



<b>Early Access to Medicines Scientific Opinion - Public Assessment Report</b>	
<b>Product</b>	<b>dupilumab</b>
<b>Condition</b>	<b>Atopic dermatitis (allergic eczema) is a type of skin inflammation</b>
<b>EAMS indication</b>	<b>For the purpose of EAMS, dupilumab is being made available to children 6 to 11 years of age with severe atopic dermatitis who are candidates for systemic therapy and where existing systemic therapies are not advisable.</b>
<b>Company</b>	<b>Sanofi</b>
<b>EAMS number</b>	<b>04425/0003</b>
<b>EAMS Scientific Opinion date</b>	<b>14<sup>th</sup> August 2020</b>

## **Introduction**

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines and medicines used outside their licence, to UK patients that have a high unmet clinical need. The MHRA scientific opinion provides benefit and risk information to physicians who may wish to prescribe the EAMS medicine under their own responsibility. More information about the scheme can be found here:

<http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm>

The scientific opinion is based on assessment of the information supplied to the MHRA on the benefits and risks of the medicine. As such this is a scientific opinion and should not be regarded as a medicine licensed by the MHRA or a future commitment by the MHRA to license such a medicine, nor should it be regarded as an authorisation to sell or supply such a medicine. A positive scientific opinion is not a recommendation for use of the medicine and should not be interpreted as such. Under EAMS the risk and legal responsibility for prescribing a 'special' remains with the physician, and the opinion and EAMS documentation published by the MHRA are intended only to inform physicians' decision making and not to recommend use. An EAMS scientific opinion does not affect the civil liability of the manufacturer or any physician in relation to the product.

The General Medical Council's guidance on prescribing unlicensed medicines can be found here:

<https://www.gmc-uk.org/ethical-guidance/ethical-hub/trans-healthcare#prescribing>

## **What is dupilumab?**

Dupilumab is a type of biological medicine called a monoclonal antibody that acts on a specific molecule in the body. Dupilumab is given by injection and is supplied as a ready to use syringe and needle.

## **What is dupilumab used to treat?**

Dupilumab is used to treat severe atopic eczema (also called atopic dermatitis). Dupilumab is available as a licensed medicine under the trade name Dupixent to treat adults and adolescents from the age of 12 years with atopic dermatitis. Dupilumab is now being made available through EAMS for children aged 6 to 11 years with severe atopic dermatitis who are suitable for this type of treatment.

### **How is dupilumab used?**

Dupilumab is given by injection under the skin either once every two or four weeks, depending on how much the patient weighs. A single syringe and needle delivers one dose. Dupilumab is available in two strengths so that an appropriate dose can be administered depending on the patient's body weight. If the patient wishes and the doctor considers it appropriate, either they or a parent or guardian can administer the injection, after suitable training has been provided by their doctor or nurse. It is recommended that children are supervised by an adult when giving their injection.

Dupilumab can only be prescribed by physicians experienced in the treatment of dermatological conditions.

### **How does dupilumab work?**

Dupilumab acts selectively on one part of the immune system involved in allergic responses that can lead to the type of chronic skin inflammation seen in atopic dermatitis patients. Dupilumab works by attaching to a molecule called the IL-4 receptor alpha subunit which sits on the surface of particular cells of the immune system. The attachment of dupilumab to its target molecule interferes with messages from molecules called IL4 and IL13 that cause inflammation in the skin of eczema patients. Dupilumab therefore works by dampening this down.

### **How has dupilumab been studied in children aged 6 to 11 years of age with atopic dermatitis?**

Dupilumab was studied in a clinical trial that enrolled 367 patients 6 to 11 years of age with severe atopic dermatitis that could not be controlled by treatments applied to the skin. During an initial period of 16 weeks, dupilumab was compared with a placebo (inactive) drug. Neither the patients nor their doctors knew which treatment they had been assigned to. After the initial treatment comparison period, patients were able to receive dupilumab for longer periods. 115 patients had received dupilumab for one year or more at the analysis point.

### **What are the benefits and risks of dupilumab?**

#### *Benefits*

In children, as in adults and adolescents, dupilumab produces a meaningful reduction in the severity of eczema lesions as well as reducing their extent (how much of the body's surface is affected). Patients also experience a significant reduction in itching and an improvement in quality of life.

#### *Risks*

Dupilumab is overall well tolerated.

During treatment with dupilumab, as with other monoclonal antibody treatments, patients may occasionally develop a serious and unpredictable allergic reaction to the drug, but this is rare. Children may be more prone to threadworm infection while taking dupilumab. Signs of this should be watched out for. Some patients may develop sore eyes. If this happens, speak with your doctor.

### **Why has dupilumab been given a positive Early Access to Medicine Scientific opinion?**

Severe atopic dermatitis (eczema) is a disease that has a major impact on quality of life and the treatment options available are limited and can have harmful effects. Under EAMS, dupilumab is being made available to children with the most severe disease. Dupilumab has been shown to produce improvement in signs and symptoms of the disease including itch; in some patients the improvement may be marked although this is not always the case. The risks associated with dupilumab do not outweigh the benefits

### **What are the uncertainties?**

A relatively small number of patients in the 6 – 11 year age group have received dupilumab for periods of one year or more. Side effects over the longer term are therefore unknown. There is insufficient information on the safety and efficacy of dupilumab in patients under the age of 6 years to make dupilumab available to younger patients.

### **Are there on-going clinical studies?**

There are ongoing studies of dupilumab in the long term treatment of children with atopic dermatitis including children under the age of 6 years. Studies are also ongoing in patients with other atopic (allergic) conditions.

**What measures are in place to monitor and manage risks?**

A risk management plan has been developed to ensure that dupilumab is used as safely as possible. Based on this plan, the company that makes dupilumab must ensure that all healthcare professionals expected to prescribe the medicine, as well as patients and their parents or guardians, are provided with information on the medicine including the side effects and recommendations for minimising these side effects.

Information will be collected about patients before they enter the scheme. Healthcare professionals will be asked by the company to report adverse effects experienced by patients receiving dupilumab through the scheme. They will receive a physician pack and comprehensive training on adverse events prior to commencement of patient treatment. These safety data will be reviewed and reported to the MHRA on a regular basis by the company.

The patient and their parents or guardians will be instructed to record all side effects in a Patient's Diary throughout the treatment period. The diary will be reviewed by the treating dermatologist during regular visits with the patient and their parent or guardian.

The parents or guardians of the patients in the Early Access to Medicines Scheme will also receive a Patient's Card from their doctor summarising what to do should the patient experience any side effects, and the details of their treating dermatologist.

The patient's parent or guardian should carry the card with them at all times in case the patient needs treatment or they need advice from a healthcare professional who is not familiar with dupilumab treatment.

**Other information about dupilumab – see EAMS Treatment Protocol**