MULTIDISCIPLINARY COLLABORATION IN THE TREATMENT OF PEDIATRIC HEMATOPOIETIC TRANSPLANT REJECTION WITH ALLOGENEIC MESENCHYMAL CELLS. A CASE REPORT

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Background Advanced treatments represent a source of hope for rare diseases. However, they are complex as they require the participation of several professionals and experience is necessary for optimal use.

Purpose To describe the outcome and collaborative multidisciplinary process undertaken for the appropriate use of allogeneic mesenchymal cells (AMC) in the treatment of graft versus host disease (GVHD) developed by a paediatric patient after hematopoietic stem cell transplantation.

Materials and Methods Retrospective study of clinical outcomes, steps taken and requirements for the preparation of AMC (Prochymal). The case involved a 2-year-old paediatric patient with steroid-refractory severe GVHD with severe gastrointestinal manifestations. The treatment involved the administration of two doses per week for a total period of 4 weeks. If the patient responds completely or not at all, the treatment is completed, if there is a partial response the treatment can be completed plus an additional weekly dose for 4 extra weeks.

Results There was cooperation between the Paediatrics, Haematology and Pharmacy Services. A protocol was developed for use based on the instructions provided by the supplier. Pharmacy processed the application as a compassionate use (expanded access clinical trial) with the agreement of the supplier and hospital management. Haematology built on its expertise in handling blood cells to ensure storage (−135°C) and initially collaborated with Pharmacy in the preparation of the doses: controlled defrosting, bottling and packaging in aseptic conditions. The treatment resulted in a partial response at completion so an additional cycle was administered. No adverse reactions to AMC were observed.

Conclusions Interdisciplinary collaboration through the optimization of hospital resources and the rapid training of participating staff allowed the administration of a new and urgent treatment of advanced treatment, allogeneic mesenchymal cells. Tolerance was good and the response to treatment was initially favourable.

No conflict of interest.

NATALIZUMAB IN CYPRiot PATIENTS WITH RELAPSING REMitting MULTIPLE SCLEROSIS: THREE YEAR DATA ON SAFETY, EFFICACY AND FREQUENCY OF ANti-JC VIRUS ANTIBIodies

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Background Natalizumab (NAT) is a recombinant humanised anti-α4-integrin antibody used in treating Relapsing Remitting (RR) Multiple Sclerosis (MS).

Purpose To evaluate the long-term safety and efficacy of NAT in Cypriot patients, to assess the frequency of anti-JCV Virus (JCV) antibodies and implement a strategy for the prevention of PML.

Materials and Methods Twenty-two patients were studied prospectively for 3 years.

The patients received 300 mg of NAT intravenously every 4 weeks. MRI examinations were performed at study entry and 12–24 months after the start of treatment. JCV antibody testing was performed after two years of treatment.

Results Six patients (27.3%) discontinued the study due to: Severe allergic reaction (9%), generalised atony, fatigue and weakness (4%), recurring herpes infection (4%), family planning (4%) and presence of anti-JCV antibodies (anti-JCV positive) due to previous immunosuppressive therapy (4%).

Most frequently reported side effects were: cardiovascular (41%), general (41%), laboratory abnormalities (41%), gastrointestinal (23%), neurological (18%), allergic reactions (18%) and depression (14%).

After three years of NAT treatment, a 55.2% decrease from the baseline mean annual relapse rate was observed, as well as improvement of 0.3 points on the mean Expanded Disability Status Scale (EDSS) Score.