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Human Medicines Research and Development Support Division

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

Brentuximab vedotin for treatment of Cutaneous T-Cell Lymphoma

On 19 June 2015, the Paediatric Committee of the European Medicines Agency agreed a product-specific waiver* for brentuximab vedotin for treatment of Cutaneous T-Cell Lymphoma.

What is Adcetrix (brentuximab vedotin) and how is it expected to work?

The active substance in Adcetris, brentuximab vedotin, is made up of a CD30 monoclonal antibody (a type of protein that attaches to CD30). The monoclonal antibody is attached to monomethylauristatin E, a cytotoxic (cell killing) molecule. The monoclonal antibody delivers monomethylauristatin E to the CD30-positive cancer cells, and once inside the cancer cells, it stops them from dividing, which eventually kills the cancer cells.

Adcetris (brentuximab vedotin) is currently authorised for the treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL) following autologous stem cell transplant (ASCT) or following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option.

ADCETRIS is also indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL). The safety and efficacy of children less than 18 years have not yet been established. No data are available.

What was the proposal from the applicant?

The applicant proposed not to do any study in children (from birth to less than 18 years of age), because the medicine did not represent a significant therapeutic benefit over existing treatments for paediatric patients with Cutaneous T-Cell Lymphoma, for whom only topical therapies are generally required. Furthermore the disease occurs almost exclusively in adult populations. Therefore, the applicant requested an exemption (waiver*) from the obligation to study the medicine in any children, in the condition(s) Cutaneous T-Cell Lymphoma.

Is there a need to treat children affected by Cutaneous T-Cell Lymphoma?

The Committee concluded that the condition might very rarely affect children. Futhermore, they are generally treated with topical therapies.

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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 exempted from performing studies in children and adolescents from birth to less than 18 years old, since this medicinal product does not represent a significant therapeutic benefit as clinical studies(s) would be exceedingly difficult to conduct.

What happens next?

The applicant has now received the EMA Decision (P/0168/2015)* on this medicine. The Decision itself is necessary for the applicant to request a new indication, a new route of administration* or a new pharmaceutical form*, as this medicine is already authorised and protected by a patent.

*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 18th 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).