Pediatric Rheumatology



Poster presentation



# Efficacy, safety and effect on gene expression profiling of anakinra in systemic-onset juvenile idiopathic arthritis: final results of a randomised, double-blind, placebo-controlled trial (ANAJIS)

P Quartier*[1], F Allantaz[2], R Cimaz[3], P Pillet[4], O Richer[4], M Desjonqueres[3], A Duquesne[3], C Messiaen[1], C Bardin[5], X Bossuyit[6], A Boutten[7], J Bienvenu[8], V Menoni[1], S Sotou-Bere[1], B Neven[1], N Mahloui[1], A Mogenet[1], B Kassai[3], D Chaussabel[2], JM Treluyer[1], JL Bresson[1], P Landais[1] and V Pascual[2]

Address: [1]Necker-Enfants Malades, Assistance Publique Hopitaux de Paris, Paris, France, [2]Baylor Institude, Dallas, USA, [3]Edouard Herriot, Lyon, France, [4]Pellegrin, Bordeaux, France, [5]Hotel Dieu, Paris, France, [6]University of Leuwen, Leuwen, Belgium, [7]Bichat, Assistance Publique Hopitaux de Paris, Paris, France and [8]Laboratoire d'Immunologie Lyon sud, Lyon, France

* Corresponding author

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## Purpose

To investigate efficacy and safety of anakinra in systemic-onset juvenile idiopathic arthritis patients. To assess treatment effect on gene expression profiling, immune response to anti-pneumococcal Pneumo23 vaccine, serum amyloid A level, serum ferritin level and the percentage of glycosylated ferritin.

## Methods

Multicenter randomized double-blind trial. The primary objective was to compare the efficacy of a one-month treatment with anakinra to a placebo between 2 groups of 12 patients each. Response was defined by 30% improvement of pediatric ACR core-set criteria for JIA, resolution of fever and systemic symptoms and normalization or a decrease of at least 50% of both CRP and first hour ESR compared to baseline. Intention-to-treat analysis. Secondary objectives included tolerance and efficacy assessment over 12 months and treatment effect on blood gene expression profiling.

## Results

At one month, there was a significant difference in the response rate between patients treated with anakinra (8/12) and placebo (1/12). During the double-blind phase, the number of adverse events, mainly pain to injections, was similar between both groups. Ten patients from the placebo group switched to anakinra at Month 1 and 9 were responders at month 2. Eight patients discontinued anakinra before Month 12: painful injections during the double-blind phase (2 patients, both on placebo), ileocolic symptoms leading to the diagnosis of Crohn's disease (1 patient), transient hepatic cytolysis (one case), lack of efficacy or a disease flare (4 cases). Gene expression profile analyses showed a set of gene pathways dysregulated in SOJIA whose expression dramatically changed upon anakinra treatment.