



MEETING ABSTRACT

Open Access

PW03-020 – A decade of ANTI-IL-1 therapy for CAPS in the UK

T Lane^{1*}, NM Stewart¹, A Bybee¹, DM Rowczenio¹, K Wynne², H Trojer¹, A Baginska¹, PA Brogan², PN Hawkins¹, HJ Lachmann¹

From 7th Congress of International Society of Systemic Auto-Inflammatory Diseases (ISSAID) Lausanne, Switzerland. 22-26 May 2013

Introduction

In October 2002, the first patient with CAPS was treated successfully with the anti-IL-1 agent anakinra at our Centre in the UK, and in 2009 a nationally funded canakinumab treatment service initiated for CAPS in England. By the end of 2012, 82 symptomatic individuals have been assessed at our Centre.

Objectives

To describe our experience, and outcomes of 82 individuals with clinical CAPS, including treatment and natural history.

Methods

We examined all available medical and laboratory records.

Results

Of 82 patients with clinical CAPS a pathogenic sequence variant (PSV) was detected in 77 (94%); 17 PSVs were identified, the commonest were R260W (27%), A439V (23%), T348M (17%), V198M (5%). 5 patients had no PSV detected on Sanger sequencing: 3 children with CINCA and 2 patients with adult onset clinical MWS. 90% were white, 8.5% South Asian and 1% African. 46 (56%) gave a positive family history. There were 8 CINCA cases, 4 MWS/CINCA overlap (75% T348M), 59 MWS (62% A439V, 31% R260W, 19% T348M, 5% V198M) and 11 FCAS (36% A439V, 27% R260W, 9% V198M). Hearing impairment was present in 31 (38%); 12/14 patients (86%) with T348M, 1/19 (5%) of A439V, 3/22 (14%) of R260W and 3/4 (75%) of V198M.

51 patients are receiving canakinumab (20 having converted from anakinra). Over a median follow up (FU)

period of 28 months (IQR 15-40), 43 (84%) have experienced complete remission (CR) of disease activity whilst the remainder (8, 16%) have experienced partial remission (PR), defined as good but incomplete resolution of symptoms or serum inflammatory markers. 15 (29%) patients are being treated with double the licensed dose – 4 CINCA (including 2 children), 4 MWS/CINCA overlap, 6MWS (including 2 children) and 1 FCAS with uveitis. 3 patients have discontinued canakinumab and are currently on no treatment (one each with A439V, T348M, R260W) due to: an episode of diverticulitis; pregnancy; a desire for a treatment break. Serious adverse events included infections (diverticulitis, UTI, tonsillitis).

24 patients are on anakinra and over a median FU of 47 months (IQR 12-72) 20 remain in CR and 4 in PR. 6 patients have previously tried canakinumab; 2 had CR but were converted due to: planned pregnancy; planned insertion of a ventricular peritoneal shunt. One adult CINCA patient with a good PR could not tolerate travel to our Centre. A female with mutation negative MWS previously in CR on anakinra opted for a trial of canakinumab, but developed a massive disease flare resulting in hospitalisation. She subsequently reverted to anakinra and is once again in CR. 2 males (A439V, T346I) discontinued canakinumab due to: lack of efficacy; development of major systemic inflammation and a morphea like rash. On anakinra the former remains in PR whilst the latter has experienced CR.

2 children with mild R260W disease have declined treatment.

Conclusion

In this series T348M underlies more severe disease than the other common mutations. A decade of IL-1 blockade confirms its efficacy and relative safety in CAPS across the clinical severity spectrum. Canakinumab is

¹National Amyloidosis Centre, University College London, UK
Full list of author information is available at the end of the article

the more popular drug due to its long action; however, a small number of patients are unresponsive to this therapy, suggesting a possible role of IL-1 α .

Disclosure of interest

None declared.

Authors' details

¹National Amyloidosis Centre, University College London, UK. ²Rheumatology and Vasculitis, Great Ormond Street Hospital NHS Trust, London, UK.

Published: 8 November 2013

doi:10.1186/1546-0096-11-S1-A246

Cite this article as: Lane *et al.*: PW03-020 – A decade of ANTI-IL-1 therapy for CAPS in the UK. *Pediatric Rheumatology* 2013 11(Suppl 1): A246.

**Submit your next manuscript to BioMed Central
and take full advantage of:**

- Convenient online submission
- Thorough peer review
- No space constraints or color figure charges
- Immediate publication on acceptance
- Inclusion in PubMed, CAS, Scopus and Google Scholar
- Research which is freely available for redistribution

Submit your manuscript at
www.biomedcentral.com/submit

