

Analysis of Anatomical Therapeutic Chemical Classification and Therapeutic Area of Orphan Medicinal Products vs Reimbursement of These Drugs in 11 European Countries

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OBJECTIVES

Orphan medicinal products (OMPs) registered by the European Medicines Agency (EMA) are characterized by considerable heterogeneity. The **aim of this study** was to assess OMPs by the Anatomical Therapeutic Chemical (ATC) classification and therapeutic area, and to investigate correlations between these classifications and reimbursement decisions in selected European countries.

METHODS

The study was conducted between 2021 and 2022. Data on ATC classification and therapeutic areas of OMPs were collected from the **EMA web-based registry**, while information on reimbursement was collected using a questionnaire survey. Experts from the following **11 countries** were included in the analysis: Belgium, Finland, France, Germany, Greece, Iceland, Italy, the Netherlands, Scotland, Spain, and Poland.

TABLE 1. UNIVARIATE ASSOCIATIONS BETWEEN ATC AND REIMBURSEMENT OF AN ORPHAN MEDICINAL PRODUCTS (2022)

ATC classification	Reimbursement	
	OR (95% CI)	p-value
ATC B vs. ATC A	2.09 (0.86 to 5.11)	0.104
ATC C vs. ATC A	2.47 (0.69 to 8.75)	0.163
ATC D vs. ATC A	0.45 (0.10 to 2.08)	0.306
ATC H vs. ATC A	0.85 (0.38 to 1.93)	0.706
ATC J vs. ATC A	0.99 (0.52 to 1.87)	0.977
ATC L vs. ATC A	3.35 (1.16 to 9.69)	0.026
ATC M vs. ATC A	2.66 (0.90 to 7.83)	0.076
ATC N vs. ATC A	1.95 (0.82 to 4.65)	0.132
ATC P vs. ATC A	0.05 (0.00 to 1.61)	0.090
ATC R vs. ATC A	10.88 (0.95 to 124.67)	0.055
ATC S vs. ATC A	0.91 (0.38 to 2.17)	0.836
ATC V vs. ATC A	1.15 (0.39 to 3.42)	0.796

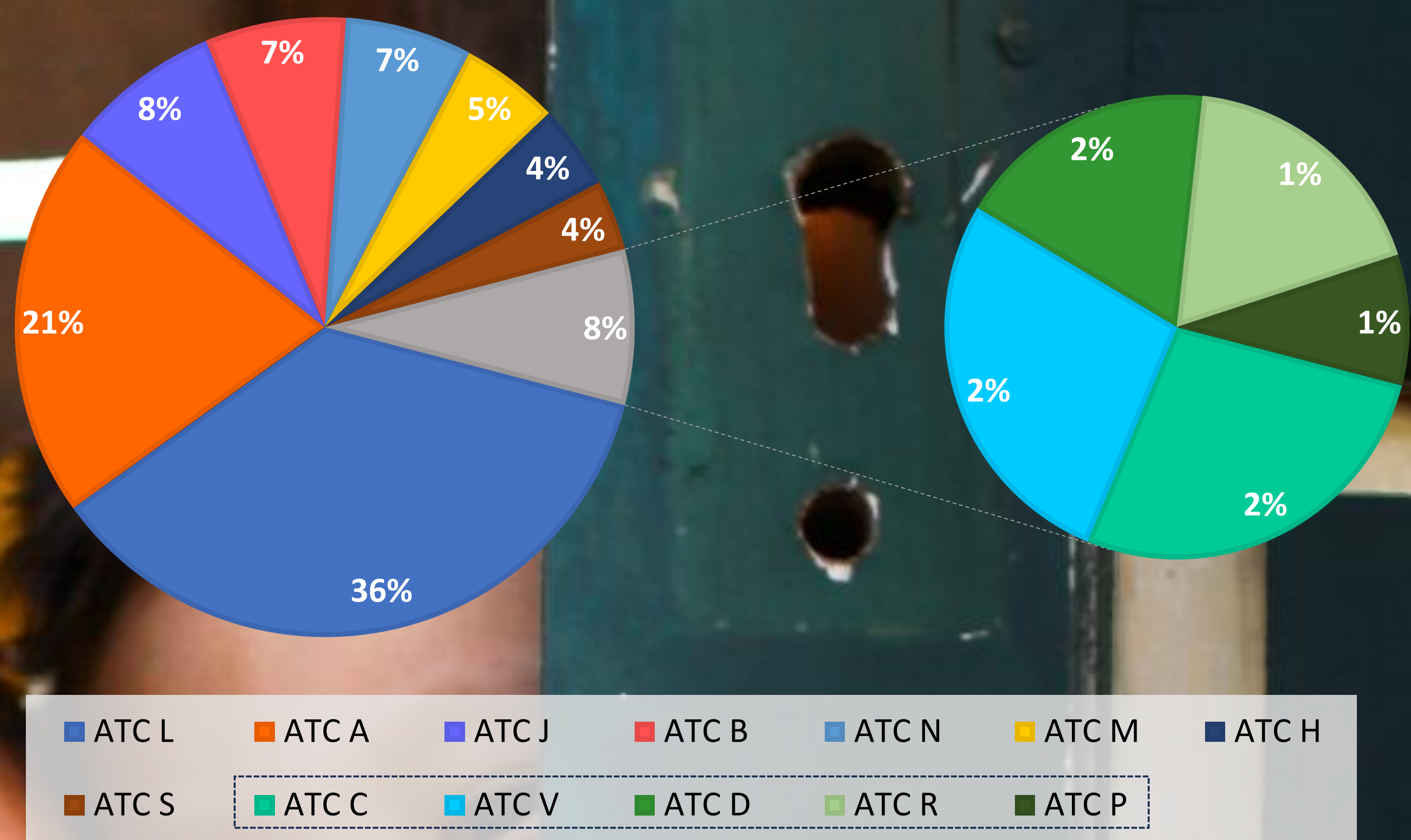
RESULTS

In September 2022, there were 136 OMPs in the EMA web-based registry. The **largest group included OMPs with ATC L** classification (antineoplastic and immunomodulating agents), representing 36% (n=49) of all medicines. On the other hand, the smallest group included OMPs with ATC P classification (antiparasitic products, insecticides, and repellents), accounting for 0.7% (n=1) of the medicines. In the countries surveyed, an average of **62% of OMPs classified as ATC L were reimbursed** (p<0.001). Class ATC L OMPs were three times more likely to be reimbursed than OMPs with other ATC classifications (p=0.026).

TABLE 2. REIMBURSEMENT OF ORPHAN MEDICINAL PRODUCTS IN EUROPEAN COUNTRIES ACCORDING TO ATC (2022)

ATC classification	Mean %	p-value
ATC A (Alimentary tract and metabolism)	43.2%	<0.001
ATC B (Blood and blood forming organs)	54.6%	0.033
ATC C (Cardiovascular system)	57.6%	0.839
ATC D (Dermatologicals)	31.8%	0.400
ATC H (Systemic hormonal preparations, excluding sex hormones and insulins)	40.9%	0.160
ATC J (Antiinfectives for systemic use)	43%	0.072
ATC L (Antineoplastic and immunomodulating agents)	61.8%	<0.001
ATC M (Musculo-skeletal system)	58.4%	0.266
ATC N (Nervous system)	53.5%	0.034
ATC P (Antiparasitic products, insecticides and repellents)	9.1%	0.358
ATC R (Respiratory system)	77.3%	0.199
ATC S (Sensory organs)	41.8%	0.535
ATC V (Various)	45.5%	0.551
All ODS	52.5%	<0.001

FIGURE 1. AUTHORISED MEDICINES WITH ORPHAN DESIGNATION IN EUROPE BY ATC CLASSIFICATION (2022)



The most common therapeutic areas of OMPs were **leukemia, lymphoma, multiple myeloma** (each disease had 8 specific OMPs), but a single medicine may have had more than one indication. **Six OMPs were reimbursed in all the countries surveyed:** daratumumab (multiple myeloma; ATC L), pomalidomide (multiple myeloma; ATC L), carfilzomib (multiple myeloma; ATC L), gemtuzumab ozogamicin (leukemia; ATC L), cabozantinib (leukemia; ATC L), and macitentan (hypertension and related conditions; ATC C - intended for the treatment of cardiovascular disorders).

FIGURE 2. THE THREE MOST COMMON THERAPEUTIC AREAS OF OMPs (2022)



CONCLUSIONS

The analysis showed that OMPs for oncological diseases (ATC L) are the largest group in terms of ATC classifications and therapeutic indications. The **ATC L classification increases the chances of reimbursement**, resulting in high reimbursement rates for these pharmacotherapies in Europe.

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