5PSQ-108 EFFICACY AND SAFETY OF INFLIXIMAB IN NF-KB ESSENTIAL MODULATOR DELETED EXON 5 AUTO-INFLAMMATORY SYNDROME: A CASE REPORT

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Background and Importance The NF-KB essential modulator deleted exon 5 auto-inflammatory syndrome (NEMO-NDAS) is an X-linked auto-inflammatory disease belonging to the systemic auto-inflammatory diseases (SAIDs). NEMO-NDAS affects the skin (ectodermal dysplasia) and the immune system. A few cases have been reported in France.

Aim and Objectives The objective of this case report was to describe the use of infliximab and its safety in NEMO-NDAS.

Material and Methods We report a 9-month-old baby who initially presented a long-lasting fever and a panniculitis. No infectious nor autoimmune causes were found, and the interferon signature was low. A corticosteroid treatment was started. Further genetic analyses showed an anomaly of the NEMO gene compatible with a NEMO-NDAS. Several pathways are modified, including the interferon pathway, which was increased. No recommendations nor relevant literature for specific treatment was found.

Results Anti-TNF-alpha such as adalimumab or infliximab could be used to down regulate this interferon pathway. Infliximab was introduced at a dose of 5mg/kg every 15 days for a month and a half, then every month. After the first injection, no fever, infection nor cutaneous manifestation were reported by the parents. The patient seemed to suffer less. Following the second injection, the corticosteroid treatment was decreased and stopped over a 15-day period.

One month after the introduction of infliximab, the patient presented a total apyrexia and no clinical signs of infection. On clinical examination, a hypertrophy of the lymphatic system was found (bilateral painless mobile axillary adenopathies, anterior cervical and supra-clavicular adenopathies). In spite of this, the patient was considered to be in clinical and biological remission (C-reactive protein = 1 mg/L, sedimentation rate < 2 mm in the first hour, amyloid A serum < 6,4 mg/L, transcriptomic signature of negative interferon gamma). Infliximab is currently being continued.

Conclusion and Relevance Infliximab was used successfully in our case and led to remission in 1 month with good tolerance and no adverse effect. Infliximab seems to be a well-tolerated treatment option for NEMO-NDAS in infants.

Introduction of infliximab allowed a total remission in 1 month without any adverse effect on the patient.

REFERENCES AND/OR ACKNOWLEDGEMENTS

Conflict of Interest No conflict of interest.

5PSQ-109 HAEMOASSIST: A DIGITAL BRIDGE BETWEEN HAEMOPHILIA PATIENTS AND PHARMACISTS

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Background and Importance Patients with severe haemophilia will need regular parenteral treatment throughout their lives to restore their haemostasis.

These patients reach a high degree of autonomy and their follow-up can be a challenge for healthcare professionals.

In 2020 our Pharmacy Service (PS) offered a mobile application (Haemoassist®) to 315 patients so that they could record their pharmacological administrations, specifying whether for prophylactic purposes or to treat active bleeding.

Aim and Objectives Compare app usage data obtained in 2022, with the data published in 2020, to know if we are achieving:

- Increase the number of patients using the app.
- Improve the quality of the data entered in the app.

Material and Methods

- Count the number of patients who used the app in 2022.
- We studied the degree of concordance between the adherence offered by the app (reported administered doses/prescribed doses) and that calculated from the PS (dispensed doses/prescribed doses).
- Check whether all patients who, according to the data collected in the hospital's medical record, had bled and were using the app, had reported these bleeds in the app.

We compared these 2022 data with those published in 2020.

Results 190 patients used the app on some occasion during 2022 compared to 169 patients in 2020.

In 2022, the median adherence achieved by the 190 patients, according to the app, was 8% and the Interquartile Range (IR):0–57% and according to the SF dispensations was 92% (IR: 77 -99%). The degree of concordance between the two calculation methods was 18%. In 2020, concordance was 9%.

Of the 190 patients using the app in 2022, according to the hospital's medical records, 153 of them had a bleeding episode, but only 74 reported their bleeds in the app. The 48% of patients reported their bleeds in the app in 2022 versus 54% in 2020.

Conclusion and Relevance The number of patients using the app has been increasing. The quality of patient-reported data is slowly improving.

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5PSQ-110 DESENSITISATION TO MONOCLONAL ANTIBODIES IN ONCOHAEMATOLOGICAL PATIENTS

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Background and Importance The increased use of monoclonal antibodies (mAb) for cancer treatment has been associated with a higher incidence of hypersensitivity reactions (HR). Drug desensitisation is a procedure that, by inducing temporary tolerance, allows patients who have developed a drug HR to safely receive it. This technique is performed according to previously published studies and plays a significant role for patients with HR, enabling treatment continuation.
Aim and Objectives To conduct a descriptive analysis of the use of mAb as a desensitisation protocol and to evaluate their effectiveness in a series of cases.
Material and Methods All oncological-haematological patients, who underwent desensitisation using a 3-concentration protocol due to HR to mAb in a University Hospital between 2019 and 2022, were included. Clinical information was retrospectively collected from medical records (SAP®, Genomi®), including oncohaematologic cancer type, mAb desensitised, time and severity of the reaction, allergology study results (skin test and/or Basophil Activation Test (BAT)), suspected underlying mechanism (Inmunoglobulin E (IgE) mediated or non-IgE mediated), breakthrough reactions during any of the desensitisation and final outcome.
Results Thirty-six patients received mAb desensitisation regimens, with a total of 357 desensitisations of eight different drugs [rituximab (123), cetuximab (87), daratumumab (68), trastuzumab (45), brentuximab (13), Obinutuzumab (9), isatuximab (9), trastuzumab entamsine (3)]. Each patient received an average of 10 administrations (1–52) in desensitisation regimen. Twenty-eight patients had haematological pathologies (77%), most of them treated with rituximab. Seventeen out of 36 (47%) patients desensitised experienced a reaction at first contact with the drug. Half of all patients (18) suffered moderate to severe HR; and only five patients had a confirmed IgE-mediated HR, confirmed by skin tests or BAT. 86% of the patients did not experience any reaction (breakthrough reactions) during the desensitisation. The remaining experienced some mild reaction during at least one of the desensitisations, but after adjusting the infusion regimen they tolerated treatment adequately. All (100%) of the desensitisations were successful; patients were able to receive the medication they were being treated without experiencing any adverse reactions that require discontinuation.
Conclusion and Relevance The high success of desensitisations to mAb in our hospital highlights the importance of this technique preventing switching to other treatments that might be more expensive and less effective.

REFERENCES AND/OR ACKNOWLEDGEMENTS

Conflict of Interest No conflict of interest.

5PSQ-111 SODIUM-GLUCOSE COTRANSPORTER 2 INHIBITORS AFTER HEART TRANSPLANTATION

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Background and Importance Sodium-glucose cotransporter 2 inhibitors (SGTL2i) are widely used to manage diabetes mellitus (DM) and heart failure (HF). Recently, safety studies have been published on their use in renal recipients, however, no evidence exists in heart transplant recipients (HTR).
Aim and Objectives To evaluate safety, tolerability and effectiveness of SGTL2i in HTR.
Material and Methods Retrospective descriptive cohort study conducted in a tertiary hospital. All adults undergoing heart transplantation (HT) from January 2016 to July 2023 treated with SGLT2i were included. Demographic, clinical and pharmacological data were recorded. Outcome measures: Body Mass Index (BMI) and HbA1c evolution, number of hospitalisations in patients with HF and adverse events (AE).
Results Among 154 HTR, 28 patients were on SGLT2i, 21.4% women, 62.1 [50.9 – 63.4] years old), 9 (32.1%) with dapagliflozin and 19 (67.9%) with empagliflozin.

SGLT2i indication were: 75% DM, 21% HF and 4% DM +HF. A total of 22 (78.6%) patients were DM, 81,8% of whom were on a combined antihyperglycemic therapy. Seven (25%) patients developed DM after HT. Median time from HT to SGTL2i initiation was 20 [4–40] months.

Three patients (10.7%) reported AE while on SGLT2i: two suffered urinary tract infections and one cephalic instability. Moreover, two patients discontinued SGTL2i, one after 4 months due to intolerance and the other after 11 months because of HbA1c normalisation. At 6 months after initiation of ISGLT2, a reduction in HbA1c of 0.2 [-1,9 – 0.3] points was observed. It was also noted a reduction in BMI of 1.4 [-2,4 – 0,8] points. In patients with HF, no HF hospitalisations occurred after initiation.
Conclusion and Relevance Our results show that SGTL2i are well-tolerated in HTR. Although these data are consistent with findings in renal recipients[1], further investigation is needed.

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5PSQ-112 A SURVEY OF HOME STORAGE TEMPERATURE OF IN-USE INSULINS AND ANALYSIS OF THEIR STABILITIES UNDER THE SIMULATED HIGHEST HOME TEMPERATURE

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Background and Importance Insulins remain essential for people living with diabetes worldwide. As a biological product, it is susceptible to heat, light and sheer conditions. Little is known about actual household storage temperature of insulins, especially in the setting where room temperature is far beyond 25°C, under which insulin stability might be compromised.
Aim and Objectives To determine home storage temperature of in-use human insulins among ambulatory type 2 diabetes (T2D) people and to subsequently test insulin stability under the simulated maximum storage temperature identified.