

DRUG UTILIZATION PROFILES OF ADVANCED THERAPY MEDICINAL PRODUCTS: A REAL-WORLD EVIDENCE STUDY



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BACKGROUND AND IMPORTANCE



Advanced therapy medicinal products (ATMPs) represent the **forefront of healthcare innovation**. Despite the approval of the first ATMP in Italy in 2016, there is currently a lack of scientific evidence concerning the utilization patterns of ATMPs.

AIM AND OBJECTIVES



The study aimed to evaluate the drug utilization patterns among patients receiving ATMP treatments in Italy.

MATERIALS AND METHODS



A **Retrospective study** was performed using data sourced from the Monitoring Registries of the Italian Medicine Agency, specifically the **Drug Product Registry (DPR)**, between 2016 and 2023 containing information on:



- dispensed treatments and clinical data for patients utilizing ATMPs in the Campania Region (~6 million, 10% of the national population)
- residents were treated in a **different Italian Region**. The final cohort included individuals who received at least one prescription for ATMP drugs.



We analyzed prescription patterns focusing on the **index treatment, diagnoses, treatment interruptions, mortality rates and adverse events**.

RESULTS



In total, 92 patients initiated ATMP treatments between 1 January 2016 and 1 September 2023.

26.1% of patients received voretigene neparovec, 25% onasemnogene abeparovec, 22.8% tisagenlecleucel and 21.7% axicabtagene ciloleucel. The overall occurrence of adverse events was very low (1.1%), primarily associated with Autologous human corneal epithelial cell treatments.

The overall mortality rate was 12%, affecting only two drugs: 28.6% tisagenlecleucel and 25.0% axicabtagene ciloleucel. Notably, nearly 90% of subjects completed their treatment without experiencing adverse events or mortality (see Table 1).

Figure 1. Number of patients treated with ATMPs between 2016 and 2023

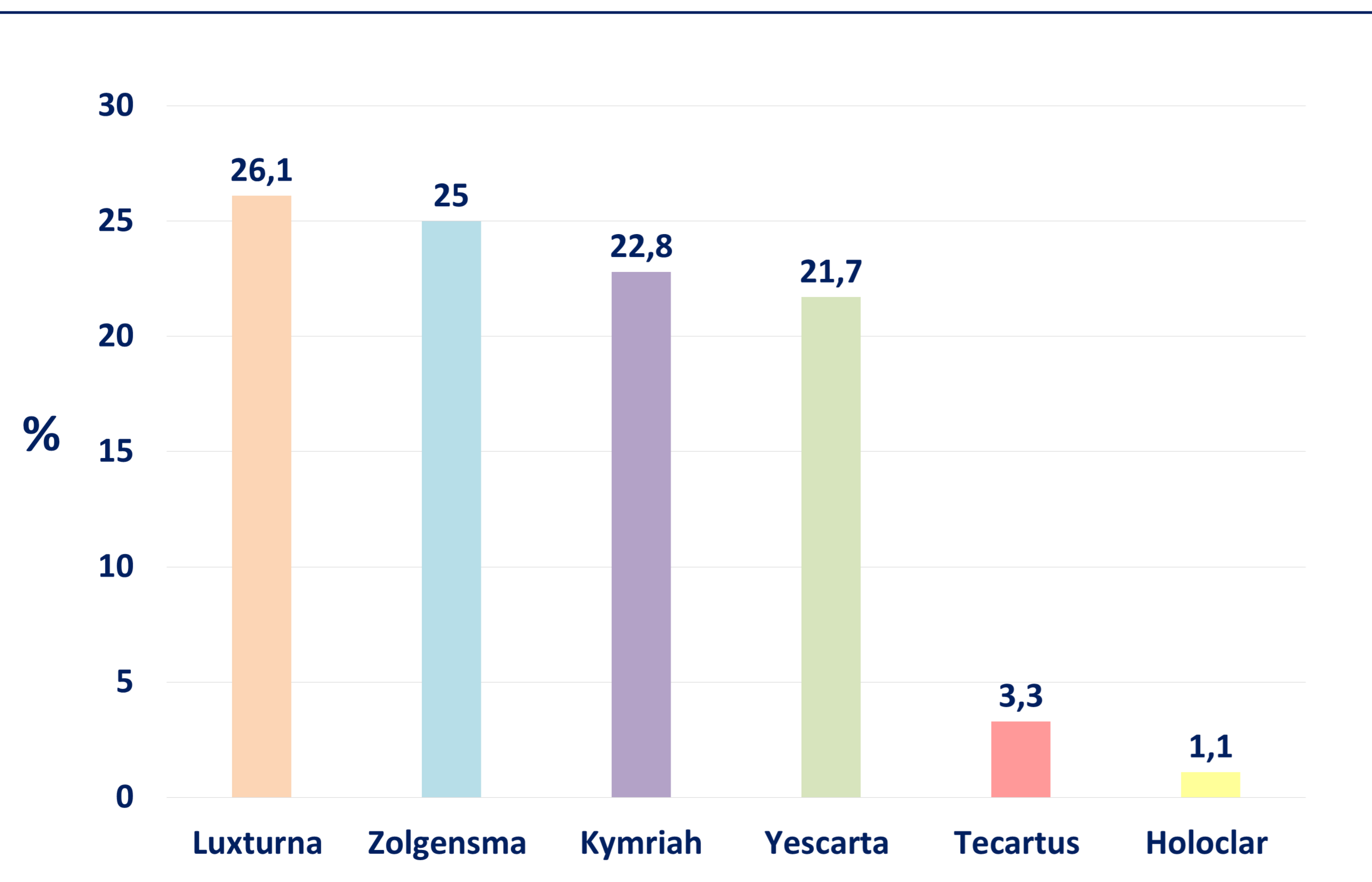


Table 1. Drug utilization profiles of Advanced therapy medicinal products

Drug (Diagnosis)	Regular end of follow-up	Significant epithelial defects and/or corneal neovascularization	Patient death
Overall	80 (87.0)	1 (1.1)	11 (12.0)
Luxturna (<i>Inherited retina dystrophie - Rpe65</i>)	24 (100)	-	-
Zolgensma (<i>Spinal muscular atrophy - SMA</i>)	23 (100)	-	-
Kymriah	15 (71.4)	-	6 (28.6)
<i>Non-Hodgkin Large B-Cell Lymphoma (LNH)</i>	13 (72.2)	-	5 (27.8)
<i>Acute lymphoblastic leukaemia (LLA)</i>	2 (66.7)	-	1 (33.3)
Yescarta (<i>Diffuse Large B-Cell Lymphoma - DLBCL</i>)	15 (75.0)	-	5 (25.0)
Tecartus (<i>Mantle cell Lymphoma - MCL</i>)	3 (100)	-	-
Holoclzar (<i>Limbal Ster Cell Deiciency - LSCD</i>)	-	1 (100)	-

CONCLUSION AND RELEVANCE



This study highlights the **low occurrence of adverse events and mortality associated with ATMPs**, emphasizing their potential as a **promising frontier for treating severe diseases** lacking therapeutic alternatives in real-world scenarios.

