CASE STUDY
Rheumatology

Study description
Phase II, open label, international multicentre clinical trial to investigate to preliminary assess the safety and the efficacy of X in subjects with active Systemic Onset Juvenile Idiopathic Arthritis

Study objective
To determine the safety and tolerability of X in patients with active Systemic Onset Juvenile Idiopathic Arthritis with inadequate response or intolerance to standard therapy with oral steroids and methotrexate, with or without previously used biologic agents.
The study also aimed to evaluate the effect of X on disease activity in patients with active SOJIA, to investigate the possibility of steroid dose tapering in patients with active SOJIA during the treatment with the investigational drug, to assess the effect of X on levels of circulating cytokines and last to consider pharmacokinetic properties of X.

Patient population
Twelve to sixteen subjects of both genders, age 2 to 25 years at enrolment, diagnosed as SOJIA with active disease

Treatment period
The investigational drug was initially administered for 4 weeks and treatment was to be further prolonged up to 12 weeks in total, if the observed benefits (evaluation of therapeutic response to be performed after 4 weeks and every 2 weeks thereafter) and the lack of treatment-limiting toxicity. Patients may continue on treatment until disease progression or intolerable toxicity or study completion. In any event, the overall treatment duration shall not exceed 12 weeks, with two months of follow-up.

Primary Efficacy parameter
The sponsor wanted to evaluate the percentage of patients completing 12 weeks of treatment

Participating countries
Serbia and Montenegro, Italy, Macedonia, Romania

Study details
Total number of patients: 16
Recruitment period: 4 months
First patient in: 14 11 2007
Last patient out: 15 10 2008

Sintesi Research Services
Project management, regulatory, sites coordination, monitoring activities, investigators and CRA meetings, coordination with the central lab.

Key challenges
The study was initially started in three countries, but since recruitment was problematic due the fact that the pathology under study is a very rare one, the Sponsor decided to extend the participation to the trial to other countries, Sintesi Research performed very detailed feasibility enquiries in a series of countries, focusing above all on countries where non competitive study were on going and where investigators and patients may not have access to newer medications. The focus was on Romania due high potential and very motivated physicians.

The study also required the selection of a very qualified central lab and the organization of the pick-up and the delivery of biological samples. Also from this point of view Romania was equal.
The trail required a very diffused communication among the sites, the lab and the site in charge to allocate randomization upon patient inclusion.

The study procedures and the numerous tests for the assessment of the tolerability and the efficacy required a very experienced staff and study team.

A particular attention to the population under study: since the most of the subject include were children a very careful approach was need.

Keys of project success

Sintesi Research organized a local investigators with the participation CRAs and with the staff from the laboratory to familiarize them with the difficult study and drug administration procedures

To maximize patients recruitment Sintesi Research performed a very exact country and sites evaluation and selected highly experienced and motivated investigators.

To keep constantly update the sponsor about screenings and screening results Sintesi Research gave priority on team communication performing weekly teleconferences.

Sintesi Research team worked closely with each clinical site encouraging the investigators to complete enrolment and determining what resources were need to speed up enrolment.

Sintesi Research provided the sites with the study materials: booklets on study procedure, patients questionnaire, patients diaries, guidelines for performing tests and for completion of tolerability and efficacy assessments.

Sintesi Research

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