



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 May 2010
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Orphan medicines in numbers

The success of ten years of orphan legislation

The orphan legislation came into force in 2000. Its aim is to give patients suffering from rare diseases access to high quality of treatments by stimulating research and development of medicines for their conditions. The legislation provides a set of incentives to the pharmaceutical industry to develop 'orphan' medicines. These are medicines intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in the European Union, or that, for economic reasons, would be unlikely to be developed without incentives.

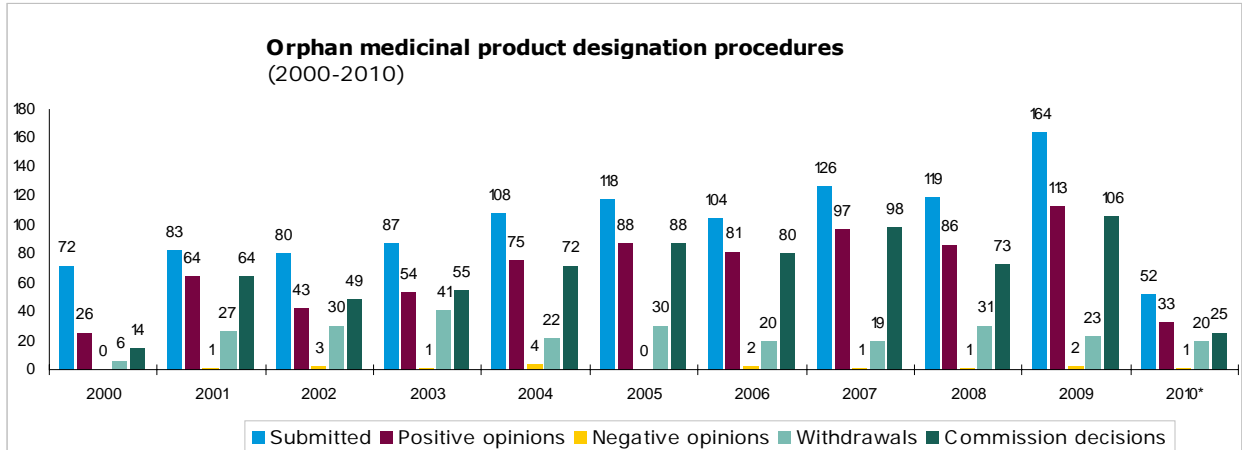
Ten years on, the European Medicines Agency has received over 1000 applications for orphan designation, which have been reviewed by the Agency's Committee for Orphan Medicinal Products (COMP). Over 700 medicines have been granted orphan status by the European Commission. By the end of April 2010, 62 orphan designated medicines have been authorised for marketing in the European Union, potentially benefiting more than 2.6 million European patients suffering from rare diseases. These medicines cover a wide variety of rare diseases, including many genetic diseases and rare cancers, for which there are no satisfactory treatments. A large number of these diseases affect children and newborn babies.

In the last few years the number of applications submitted to the Agency for orphan designation has increased significantly. The continued interest in the orphan designation process by pharmaceutical companies indicates that more orphan medicines will be coming to the market offering treatments for patients with rare diseases.



Status of orphan-designation applications

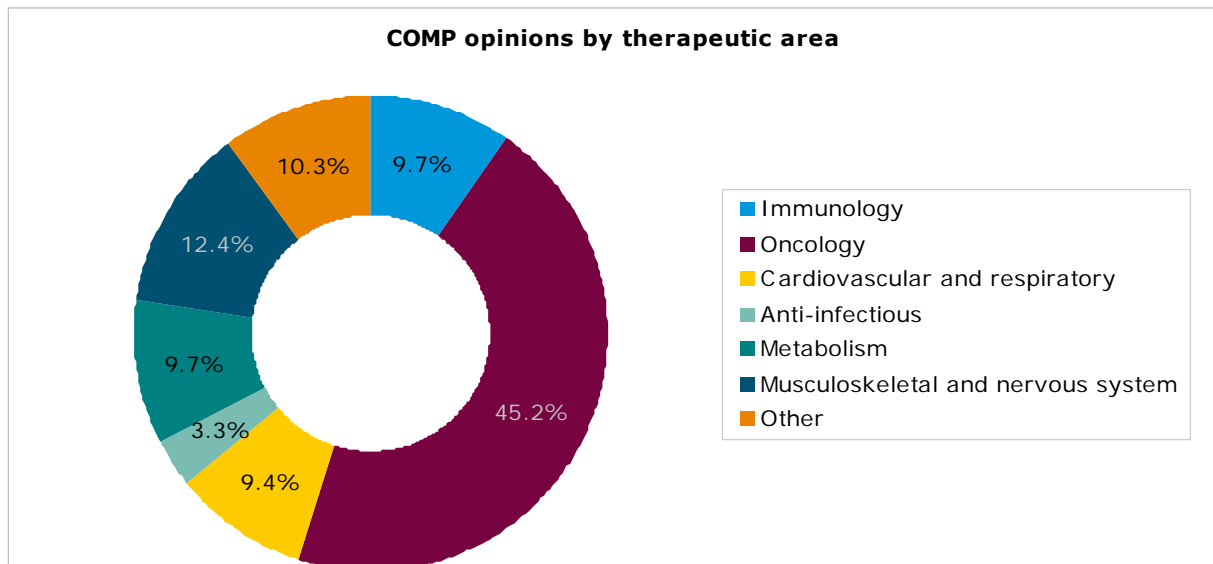
- A total of 1113 applications have been submitted to date for the designation of orphan medicines.
- A total of 760 positive opinions on orphan designation have been adopted by the COMP.
- A total of 269 applications have been withdrawn and 16 received a negative COMP opinion
- A total of 724 medicines have been granted orphan status by the European Commission.



* Figures for 2010 as per 30 April 2010.

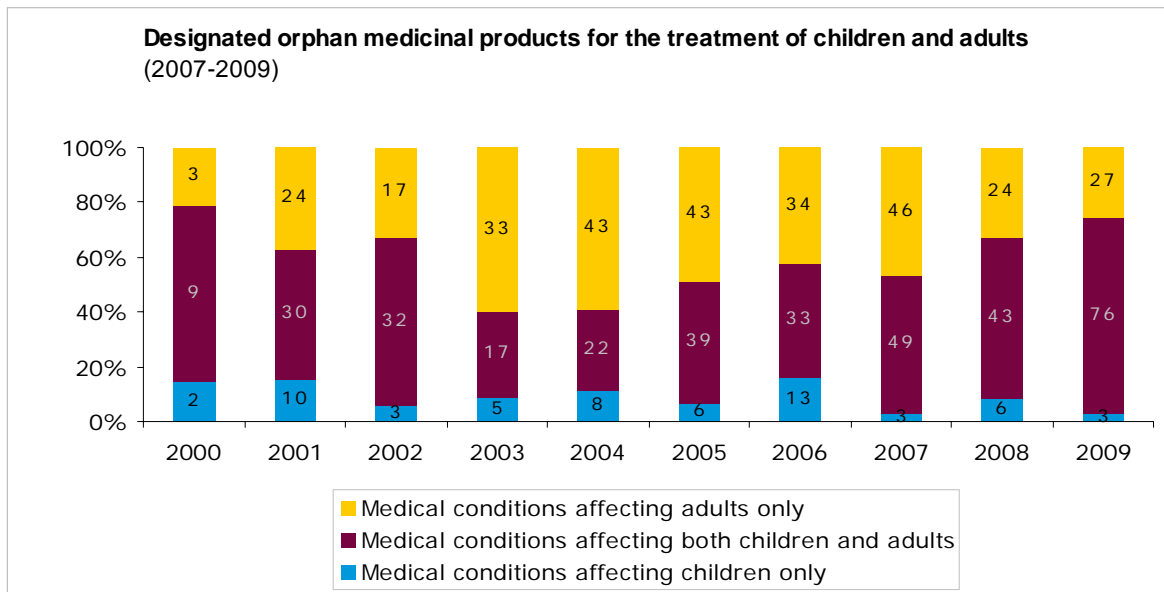
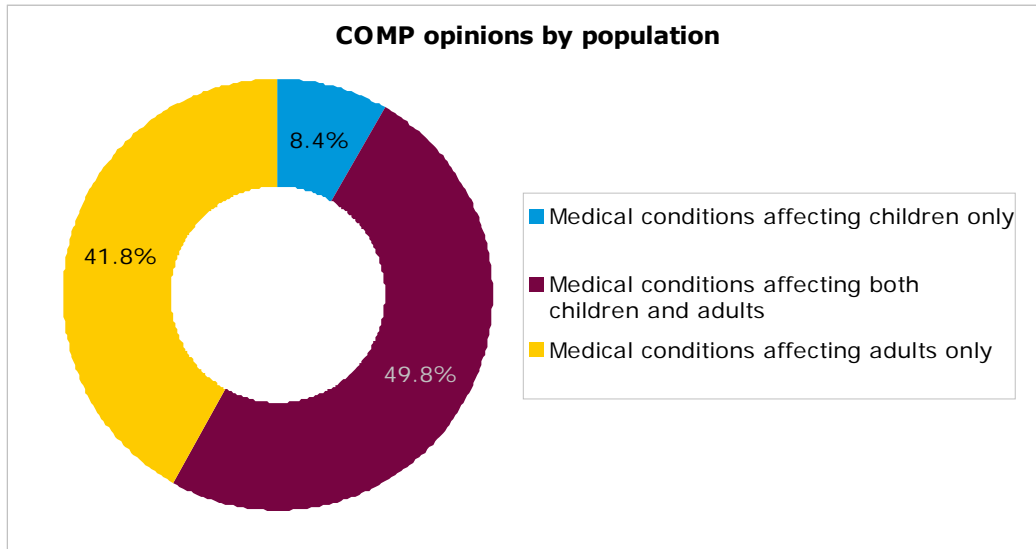
Orphan designation by therapeutic area

- The largest group of orphan medicines for which the COMP has adopted a positive opinion were for oncology treatments.



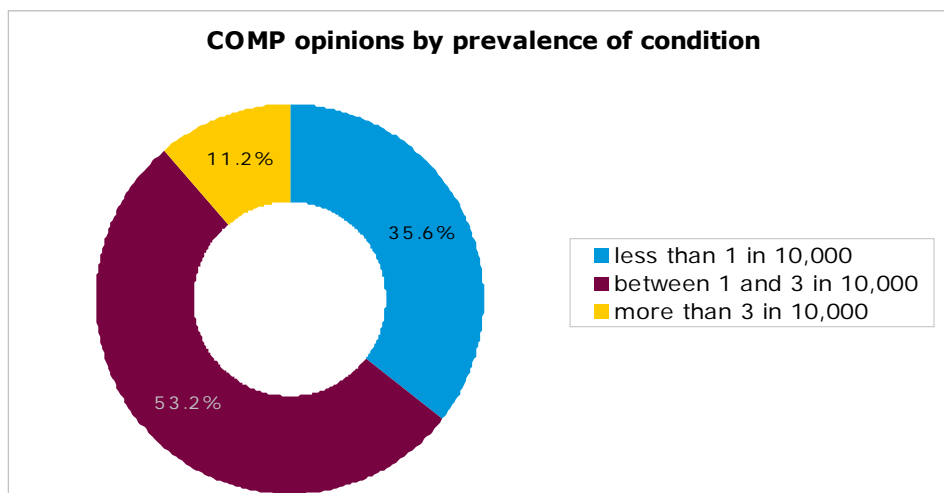
Orphan designation by population

- More than half of the medicines that have received a positive opinion on orphan designation are for conditions that affect children, with 8.4 % of them for conditions that affect only children.



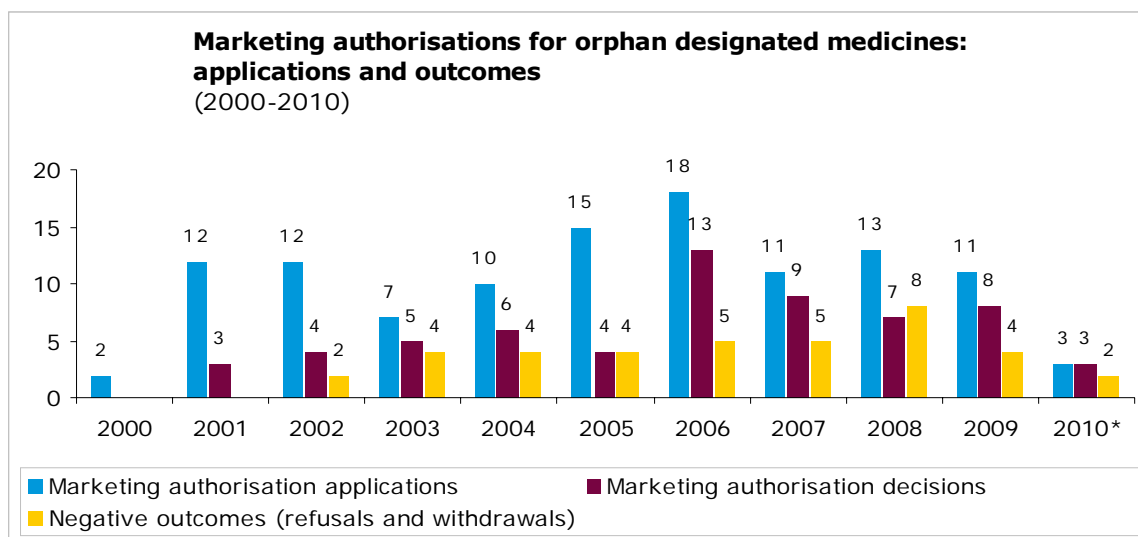
Orphan designation by prevalence of conditions

- The majority of conditions for which products have been given orphan designation affect between one and three in 10,000 people in the EU.



Marketing authorisations for orphan medicines

- A total of 114 marketing authorisation applications for orphan-designated medicines have been submitted to the European Medicines Agency since 2000.
- 62 orphan-designated medicines have received a marketing authorisation valid across the EU.

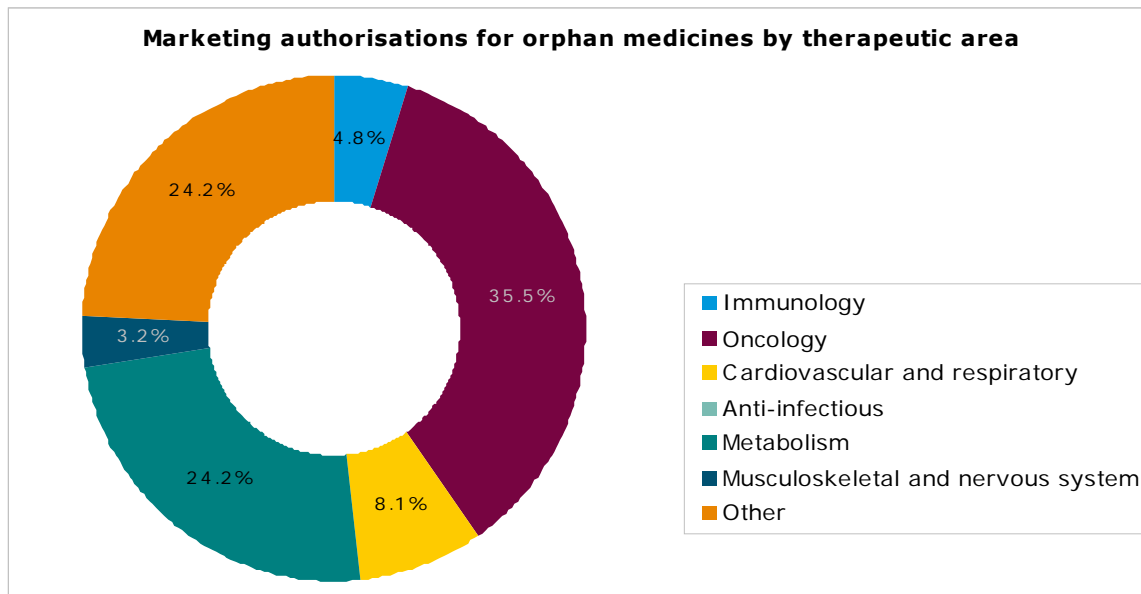


* Figures for 2010 as per 30 April 2010.

- 13 marketing authorisation applications are currently under review.

Marketing authorisations for orphan medicines by therapeutic area

- The largest group of orphan medicines that received a marketing authorisation were for oncology treatments.



List of authorised orphan-designated medicines per year

2001

- **Fabrazyme** for Fabry's disease
- **Replagal** for Fabry's disease
- **Glivec** for chronic myeloid leukemia

2002

- **Tracleer** for pulmonary arterial hypertension
- **Trisenox** for acute promyelocytic leukemia
- **Somavert** for acromegaly
- **Zavesca** for Gaucher's disease

2003

- **Carbaglu** for hyperammonemia
- **Aldurazyme** for mucopolysaccharidosis
- **Busilvex** for hematopoietic progenitor cell transplantation

- **Ventavis** for pulmonary arterial hypertension
- **Onsenal** for familial adenomatous polyposis

2004

- **Litak** for hairy cell leukemia
- **Lysodren** for adrenal cortical carcinoma
- **Pedea** for patent ductus arteriosus
- **Photobarr** for Barret's oesophagus
- **Wilzin** for Wilson's disease
- **Xagrid** for thrombocytopenia

2005

- **Orfadin** for hereditary tyrosinemia type 1
- **Prialt** for chronic pain requiring intrathecal analgesia
- **Xyrem** for cataplexy in patients with narcolepsy
- **Revatio** for pulmonary arterial hypertension

2006

- **Naglazyme** for replacement therapy in patients with mucopolysaccharidosis VI
- **Myozyme** for glycogen storage disease type II (Pompe's disease)
- **Evoltra** for acute lymphoblastic leukemia
- **Nexavar** for advanced renal cell carcinoma
- **Sutent** for gastrointestinal stromal tumor and metastatic renal cell carcinoma
- **Savene** for anthracycline extravasation
- **Thelin** for idiopathic pulmonary arterial hypertension or pulmonary arterial hypertension
- **Exjade** for chronic iron overload due to blood transfusions
- **Sprycel** for acute lymphoblastic leukemia and chronic myeloid leukemia
- **Diacomit** for severe myoclonic epilepsy in infancy
- **Elaprase** for mucopolysaccharidosis type II (Hunter's syndrome)
- **Inovelon** for Lennox–Gastaut syndrome
- **Cystadane** for homocystinuria

2007

- **Revlimid** for multiple myeloma
- **Soliris** for paroxysmal nocturnal hemoglobinuria
- **Siklos** for sickle cell syndrome
- **Atriance** for acute lymphoblastic leukemia
- **Increlex** for primary insulin-like growth factor 1 deficiency due to molecular or genetic defects
- **Gliolan** for intraoperative photodynamic diagnosis of residual glioma
- **Yondelis** for soft tissue sarcoma
- **Tasigna** for chronic myeloid leukemia
- **Torisel** for renal cell carcinoma

2008

- **Thalidomide Celgene** for multiple myeloma
- **Volibris** for pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
- **Firazyr** for angioedema
- **Ceplene** for acute myeloid leukemia
- **Kuvan** for hyperphenylalaninemia
- **Mepact** for osteosarcoma
- **Vidaza** for acute myeloid leukemia and myelodysplastic syndromes

2009

- **Nymusa** for primary apnea in premature newborns
- **Afinitor** for renal cell carcinoma
- **Mozobil** for mobilize progenitor cells prior to stem cell transplantation
- **Cayston** for Gram-negative bacterial lung infection in cystic fibrosis
- **Arcalyst** for cryopyrin-associated periodic syndromes, including familial cold autoinflammatory syndrome and Muckle-Wells syndrome
- **Ilaris** for cryopyrin-associated periodic syndromes, including familial cold autoinflammatory syndrome and Muckle-Wells syndrome
- **Nplate** for idiopathic thrombocytopenic purpura
- **Firdapse** for the treatment of Lambert-Eaton myasthenic syndrome (LEMS)

2010

- **Revolade** for the treatment of chronic immune (idiopathic) thrombocytopenic purpura (ITP)
- **Tepadina** for conditioning treatment prior to autologous or allogeneic haematopoietic progenitor cell transplantation
- **Arzerra** for the treatment of chronic lymphocytic leukaemia (CLL)