Background and importance In Italy, off-label (OL) drugs are regulated by 94/98 law: medication is used according to a therapeutic indication, dosage, frequency of administration, duration or route of administration different from approved indications.

Aim and objectives The purpose of this study was to evaluate the frequency of OL prescriptions, duration of treatment, effectiveness and the economic impact of this treatments in a large tertiary hospital.

Material and methods A retrospective analysis was conducted on authorised OL applications received from January to December 2018. We included only OL managed by the pharmacy’s antiblastic unit (UFA). Clinical data were collected from the hospital prescription database ‘Farmasase’ (drug, indication, department, duration of treatment and cost). Data were followed-up until September 2019 to ensure the justified maintenance of OL in terms of effectiveness and cost. We considered total effectiveness (healings), partial effectiveness (arrested pathology) or not assessable (drug was not given, treatment not completed for progression, toxicity or never started treatment).

Results During 2018, the UFA received a total of 56 OL authorised requests. The departments were: haematology (35%), nephrology (26.3%), oncology (12.2%), ophthalmology (8.7%) and other (12%). The most prescribed drugs were: rituximab (35%), cyclophosphamide (26.7%), aciclovir (25%), azathioprine (25.3%) and other (12%). The most prescribed drugs were: rituximab (35%), cyclophosphamide (5.3%), decitabine (12%), and other (12%).

Treatment for humoral rejection of kidney transplantation (26.7%), acute myeloid leukaemia in allogeneic post-transplant relapse (16%), Hodgkin’s lymphoma (8.9%), glaucoma (7%), others such as CA metastatic breast and LNH with T cells (5.3%) were the most represented OL indications.

The total hospital cost was estimated at €263,378.00, against a hypothesis of €302,843.00. The prescriptions with the most economic impact per cycle were brentuximab vedotinib (€13,232) and pembrolizumab (€5656). The prescriptions with the lowest economic impact were cyclophosphamide (€11,792), mitomycin (€290) and benda- mustin (€500).

For all 56 patients, 67% were totally effective, 19% were partially effective and 14% were not assessable.

Conclusion and relevance The use of OL had a strong ethical value and the pharmacist has an important role to uphold the national law, to consider the appropriateness of prescriptions and to correct allocation of resources. The OL treatments were effective in most patients and were justified on economic grounds and provided a benefit for patients with few therapeutic options.