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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