



N.Monti Guarnieri¹, G.B.Ortenzi¹, E.Andresciani¹, A.M.F.Garzone¹, M.C.Ieracitano¹, C.Polidori², A.Pompilio¹

¹SOD Farmacia, AOU Ospedali Riuniti di Ancona, Marche Region, Italy

²Dipartimento di Medicina Sperimentale, Università degli Studi di Camerino, Marche Region, Italy



BACKGROUND AND AIMS: *Off-label* use of a medical product is the intentional use of drugs for any indication, population, dosage, administration route or treatment duration other than that approved by Country's Regulatory Authority. This practice is very common for Rare Disease due to the limited number of Orphan Drugs designed. It was estimated that only 5% of patients with rare disease received a specific authorized treatment. The aim of this work is to investigate how many orphan *off-label* drugs were authorized each year from January 2018 to December 2020 and to analyze what was the most used orphan *off-label* drug in our Hospital, the main center for Rare Disease's Diagnosis and Research in Marche Region.

METHODS: Starting from the *off-label* drugs authorized from January 2018 to December 2020 by our Hospital Ethic Committee (composed with a doctor, a Pharmacologist and a Hospital Pharmacist) in accord to Law 94/98, we created a database where we reported name of patients, drugs and disease (Figure 1). Then we identified which diseases were considered rare using Orphanet.com and Malattierare.gov. Finally we calculated the percentage of orphan *off-label* drugs by the total of the authorized expressed by year. We also investigated what was the most used orphan *off-label* drug in these three years and for which conditions it was prescribed.(Figure 2).

Patient's name	Patient's surname	Drug	Disease	Rare disease
Mario	Rossi	Intravenous Immunoglobulin	Systemic Sclerosis	Yes
Luca	Verdi	Omalizumab	Allergic Asthma	No

Figure 1

RESULTS: The number of orphan *off-label* drugs by the total of the authorized, expressed by year, and its percentage is represented in the following figure (Figure 3):

- 2018: of to 111 *off-label* drugs, 92 (82,88%) were orphan;
- 2019: of to 125 *off-label* drugs, 105 (84%) were orphan;
- 2020: of to 103 *off-label* drugs, 95 (92,23%) were orphan.

We noticed an increase of orphan *off-label* drugs of 9,35% from 2018 to 2020.

Intravenous Immonoglobulines resulted the most used orphan *off-label* drug in these years (prevalence=17,40%) to treat conditions like Dermatopolymyositis, Polymyositis, Giant-Cell Hepatitis with autoimmune hemolytic anemia, Systemic Sclerosis, Idiopathic Inflammatory Miopatya and others.

We created a database where we reported all *off-label* drugs authorized in our Hospital from January 2018 to Dicember 2020. For each drug we annotated name of patient and disease.



We identified which of these conditions were considered rare using Orphanet.com and Malattierare.gov



We calculated the percentage of orphan *off-label* drugs by the total expressed by year.



We investigated what was the most used orphan *off-label* drug in those three years and for which conditions it was prescribed.

Figure 2

CONCLUSIONS: Orphan *off-label* drug prescription is a very common practice but not underpinned by clinical studies to confirm efficacy and safety. During these years we noticed and increase of 9,35% of this procedure and Intravenous Immunoglobulines resulted the most used orpahn *off-label* drug. With our work we would like to highlight how is important research new orphan drugs to reduce risks associated with *off-label* use.

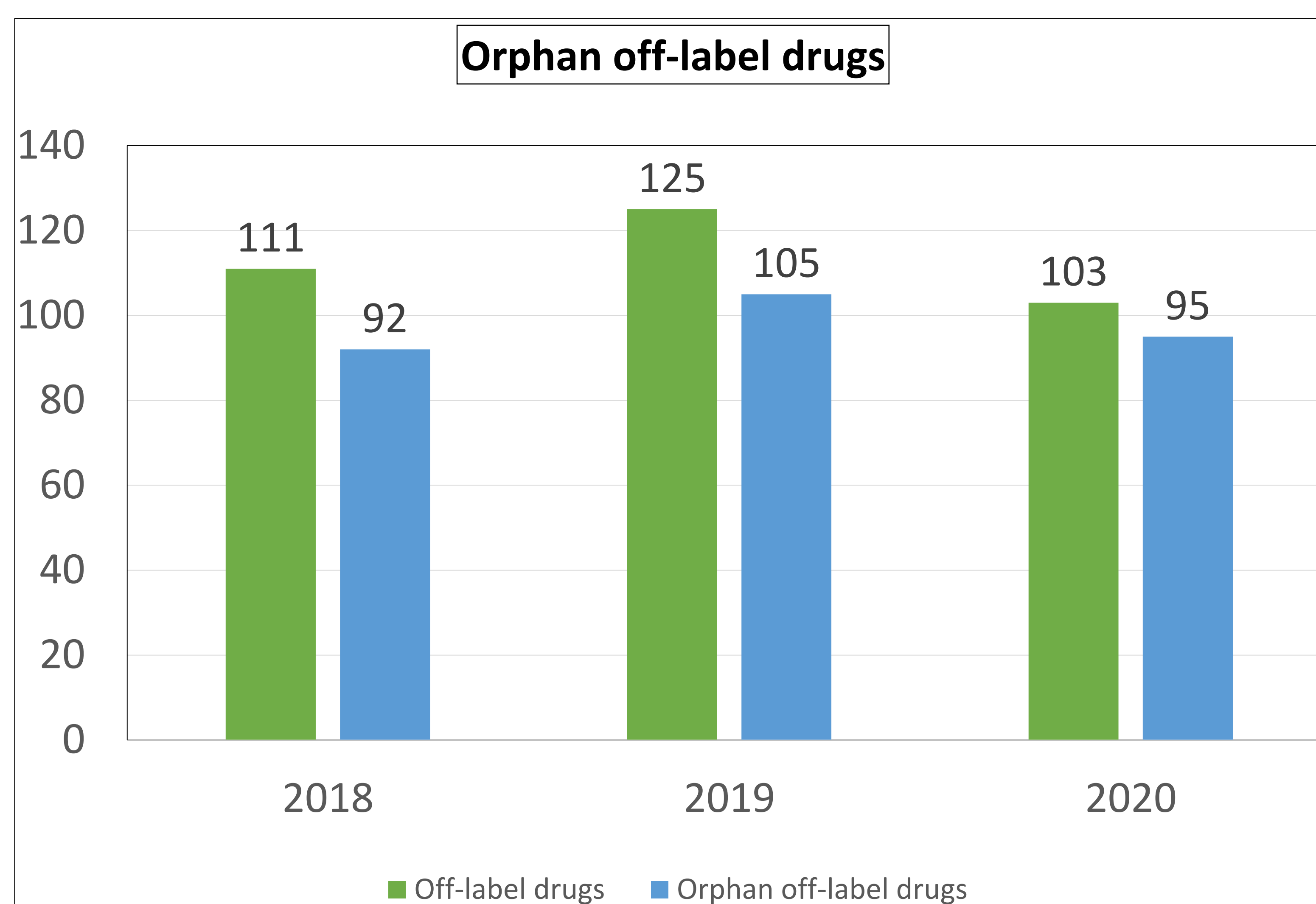


Figure 3

REFERENCES: nicoletta.montiguarnieri@ospedaliriuniti.marche.it