Material and methods We describe the case of a child affected by TRAPS and its pharmacotherapeutic management. Treatment options included oral glucocorticoids and biologic agents (etanercept, anakinra). Medical and pharmaceutical records were reviewed, and a bibliographic research was made to establish the state of the art treatment of TRAPS. UpToDate, Pubmed and the Cochrane Library were consulted, finding little information on this very rare medical condition.

Results Our patient was a 7 year old boy who presented with recurrent febrile episodes, accompanied by abdominal pain and periorbital eczema. There was no infectious focus. Laboratory data showed elevated inflammatory markers. The rheumatologist suspected an autoimmune syndrome rather than an autoinflammatory disease. Lack of autoantibodies and a genetic diagnosis confirmed TRAPS.

Initial treatment was oral prednisone, with a response similar to NSAIDs. Due to persistence of symptomatology, the clinician indicated etanercept, which achieved a partial response but had to be interrupted because of respiratory related sepsis. Afterwards, this biologic was reintroduced with low doses of prednisone. Over the following months the patient relapsed, and anakinra was prescribed instead of etanercept. Anakinra treatment showed satisfactory results, achieving symptomatology control and normalisation of laboratory parameters with no remarkable safety concerns.

Conclusion and relevance We have presented the case of a patient refractory to anti-TNF treatment who experienced dramatic improvement with the recombinant human IL-1 receptor antagonist anakinra. There are only a few cases published on this subject, and our experience supports the evidence that anakinra can be considered a firstline treatment for TRAPS due to its efficacy and lack of adverse reactions.

REFERENCES AND/OR ACKNOWLEDGEMENTS

No conflict of interest.

4CPS-131 EFFECTIVENESS OF NUSINERSEN IN PAEDIATRIC PATIENTS SMA1 AND SMA2

[1]GB Ortenzi*, [2]G Palmas, [1]E Andresciani, [1]AMF Garzone, [3]MA Berardi, [4]S Siliquini, [4]L Porfiri, [1]MS Demeo, [1]S Guglielmi, [3]C Polidori, [1]A Pompilio. *[1]AO Ospedali Riunti Di Ancona, Sod Farmacia, Ancona, Italy; [2]AO Ospedali Riuniti Ancona, Sod Clinica Pediatrica, Ancona, Italy; [3]Università Degli Studi Di Camerino, Scuola Di Scienze Del Farmaco E Dei Prodotti Della Salute' Facoltà Di Farmacia, Camerino, Italy; [4]AO Ospedaliriunti Ancona, Sod Neuropsichiatria Infantile, Ancona, Italy*

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Background and importance Nusinersen is an innovative drug given by intrathecal injection and used to treat 5q-spinal muscular atrophy (SMA), a severe neuromuscular disorder due to a defect in the survival motor neuron 1 (SMN1) gene. This antisense oligonucleotide drug modifies RNA splicing of the SMN2 gene, thus increasing the production of full length SMN protein. The first dose, given as soon as possible after the diagnosis, should be followed by three more doses after 2, 4 and 9 (L1, L2, L3, L4) weeks and one dose every 4 months (M1, M2, M3...) thereafter.

Aim and objectives This study aims to describe the efficacy of nusinersen in terms of improvements in motor function in paediatric patients with SMA1 and SMA2.

Material and methods From February 2018, we collected data from 8 patients, 3 with SMA 1 and 5 with SMA 2, using specific neuromuscular functional tests: CHOP-INTEND, HINE and HFMSE

Results Results are expressed as points of increase (p) in motor function scores from baseline (or from the first score recorded in our centre*) to the score obtained at the time of the last injection for each patient.

SMA1 patients:

2 months old at the time of first injection (TFI): CHOP-INTEND 8/64 to +38p (M2); HINE 0/26 to +5p (M2).

3.3 years old TFI*: CHOP-INTEND 18/64 (M2) to +16p (M6); HINE 2/26, stable at M6.

5.6 years old TFI*: CHOP-INTEND 1/64 (M2) to −1p (M4); HINE 0/26 (M2) to +1p (M4), then suspended for absence of efficacy.

SMA2 patients:

1.2 years old TFI: CHOP-INTEND 59/64 to −1p (M1).

3.4 years old TFI: CHOP-INTEND 41/64 to +8 (M1), +14p (M3); HFMSE 8/66 to stable at M1 +4p (M3).

4.6 years old TFI: CHOP-INTEND 55/64 to +6p (M1) +7 (M2); HFMSE 22/66 to +3p (M1), +3p (M3).

8.5 years old TFI: CHOP-INTEND 42/64 to +5p (M1); HFMSE 17/66 to +10p (M1).

11.5 years old TFI: CHOP-INTEND 37/64 to +2p (M1); HFMSE 8/66 stable at M1.

Conclusion and relevance Our results showed an average increase of 4 points for CHOP-INTEND and 3.75 points for HFMSE in SMA2 patients, after 6 months (M1) of treatment. For SMA1 patients, it was not possible to evaluate the average trend for CHOP-INTEND and HINE scores after 6 months of treatment because two patients started nusinersen in other hospitals (motor scores at L1–M1 not available). A longer follow-up and data from other parameters, such as swallowing and respiratory function, are important to better understand the overall efficacy of nusinersen.

REFERENCES AND/OR ACKNOWLEDGEMENTS

No conflict of interest.

4CPS-132 INDIRECT TREATMENT COMPARISON OF ANTI-CALCITONIN GENE RELATED PEPTIDE PATHWAY ANTIBODIES IN CHRONIC MIGRAINE

MDP Briceño Casado*, S Fenix-Caballero, MD Gil-Sierra, M Dominguez Cantero, EJ Alegre-Del Rey. *Hospital Universitario Puerto Real, Hospital Pharmacy, Cadiz, Spain*

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Background and importance Erenumab, fremanezumab, galcanezumab and eptinezumab are monoclonal antibodies targeting the calcitonin gene related peptide pathway (anti-CGRP), used as preventive treatment in chronic migraine (CM).

Aim and objectives To evaluate whether anti-CGRP drugs are equivalent therapeutic alternatives (ETA) in CM through an adjusted indirect treatment comparison (ITC).

Material and methods A bibliographic search of randomised clinical trials (RCTs) in Pubmed was performed (20 May 2019). Inclusion criteria: phase II/III RCTs of anti-CGPR with similar populations, follow-up duration and comparator treatments. CM was defined as ≥15 headache days/month, of which ≥8 were migraine days (event duration ≥4 hours). Exclusion criteria: RCTs with different clinical CM context and other CM definitions. Efficacy end point was ≥50% reduction in migraine days/month (measured from the beginning of treatment to 12 weeks). An ITC was developed using

Bucher's method. Delta value (Δ, maximum difference as a clinical criterion of equivalence) was calculated according to the ETA guide[1]: use was made of half of the absolute risk reduction (ARR) obtained in the meta-analysis of RCTs included in the ITC (pooled ARR=20%; Δ=10%).

Results

Six clinical trials were found erenumab (n=3), fremanezumab (n=2), glacanezumab (n=1) and eptinezumab (n=0). One study of erenumab[2] and another of fremanezumab[3] were selected. The rest were not included in the ITC (non-compliance with the inclusion criteria). Trials included were three arm (control and two different drug regimens), double blind, placebo controlled RCTs. Results of the ITC are shown in table 1.

Abstract 4CPS-132 Table 1

Reduction of ≥50% migraine days/month (ARR (95% CI))	Erenumab 70 mg	Erenumab 140 mg
Fremanezumab quarterly	3 (−7.56 to 13.56)	2 (−8.64 to 12.64)
Fremanezumab monthly	6 (−4.59 to 16.59)	5 (−5.66 to 15.66)

In all cases, there were no statistically significant differences; most 95% CI values were within the calculated delta margins.

Conclusion and relevance ITC showed no statistically significant differences in ≥50% reduction in migraine days/month between erenumab and fremanezumab. Probable clinical equivalence was found between erenumab and fremanezumab. These drugs could be considered ETA in CM. Further studies are necessary to include galcanezumab and eptinezumab in the ITC.

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No conflict of interest.

4CPS-133 IS PARACETAMOL A REAL ALTERNATIVE IN THE MANAGEMENT OF PATENT DUCTUS ARTERIOSUS IN PRETERM INFANTS?

[1]AA Garcia Robles*, [2]R Iglesias Gómez, [2]P Pérez Huertas, [3]A Parra Llorca, [3]B Torres García, [3]A Gimeno Navarro, [3]M Vento Torres, [2]JL Poveda Andrés, [3]M Aguar Carrascosa. *[1]Hospital Universitari I Politècnic La Fe, Pharmacy-Neonatology, Valencia, Spain; [2]Hospital Universitari I Politècnic La Fe, Pharmacy, Valencia, Spain; [3]Hospital Universitari I Politècnic La Fe, Neonatology, Valencia, Spain*

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Background and importance Patent ductus arteriosus (PDA) is a common cause of morbidity and mortality in preterm infants. The treatment of choice is ibuprofen but contraindications and serious adverse events limits its use. Paracetamol has been proposed as an alternative to ibuprofen, with good results (80–95% efficacy) and apparently less side effects.

Aim and objectives To analyse the effectiveness and safety of intravenous paracetamol in the treatment of haemodynamically significant PDA (hsPDA).

Material and methods A retrospective cohort study of hospitalised infants was conducted in a level III neonatal intensive care unit between July 2013 and January 2019. Criteria inclusion: gestational age (GA) ≤30 weeks and treatment of hsPDA with paracetamol 15 mg/kg/6 hours (minimum 8 doses) after contraindication or ineffectiveness of ibuprofen. Closure was considered if the ductus was <1 mm and not significant. The need for post-paracetamol treatment was also analysed.

Results Fifty-four patients were included, with a median GA of 26±1.8 weeks and median birth weight of 853±293 g. In 14 patients, paracetamol was used as the first option and in 40 after ibuprofen (table 1). The overall closure rate was 37%. No adverse effects were reported during treatment.

Conclusion and relevance Our efficacy results were much lower than those published in most studies and case series. In our series, the overall efficacy of paracetamol was 37.0% and 40.5% if deceased patients were excluded from the analysis.

Well designed clinical trials are needed to help decide the role of paracetamol in the management of hsPDA as the results are very different depending on whether it is administered as the first choice (50.0% or 71.4% excluding the deceased) or after ibuprofen (32.4% or 34.3% excluding the deceased).

Abstract 4CPS-133 Table 1

Paracetamol	First option	Second option after ibuprofen			Overall
Reason	Contraindication	Overall	Ineffectiveness	Contraindication	All
Patients (n)	14	40	18	22	54
Closure (n)	7	13	3	10	20
Reopen (n)	2	3	0	3	5
No additional treatment required (n)	0	6	6	0	6
Additional treatment required (n)	7	21	10	11	28
Surgery (n)	1	16	9	7	17
Died (n)	7	5	0	5	12
Closure rate (%)	50.0	32.5	16.7	45.5	37.0
Closure rate+no additional treatment (%)	50.0	47.5	50.0	45.5	48.1
Closure rate (excluding deceased patients) (%)	71.4	34.3	16.7	52.9	40.5