Drug Therapy

Version of 2016

Introduction

This section reports information on drug therapies that are commonly used to treat paediatric rheumatic diseases. Each section is divided into 4 main parts.

Description

This section provides a general introduction to the drug with its mechanism of action and expected side effects.

Dosage/modes of administration

This section provides the dose of the drug, usually in mg per kg per day or mg per body surface area (square metres), as well as information on the mode of administration (e.g. pills, injections, infusions).

Side effects

This section provides information on the most widely known side effects.

Main paediatric rheumatic diseases indications

This final section reports the list of paediatric rheumatic diseases for which the drug is indicated. Indication means that the drug has been specifically studied in children, and regulatory authorities such as the European Medicines Agency (EMA) or the Food and Drug Administration (FDA) of United States and others allow its use in children. In certain cases, your doctor may decide to prescribe the drug, even if a specific authorisation is not available.

The paediatric legislation, the label and off-label use and future therapeutic possibilities

Until 15 years ago, all drugs used to treat JIA and many other paediatric diseases had not been properly studied in children. This meant that physicians were prescribing drugs based on personal experience or on studies conducted in adult patients.

Indeed, in the past, conducting clinical trials in paediatric rheumatology has been difficult, mainly because of the lack of funding for studies in children and the lack of interest by pharmaceutical companies for the small and non-rewarding paediatric market. The situation changed dramatically a few years ago. This was due to the introduction of the Best Pharmaceuticals for Children Act in USA and specific legislation for paediatric medicines development (Paediatric Regulation) in the European Union (EU). These initiatives essentially forced pharmaceutical companies to study drugs in children as well.

The USA and EU initiatives, together with the existence of 2 large networks, the Paediatric Rheumatology International Trials Organisation (PRINTO at www.printo.it), which unites more than 50 countries worldwide, and the Paediatric Rheumatology Collaborative Study Group (PRCSG at www.prcsg.org), based in North America, had a very positive impact in paediatric rheumatology, in particular on the development of new treatments for children with JIA. Hundreds of families of children with JIA treated by PRINTO or PRCSG centres worldwide have participated in these clinical trials, allowing all children with JIA to be treated with drugs specifically studied for them. Sometimes, participation in these studies requires the use of a placebo (i.e. a tablet or infusion with no active substance) to be sure that the drug under evaluation does more benefit than harm.

Because of these important developments, several drugs are specifically approved for JIA today. This means that regulatory authorities, such as the Food and Drug Administration (FDA), the European Medicines Agency (EMA) and several national authorities, have reviewed scientific information coming from clinical trials and have allowed pharmaceutical companies to state in the drug label that it is efficacious and safe for children.

The list of drugs specifically approved in the treatment of JIA includes methotrexate, etanercept, adalimumab, abatacept, tocilizumab and canakinumab.

Several other drugs are currently being or will be studied in children, so your child might be asked by his/her doctor to participate in such studies.

There are other drugs that are not explicitly approved for use in JIA, such as several non-steroidal anti-inflammatory drugs (NSAIDs), azathioprine, cyclosporine, anakinra and infliximab. These drugs are used without an approved indication (so called off-label use) and your doctor might propose their use especially if there are no other available treatments.

Adherence

Adherence to treatment is of the greatest importance for maintaining good health both on a short and long term basis.
Adherence to treatment entails following the course of treatment prescribed by your doctor; this may include various components: taking medicine on a consistent basis, routine checkups at the clinic, regular physiotherapy, routine follow-up of lab work (blood tests), etc. These various components work together, creating a complementary program which fights the disease, strengthens your child's body and keeps him/her healthy. The frequency and dosage of medications are determined by the need to maintain certain levels of the drug in the body. Lack of adherence to this protocol can result in ineffectively low levels of medication and increase the chance of a flare up of the disease. In order to stop this from happening, it is important to take both shots (injections) and oral medications (tablets or syrup) regularly. The most common reason for lack of success in treatment is not adhering to the recommended treatments. Adherence to all details of the medical program prescribed by the doctor and medical team greatly increases the chance of remission (no active arthritis). Maintaining the various components of treatment can sometimes be taxing on parents and guardians. Nevertheless, it is up to them to make sure that the child receives the best chance for a healthy outcome. Sadly, as a child progresses in age, especially as he/she enters the teenage years, lack of adherence becomes more of an issue. Teenagers resist defining themselves as patients and skip inconvenient parts of their treatment. Consequently, flare ups are very common during these years. Adhering to the medical treatment regime ensures the best chances for remission and improvement in quality of life.