5 March 2015
EMA/COMP/88624/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

5-chloro-N2-[2-isopropoxy-5-methyl-4-(4-piperidinyl)phenyl]-N4-[2-(isopropylsulfonyl)phenyl]-2,4-pyrimidinediamine for the treatment of non-small cell lung cancer (NSCLC) that is anaplastic lymphoma kinase (ALK)-positive

On 17 February 2014, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for 5-chloro-N2-[2-isopropoxy-5-methyl-4-(4-piperidinyl)phenyl]-N4-[2-(isopropylsulfonyl)phenyl]-2,4-pyrimidinediamine for the treatment of non-small cell lung cancer (NSCLC) that is anaplastic lymphoma kinase (ALK)-positive. A negative decision was issued by the European Commission on 28 March 2014.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition, as well as an assumption of potential benefit over currently available methods of treatment.

The negative opinion was based on the following reasons:

- The condition applied for (NSCLC that is ALK-positive) is not a distinct, recognised medical entity but rather a subset of a broader medical condition, namely NSCLC, which is not rare.
- The condition applied for is also not a valid subset for orphan designation, because it cannot be established that the medicinal product would only work in patients in this subset as opposed to other patients with NSCLCs.
- Given that there is no valid subset of patients with NSCLC in which the product would be used, orphan designation for the broader medical condition (NSCLC) should have been applied for. As the prevalence of NSCLC (estimated to be about 6 in 10,000) is above the ceiling for orphan designation, which 5 in 10,000 people, the Committee could not recommend the granting of orphan designation.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.