



USE OF ORPHAN DRUGS IN A SMALL COUNTRY: A 14-YEAR REVIEW (2011-2024). 4CPS-008



E. GEA , E. GIL, M. AVELLANET.

UNIVERSITAT D'ANDORRA, HEALTH SCIENCE RESEARCH GROUP, SANT JULIA DE LORIA, ANDORRA. SERVEI ANDORRA D'ATENCIÓ SANITÀRIA

Background

Since 2012, an annual registry of orphan drug (OD) use in patients with rare diseases (RD) has been maintained. ODs are essential for treating RDs, but their utilization, and economic impact represent a challenge for healthcare systems.

OBJECTIVE

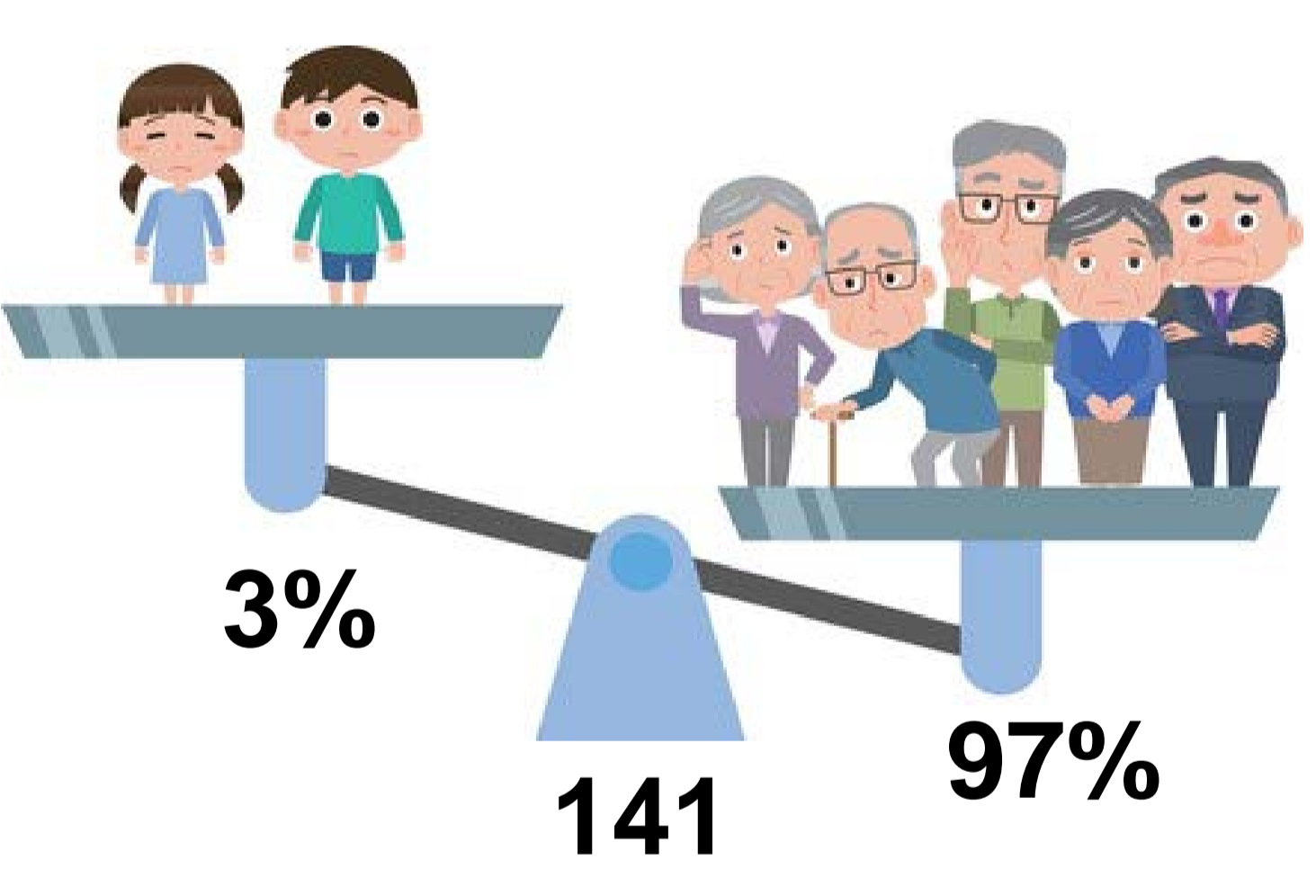
To assess the prevalence and evolution of OD use over a 14-year period and its economic impact on the hospital pharmacy budget (HPB).

MATERIAL AND METHODS

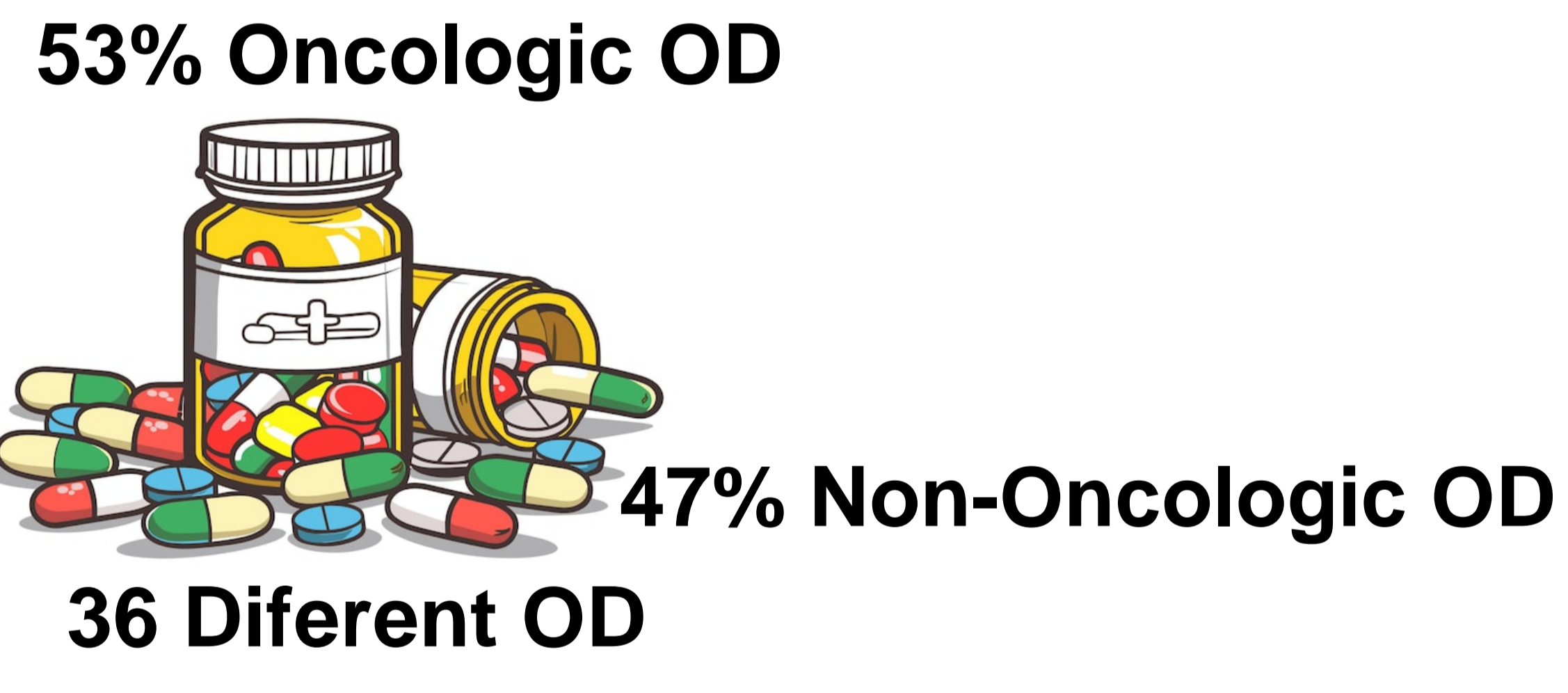
<p>1. STUDY</p> <p>Retrospective, observational, longitudinal.</p> <p>January 2011 to December 2024.</p> <p>Andorra's single public hospital.</p>	<p>2. CRITERIA</p> <p>Inclusion: adult and pediatric patients diagnosed with RD and treated with OD, both oncologic. and non-oncologic.</p> <p>Exclusion: CAR-T therapies.</p>	<p>3. DATA</p> <p>Data were extracted from the oficial inpatient and outpatient pharmacy software (Farmatools).</p> <p>Use OD classification validated ORPHANET (Update: April 2024).</p>	<p>4. DRUG PRICES</p> <p>Drug prices were based on those published by the national health authorities.</p>	<p>5. VARIABLES ANALYZED</p> <p>Number and type of OD (ATC classif.)</p> <p>Number of patients.</p> <p>Drug utilization.</p> <p>OD-related costs as a percentage of HPB.</p>
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RESULTS

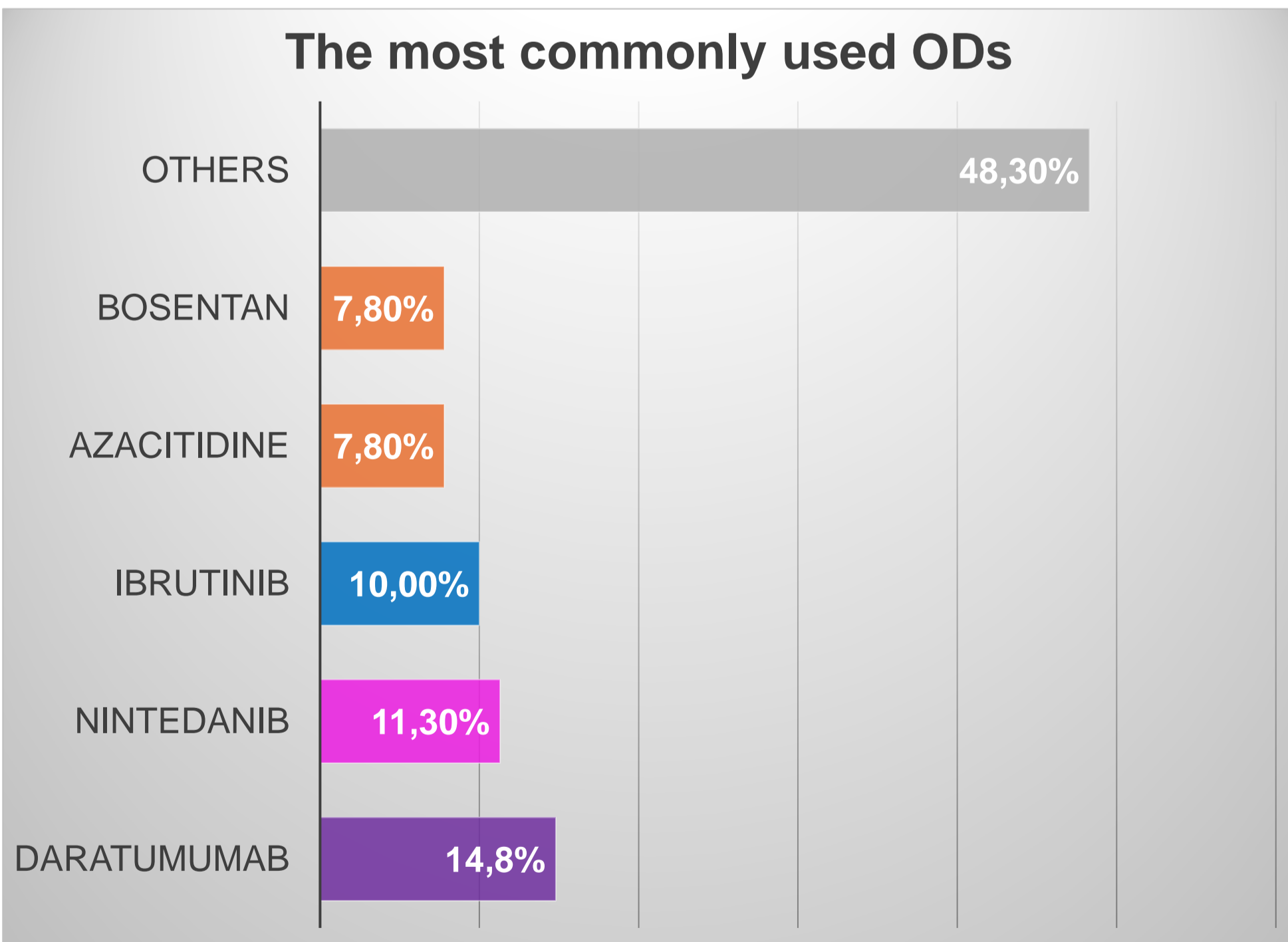
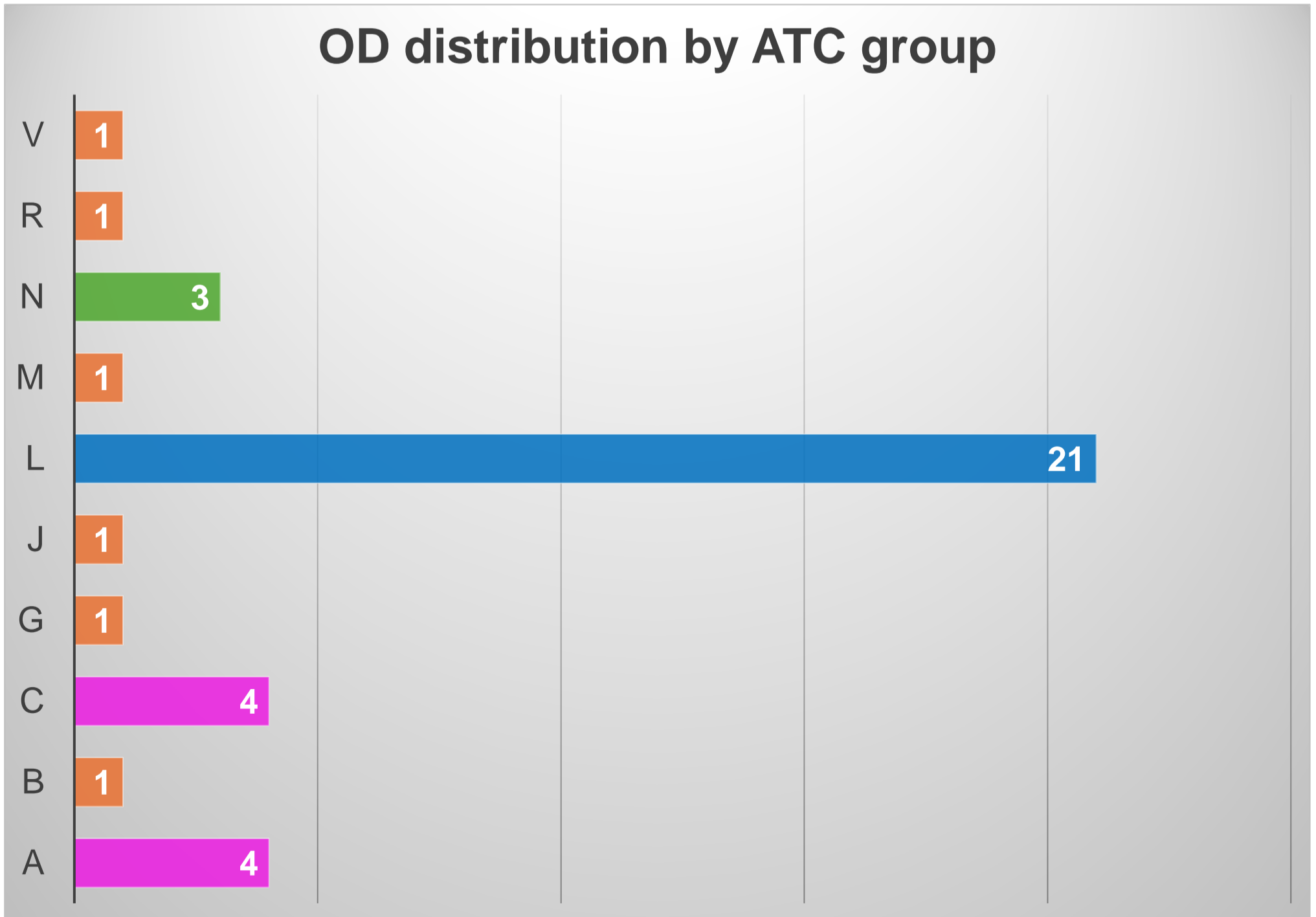
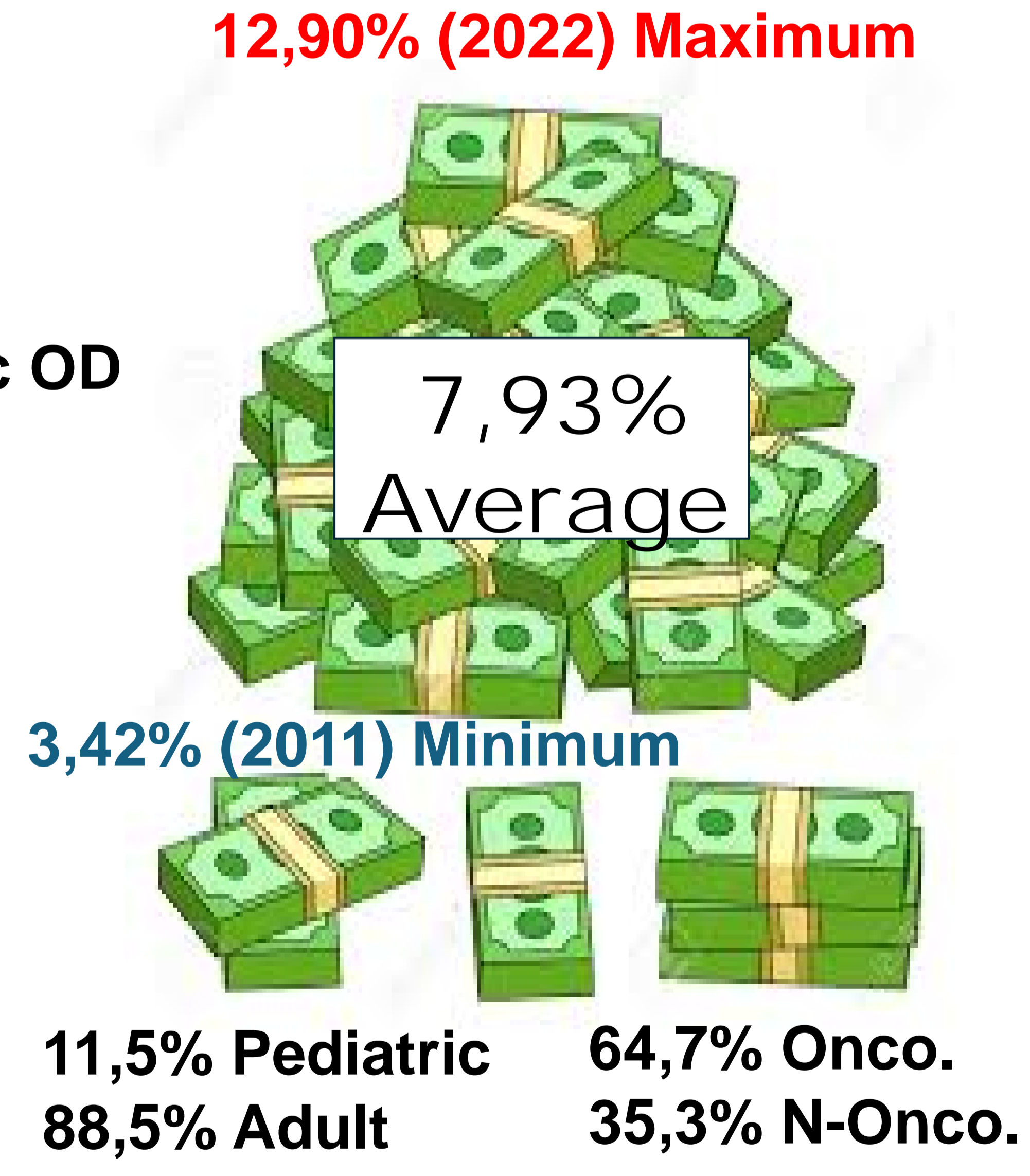
Population sample



Orphan Drugs Distribution



Economic impact



CONCLUSIONS:

This long –term project reflects the University of Andorra's commitment to improving care and visibility for rare diseases.

<p>Oncologic OD represented 53% of treatments and accounted for 64,7% of OD-related.</p>	<p>Daratumumab was the most used and costliest OD.</p>	<p>Bosentan was the most frequently used non-oncologic OD.</p>	<p>The average OD budget impact over 14 years was 7,8%.</p>
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