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Human Medicines Research and Development Support Division

Public summary of the evaluation of the proposed product-specific waiver

Human recombinant follicle-stimulating hormone (FE 999049) for treatment of female infertility and treatment of hypogonadotrophic hypogonadism

On 20 June 2014, the Paediatric Committee of the European Medicines Agency agreed a product-specific waiver* for human recombinant follicle-stimulating hormone (FE 999049) for treatment of female infertility and treatment of hypogonadotrophic hypogonadism (EMA-001183-PIP02-13).

What is human recombinant follicle-stimulating hormone (FE 999049), and how is it expected to work?

Human recombinant follicle-stimulating hormone (FE 999049) is not authorised in the European Union. This medicine is proposed in adults for the controlled ovarian stimulation to induce the development of multiple follicles for assisted reproductive technologies (ART) (e.g. in vitro fertilisation / embryo transfer (IVT/ET), gamete intra-fallopian transfer (GIFT) and intracytoplasmic sperm injection (ICSI)) and induction of ovulation and pregnancy in the oligo-anovulatory infertile patient in whom the cause of infertility is functional and not due to primary ovarian failure.

This medicine is a hormone that is used in women undergoing assisted reproductive technologies such as IVF and that assists ovulation.

What was the proposal from the applicant?

For children, the applicant proposed:

Not to do any study in children (from birth to less than 18 years of age), because the disease female infertility does not occur in children and the disease hypogonadotrophic hypogonadism (in which certain hormones needed for sexual maturation are not produced or are produced in insufficient quantities) does not occur in pre-pubertal children and for pubertal children other medicines are available. Therefore, the applicant requested an exemption (waiver*) from the obligation to study the medicine in any children, in the condition(s) treatment of female infertility and hypogonadotrophic hypogonadism.

Is there a need to treat children affected by female infertility and hypogonadotrophic hypogonadism?

The Paediatric Committee considered this medicine has no potential use in children for female infertility and hypogonadotrophic hypogonadism.

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

The Committee agreed with the request of the applicant to be exempt from performing studies in children from birth to less than 18 years, because the condition for which the medicinal product could potentially be used does not exist in children (female infertility) and that this medicinal product does not seem to have a potential significant benefit over existing treatments for adolescents with hypogonadotrophic hypogonadism.

What happens next?

The applicant has now received the EMA Decision* on this medicine. The Decision itself is necessary for the applicant to request in the future a marketing authorisation* for this medicine in adults.

***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).