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Human Medicines Research and Development Support Division

Public summary of the evaluation of a proposed paediatric investigation plan

Peanut allergen extract for the treatment of peanut allergy

On 17 April 2015 the Paediatric Committee of the European Medicines Agency agreed a Paediatric Investigation Plan* (PIP) for peanut allergen extract for the treatment of peanut allergy (EMA-001481-PIP01-13).

On 9 October 2015, the Paediatric Committee of the European Medicines Agency agreed on some modifications to this paediatric investigation plan. See last paragraph "Subsequent modifications of the agreed Paediatric Investigation Plan" of this document (EMA-001481-PIP01-13-M01).

What is peanut allergen extract, and how is it expected to work?

Peanut allergen extract is expected to diminish or even abolish the allergic response to peanuts in peanut-allergic individuals by making them tolerant to peanuts. This medicine is not authorised in the European Union but studies in adults and children are currently on-going. This medicine is proposed for treatment of peanut allergy in adults and children.

What was the proposal from the applicant?

For children, the applicant proposed:

to study the medicine in children and adolescents from 2 years to less than 18 years of age affected by peanut allergy, in a paediatric investigation plan*. The future indication proposed is: treatment of peanut allergy in children. The plan includes a proposal to determine the right dose for the paediatric population and to show efficacy and safety of the medicine in clinical studies.

The applicant proposed a deferral* for the paediatric clinical studies.

Is there a need to treat children affected by peanut allergy?

Taking into account the proposed indication in adults, and the characteristics of the medicine, the Paediatric Committee considered that it had a potential use for the treatment of peanut allergy. This condition occurs also in children and affects in particular toddlers, preadolescent children and adolescents.

What did the Paediatric Committee conclude on the potential use of this medicine in children?

At present no treatment is authorised for the treatment of peanut allergy in children in the European Union. Therefore, the Committee considered that data are required to decide whether the use of this medicine will bring a benefit to children from 1 year to less than 18 years affected by peanut allergy and to understand any potential risks.

Because there is a need for authorised medicines for the treatment of peanut allergy in children, and given the potential interest of this medicine has for children, the Committee considered that clinical studies were necessary.

The Committee considered that, before starting the studies in younger children, it is more prudent to confirm that the medicine is effective and safe in adults and older children.

Therefore Committee agreed with the request of the applicant that the paediatric clinical studies should be deferred to avoid a delay in the availability of the medicine for adults.

What is the content of the Plan after evaluation?

The Paediatric Committee considered that:

- studies are not necessary in children below 1 year of age because clinical studies with peanut allergen extract cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need in this age group.
- Determination of the best dose should be done with 2 trials of the medicine's behaviour in the body.
- It is necessary to study if the medicine is effective to treat the disease in children. This will be done in 5 studies comparing the medicine to placebo*.
- It is necessary to study the potential side effects of the medicine, to prevent them or to reduce the consequences if they occur. The main concern identified by the PDCO is the occurrence of local and systemic allergic reactions.

What happens next?

The applicant has received the EMA Decision (P/0121/2015)* on this medicine. The Decision itself is necessary for the applicant to request in the future a marketing authorisation* for this medicine in adults and in children.

The Decision on the agreed Paediatric Investigation Plan means that the applicant is bound to perform the studies and trials with children in the next months or years. In case of difficulties, or a change in current knowledge or availability of new data, the applicant may request changes to the plan at a later stage. This can be done through a modification of the PIP.

The agreed completion of all the studies and trials included in the Paediatric Investigation Plan is April 2022.

Trials in the Paediatric Investigation Plan will be listed in the public EU Clinical Trials Register (<https://www.clinicaltrialsregister.eu/>) as soon as they have been authorised to be started, and their results will have to be listed in the register within 6 months after they have completed.

The results of the studies conducted in accordance with the agreed Paediatric Investigation Plan will be assessed, and any relevant information will be included in the Product Information (summary of

product characteristics, package leaflet). If the medicine proves to be effective and safe to use in children, it can be authorised for paediatric use, with appropriate recommendations on the dose and on necessary precautions. The product information will also describe which adverse effects are expected with the medicine, and wherever possible, how to prevent or reduce these effects.

Subsequent modifications of the agreed Paediatric Investigation Plan

On 9 October 2015, DBV Technologies S.A. proposed to change some of the details of the previously agreed Plan.

The Paediatric Committee, after examining the request, agreed to modify the following details:

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- Details in the clinical measures have been modified.
- Two new clinical studies have been added in children from 1 to less than 4 years of age because separate dose-finding and safety / efficacy studies are needed in younger children.

An updated Decision on the Plan has therefore been published on the EMA website (P/0286/2015).

*Definitions

Applicant	The pharmaceutical company or person proposing the Paediatric Investigation Plan or requesting the Product-Specific Waiver
Children	All children, from birth to the day of the 18 th birthday.
Paediatric investigation plan (PIP)	Set of studies and measures, usually including clinical studies in children, to evaluate the benefits and the risks of the use of a medicine in children, for a given disease or condition. A PIP may include “partial” waivers (for example, for younger children) and/or a deferral (see below).
Waiver	An exemption from conducting studies in children, for a given disease or condition. This can be granted for all children (product-specific waiver), or in specific subsets (partial waiver): for example, in boys or in children below a given age.
Deferral	The possibility to request marketing authorisation for the use of the medicine in adults, before completing one or more of the studies /measures included in a PIP. The Paediatric Committee may grant a deferral to avoid a delay in the availability of the medicine for adults.
Opinion	The result of the evaluation by the Paediatric Committee of the European Medicines Agency. The opinion may grant a product-specific waiver, or agree a PIP.
Decision	The legal act issued by the European Medicines Agency, which puts into effect the Opinion of the Paediatric Committee.
Pharmaceutical form	The physical aspect of the medicine (the form in which it is presented), for example: a tablet, capsule, powder, solution for injection, etc. A medicine can have more than one pharmaceutical form.
Placebo	A substance that has no therapeutic effect, used as a control in testing new drugs.
Active control	A medicine with therapeutic effect, used as a control in testing new drugs.
Historical control	A group of patients with the same disease, treated in the past and used in a comparison with the patients treated with the new drug.
Route of administration	How a medicine is given to the patient. For example: for oral use, for intramuscular use, for intravenous use, etc. The same medicine, or the same pharmaceutical form, may be given through more than one route of administration.
Patent	A form of protection of intellectual property rights. If a medicinal product is protected by a patent, the patent holder has the sole right to make, use, and sell the product, for a limited period. In certain circumstances, a patent for a medicinal product may be extended for a variable period by a Supplementary Protection Certificate.
Marketing Authorisation	When a Marketing Authorisation is granted, the pharmaceutical company may start selling the medicine in the relevant country (in the whole European Union, if the procedure was a centralised one).