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).

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 Immunoglobulines 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 Miopathy and others.

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 orphan 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.

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