European Medicines Agency
Press office

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Press release

EMEA passes milestone of 40th positive opinion for an orphan medicinal product

The European Medicines Agency (EMEA) has passed the milestone of the 40th positive opinion recommending the granting of a marketing authorisation for an orphan medicinal product by the European Commission since the Regulation on orphan medicinal products entered into force in 2000. Orphan medicinal products covering more than 30 conditions and potentially benefiting some 1.6 million patients have been made available for use in the European Union since 2001.

The success of the Regulation on orphan medicinal products has been underpinned by the work of the Committee for Orphan Medicinal Products (COMP), the EMEA's scientific committee in charge of reviewing applications for designation of medicinal products as orphan medicines. Since 2000, more than 700 applications for the designation of orphan medicinal products have been submitted. The COMP has recommended granting of orphan designation in 470 cases and recommended refusal in 11 cases. The remaining applications were withdrawn or are currently being processed.

Once a medicinal product has been granted an orphan designation by the European Commission, on the basis of a positive opinion from the COMP, its sponsor qualifies for a number of incentives for developing the product and placing it on the market. These incentives include reductions in the regulatory fees payable to the EMEA and free protocol assistance (similar to scientific advice). The incentives are provided through a special orphan fund set up by the European Community, which in 2007 amounts to €6,000,000.

Orphan medicines are for patients suffering from rare life-threatening or chronically debilitating diseases. Examples of such diseases are renal cell carcinoma, malignant gastrointestinal stromal tumours or pulmonary arterial hypertension. Some of the orphan medicines available address serious rare conditions that mostly affect newborn babies or children, such as patent ductus arteriosus or Pompe's disease.

Of the 41 orphan medicines that have received a positive opinion on marketing authorisation, some 32% are for the treatment of rare cancers. Other major therapeutic areas targeted are: metabolic diseases (29%); blood disorders (12%); musculoskeletal and nervous system (12%); cardiovascular diseases (10%).

The 40th and 41st positive opinion were adopted by the Agency's Committee for Medicinal Products for Human Use (CHMP) at its 18-21 June 2007 plenary meeting, for **Atriance** (*nelarabine*), an antitumoral agent, and **Gliolan** (*5-aminolevulinic hydrochloride*), a visualisation agent for fluorescence-guided surgery in malignant glioma. The opinions for these two products have now been sent to the European Commission for adoption of a marketing authorisation decision valid in the whole of the European Union.

The impact of the orphan incentives is reflected in the continuing flow of applications for marketing authorisation submitted to the EMEA, where the CHMP is currently reviewing a further 19 applications for marketing authorisation of orphan-designated medicines.

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NOTES

1. Orphan medicinal products are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting no more than five in 10,000 persons in

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the European Union, or where for economic reasons such medicines would not be developed without incentives. For more information, see 'Orphan drugs and rare diseases at a glance' (EMEA/290072/2007), available here.

2. **Atriance** (*nelarabine*), from Glaxo Group Limited, has received a positive opinion under exceptional circumstances for the treatment of T-cell acute lymphoblastic leukaemia (T-ALL) and T-cell lymphoblastic leukaemia (T-LBL). Atriance was designated as an orphan medicinal product on 16 June 2005. A summary of opinion for Atriance with the exact indication is available here.
3. **Gliolan** (*5-aminolevulinic acid hydrochloride*), from Medac, has received a positive opinion for visualisation of malignant tissue during surgery for malignant glioma (WHO grade III and IV). Gliolan was designated as an orphan medicinal product on 13 November 2002. A summary of opinion for Gliolan with the exact indication is available here.
4. Regulation (EC) No 141/2000 of the European Parliament and of the Council on orphan medicinal products is available here.
5. European Commission decisions on designation of orphan medicinal products can be consulted in the Community Register, available here.
6. Designation as an orphan medicinal product does not indicate that the product has satisfied the efficacy, safety and quality criteria necessary for the granting of a marketing authorisation. This evaluation is made by the CHMP once the application for marketing authorisation has been submitted.
7. Market exclusivity for 10 years is considered to be the main incentive given to companies for the development of orphan medicines. Market exclusivity means that, during this period, similar products cannot normally be placed on the market for the same therapeutic indication. Other incentives for orphan medicines include free protocol assistance (similar to scientific advice) from the EMEA.
8. This press release, together with other information about the work of the EMEA, is available on the EMEA website: http://www.emea.europa.eu

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