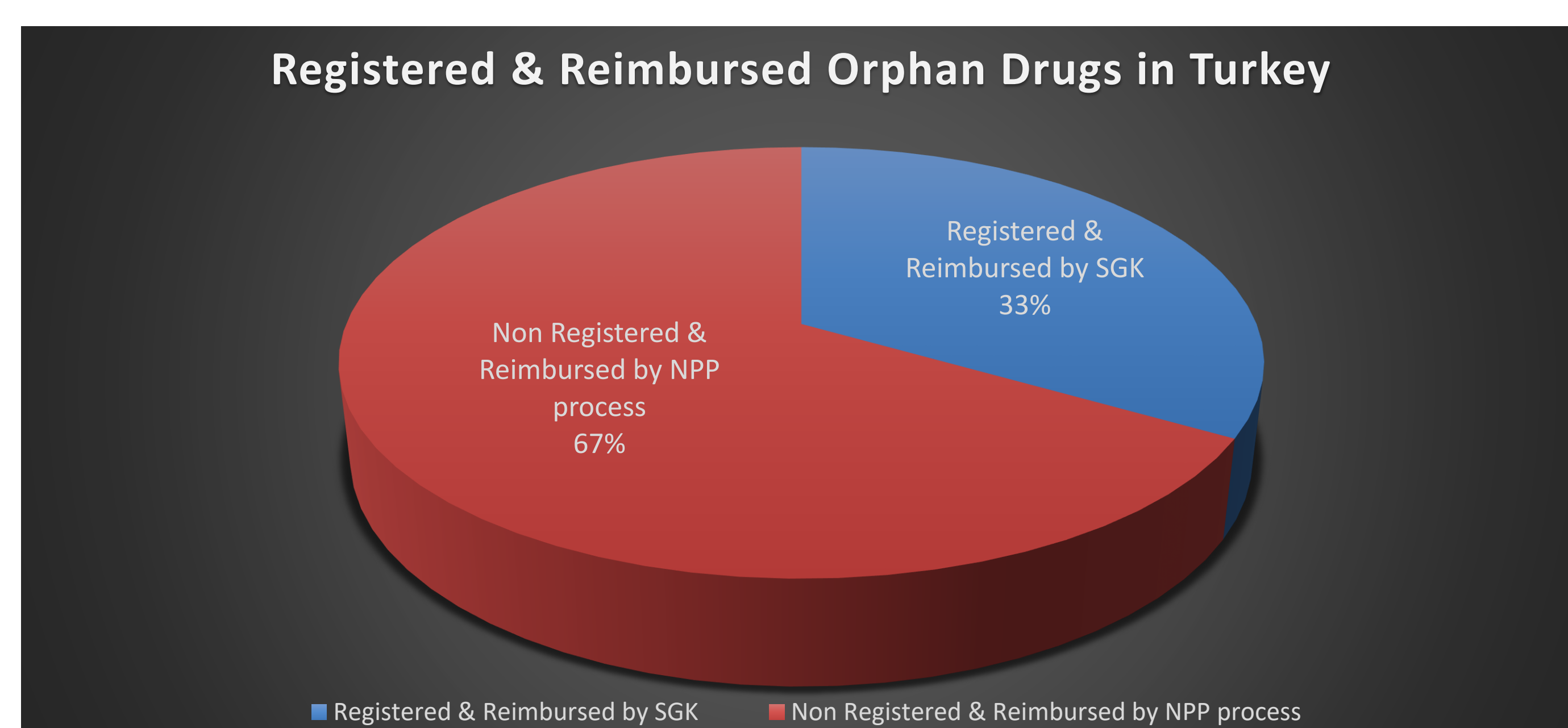
## OBJECTIVE

- The purpose of this study is to analyze the orphan drug market in Turkey and to identify its share in pharmaceutical expenditures.

## METHODOLOGY

- The study was undertaken from the Turkish health care payer perspective (SSI).
- An Excel sheet was formed to calculate the ratios and results.
- Resource utilization data were obtained from Orphanet Report April 2016, RxMediapharma, Social Security Institution Health Implementation Guideline Annex 4/A and Annex 4/C.

**Graphic 1: Registered & Reimbursed Orphan Drugs in Turkey**

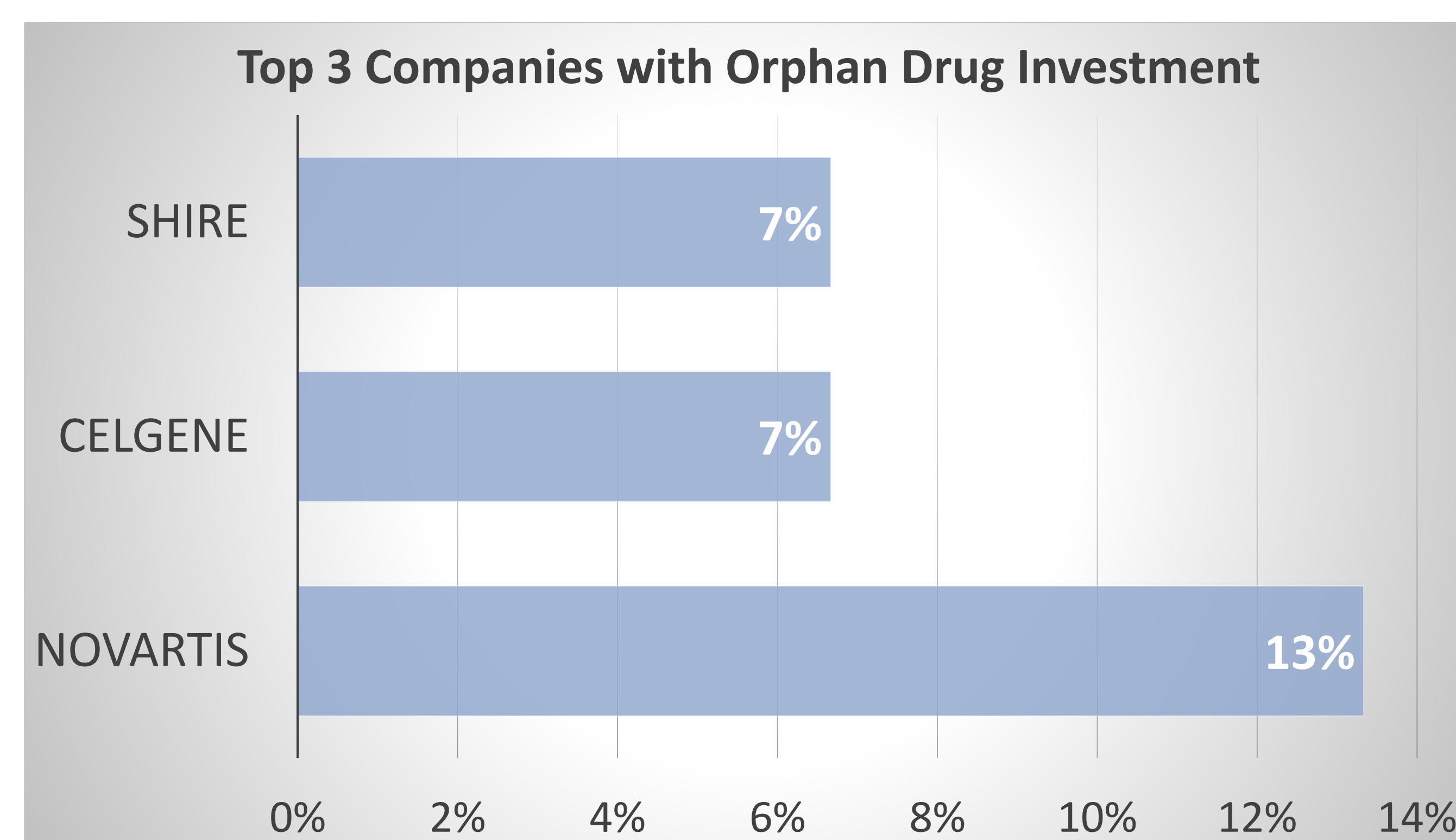


**Table 1: Top 10 Prescribed Orphan Drugs Based On Unit Box in 2015**

Active Ingredient Name	Company
Elosulfase Alfa	Biomarin
Eculizumab	Alexion
Sapropterin Dihydrochloride	Biomarin
İdursulfase	Shire
Defibrotide	Gentium
Carfilzomib	Amgen
Thiotepa	Adienne S.r.l
Brentixumab vedotin	Takeda
Mifamurtide	Takeda
Pirfenidone	Roche

Source: Turkish Pharmacists' Association

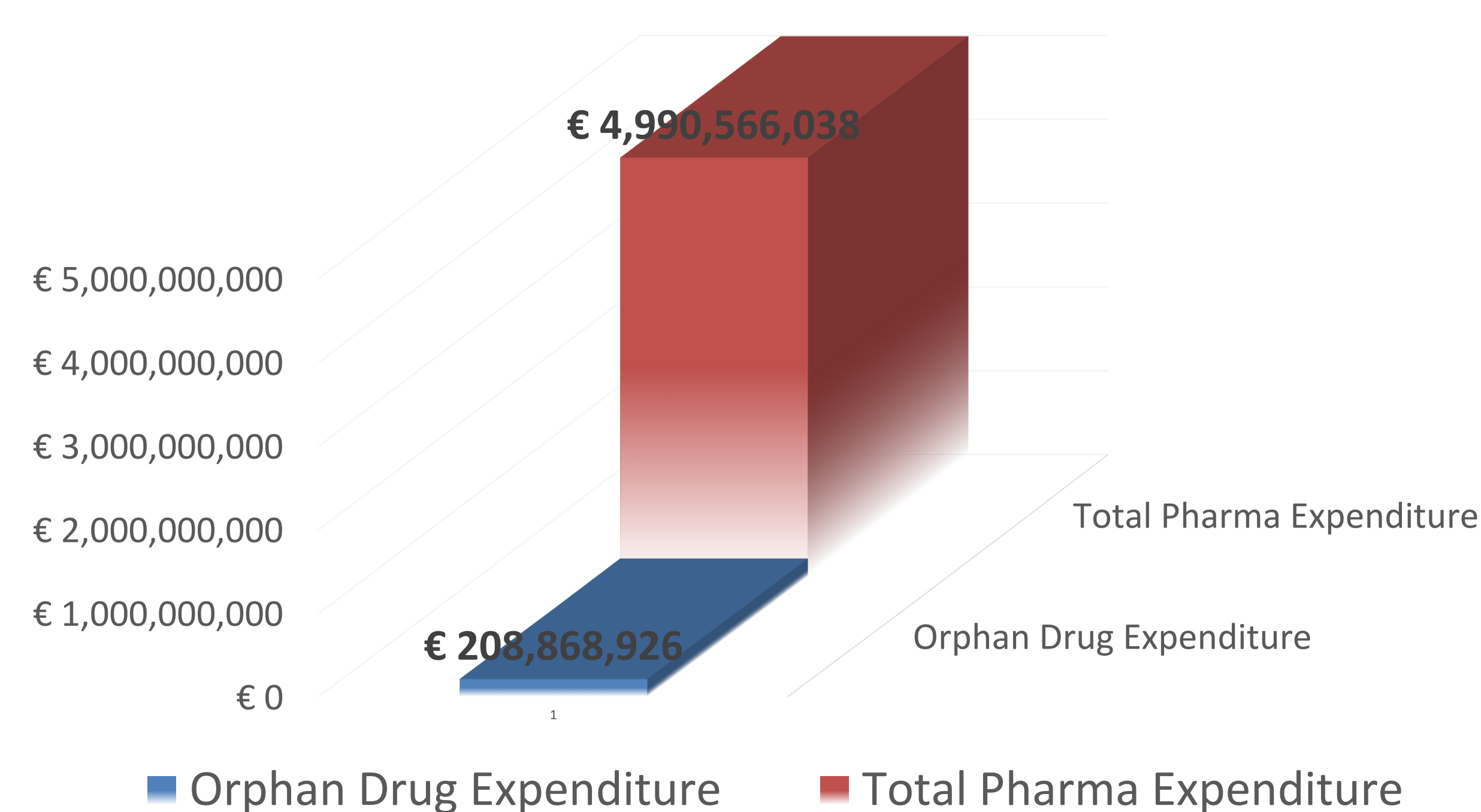
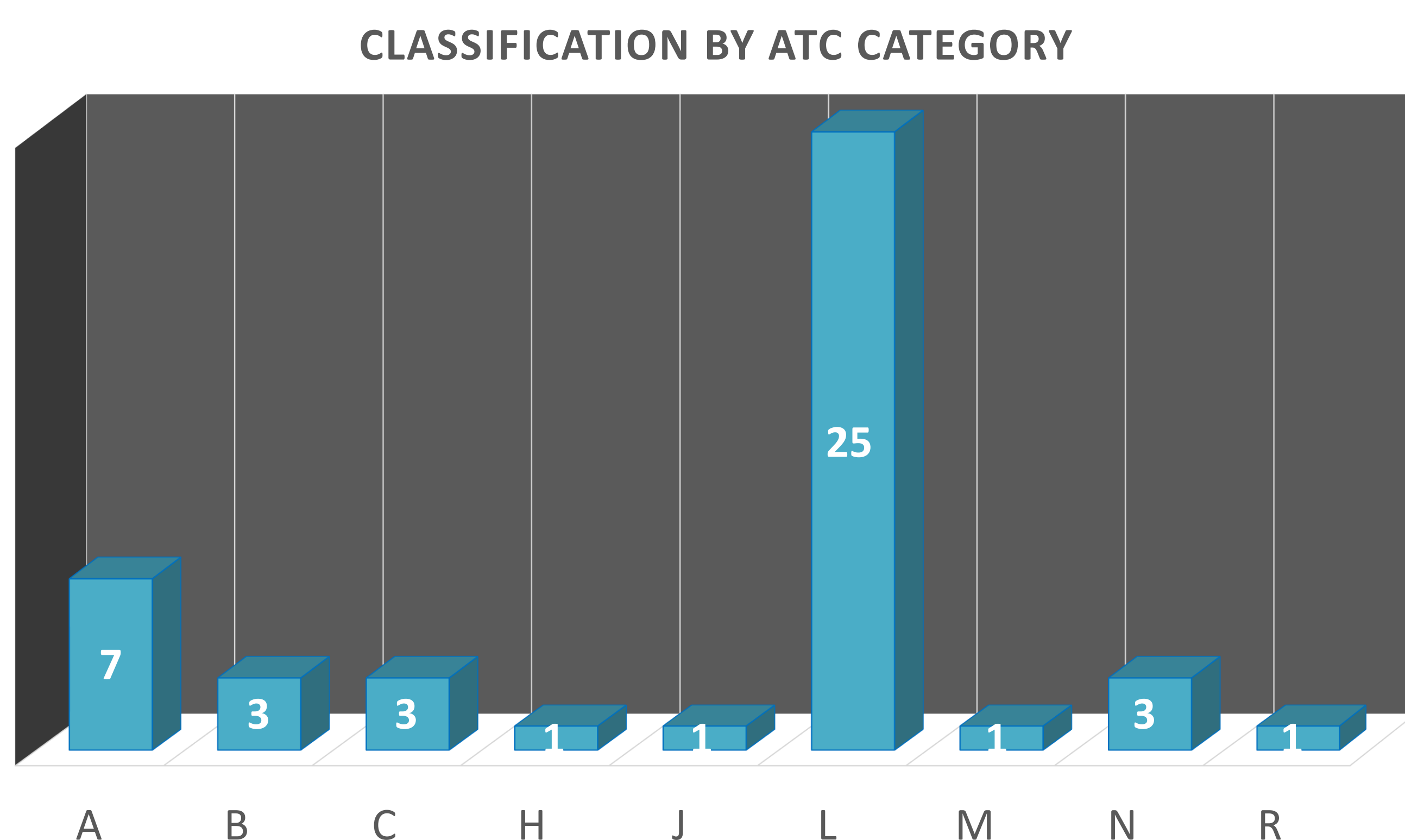
**Graphic 3: Top 3 Companies with Orphan Drug Investment**



**Graphic 4: 2015 Orphan Drug Expenditure**

## ORPHAN DRUG EXPENDITURE

**Graphic 2: Orphan Drugs Classification by ATC Category**



## RESULTS

- 45 products classified as "Orphan Drug" by the EMA are reimbursed by the SSI in the Turkish Market. These constitute 52% of 86 products.
- There are 15 orphan drug products with marketing authorization and in the positive list (Annex 4/A) that constitute 33% of the overall orphan drug market. On the other hand, products with orphan drug status, supplied through the Turkish Pharmacists' Association channel on NPP basis and in the overseas product list (Annex 4/C) comprise 67% of the total orphan drug market. (Graph1)
- The top 3 companies with orphan drug investment are; Novartis, Celgene and Shire. (Graph 3)
- In 2015, 5 orphan drugs entered the market with reimbursement approval in Antineoplastic and Immunomodulating, Respiratory, Musculo-skeletal System, Alimentary Tract and Metabolism, Cardiovascular System ATC groups.
- The Turkish Orphan Drug Market mostly comprises of Alimentary Tract and Metabolism and Antineoplastic and Immunomodulator drugs based on ATC classification. (Graph 2)
- Table 1 shows the top 10 prescribed orphan drugs accessed via NPP Process in the Turkish Pharma market.

## CONCLUSION

- The total expenditure of orphan drugs in 2015 was €208,868,926 (664.203.185 TRY). Orphan Drugs had a share of %4,2 in the global pharmaceutical budget. According to first six months of the year 2016, based on unit box, drug expenditure for the top ten orphan drugs was €123,379,021.

## ABBREVIATIONS

- NPP – Named Patient Process
- EMA – European Medicines Agency
- SSI – Social Security Institution
- ATC – Anatomical Therapeutic Chemical Classification

## REFERENCES

- Turkish Phramacists' Association
- Rx Media Pharma
- SSI Health Implementation Guideline Annex 4/C and 4/A
- Import Product List of Turkish Medicines and Medical Devices Agency
- Orphanet Report Series, List of medicinal products for rare diseases in Europe, April 2016.