MONITORIZATION OF OFF-LABEL USE OF MONOCLONAL ANTIBODIES

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1. Background and Importance

The off-label use of monoclonal antibody (mAb) therapy is sometimes the only alternative when there are no official indication approved drugs regarding the disease or when the approved ones are ineffective or patients experience drug adverse events to the approved ones.

2. Aim and objectives

To identify efficacy and safety of mAbs off-label use

3. Materials and Methods

Retrospective observational study was conducted in adult patients with mAbs off-label prescription, at hospital setting, except Oncology Department, approved by the Local Commission of Pharmacy and Therapeutics (CPT) between January 2021 and December 2022. Information on efficacy and safety was extracted from CPT files and Soarian Clinics clinical files, and analyzed using Microsoft Excel 5.0 software. The treatment was considered effective when it was referred in the clinical files and/or when the administration of mAbs was maintained for at least 6 months. The study was authorized by local hospital Ethical Committee.

4. Results

69 patients were included, with a mean age of 51 years, mostly women (62%) and followed in Immuno-mediated Diseases Consultation (52%). The mAbs mostly used off-label were Rituximab (RTX) (65%) in the following indications Glomerulopathy, Purpura idiopathic thrombocytopenia (PTI) and Systemic Lupus Erythematosus; Infliximab (IFX) (15%) in Sarcoidosis and Tocilizumab (TCZ) (10%) in severe chronic urticaria (SCU). In 90% of patients, off-label treatment was effective, in 3 cases it was only partially effective and in 2 cases it was ineffective. In patients treated with Omalizumab (OMA) and TCZ, the treatment was 100% effective (n=3 and n=7), in those treated with RTX, the rate of effectiveness was 87% and in the case of IFX 80% was effective. In the cases of partial response (n=3), the mAbs involved were Natalizumab for advanced fulminant multiple sclerosis and RTX (n=2) for PTI and optical neuromyelitis. Of the patients unresponsive to treatment, one was treated with RTX and the other with IFX.

Regarding safety, 49% of patients had intercurrences. Of these, 12% of the patients suffered infections, 7% worsening of tiredness, 6% thrush blisters and 2% non-severe allergic reaction at the injection site of administration. Of the uneventful patients, mostly were treated with RTX in the Neurology department and with OMA in the Immunoallergology department. Of the patients included in the study, two died during treatment due to severe pneumonia.

5. Conclusions and Relevance

The results showed high rates of efficacy to off-label treatment with mAbs, in line with other studies, as well as the minor and major complications found, namely infections (1). Additionally, autoimmune disease itself may be a risk factor for infection and further studies are needed to ascertain causal relationships.

6. References and Acknowledgements

1.PROFT Fabian et al– Safety and efficacy of off-label use of biologic therapies in patients with inflammatory rheumatic diseases refractory to standard of care therapy: Data from a nationwide German registry (Graid2). In Z Rheumatol: Springerlink, 2018 Feb; 77(1): p. 28-39.

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