PEDIATRIC RHEUMATOLOGY

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Long-term safety and efficacy of Canakinumab in cryopyrin-associated periodic syndrome (CAPS) patients: results from beta-confident registry

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Background

CAPS encompasses a spectrum of three phenotypes: familial cold auto-inflammatory syndrome (FCAS), Muckle-Wells syndrome (MWS), and chronic infantile neurologic cutaneous and articular syndrome/neonatal onset multisystem inflammatory disease (CINCA/NOMID)[1]. The β-Confident Registry, the largest CAPS cohort documented in a registry, enrolled the last patient in December 2014. Here, we report interim data for the complete cohort of enrolled patients.

Objectives

To monitor the overall safety of canakinumab (CAN) focusing on SAEs including serious infections, vertigo, malignancies, and hypersensitivity reactions.

Patients and methods

The registry protocol does not mandate any visits or procedures, but records all observed and reported AEs and SAEs or AEs potentially CAN-related. Cumulative safety data are reported as incidence rate per 100 patient-years (IR/100 pyr). Data is partial for 11 patients due to the cut-off date for the analysis and will be updated at a later date. Efficacy was measured using physician global assessments (PGA).

Results

288 patients were enrolled with a mean±SD duration of 193±72 weeks. Of these, 21 (7.3%) patients discontinued CAN: 5 each due to AE, poor efficacy and patient preference; and 6 due to unknown reasons. The IR/100 pyr for overall AEs was 100.0. FCAS patients had the lowest AE IR/100 pyr (60.9) compared with MWS (IR/100 pyr 107.2) and NOMID (IR/100 pyr 120.3) patients. The most common types of AEs were infections and infestations (IR/100 pyr 36.7). Vertigo was reported by 19 patients (IR/100 pyr 3.7). 117 SAEs were reported by 62 patients (IR/100 pyr 15.0), with infection being the most common (IR/100 pyr 4.1). One death (metastatic rectal adenocarcinoma in 76 yr old MWS patient) was reported. Of 18 patients receiving pneumococcal vaccinations (PPV), 13 (72%) reported a local post-PPV injection site reaction, of which 5 were considered as serious. Based on PGA, nearly half the patients had no disease activity while most others had mild/moderate disease activity. Similarly, disease activity was mostly absent in *NLRP3* mutation negative CAPS patients (n=14) treated with CAN. There was no evidence of loss of effect with time. Further analyses of this cohort are ongoing.

Conclusions

Canakinumab demonstrated a safety profile consistent with that observed in the clinical trial program and provided continued effectiveness in CAPS patients for up to 5 years. Canakinumab therapy was also effective in *NLRP3* mutation negative CAPS patients.

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