EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 years of the Orphan Regulation in Europe conference - The experience up to date

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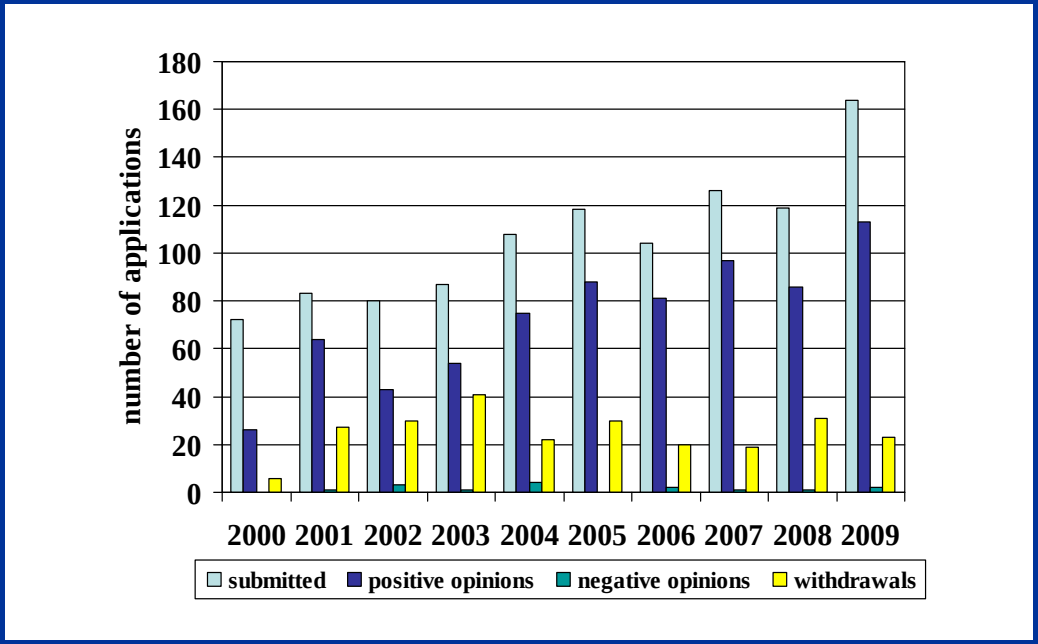
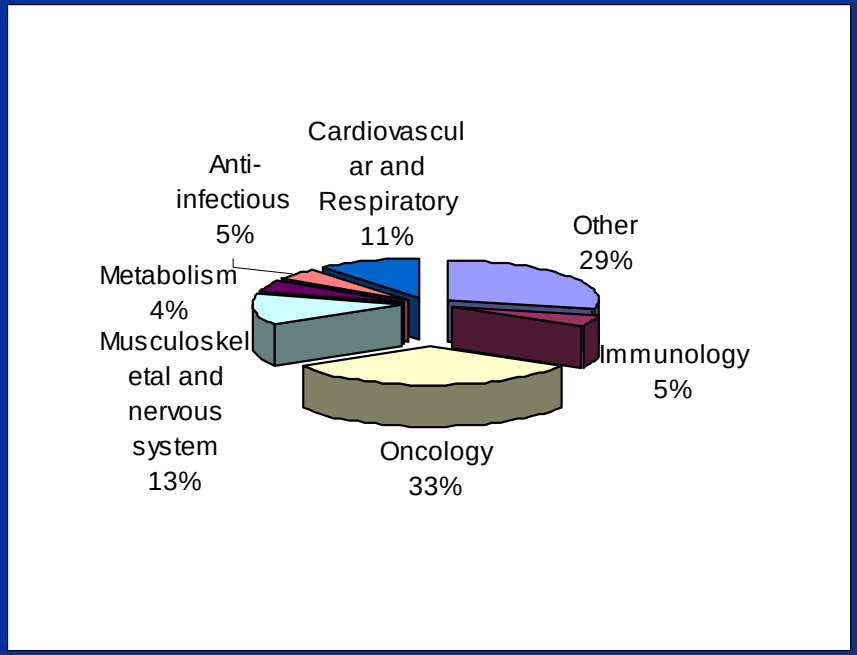
Outcomes of applications for ODD

- 1113 applications submitted
- positive outcome in 2/3 of the cases, high success rate
- few negative opinions per year
- withdrawals may reapply at a later stage

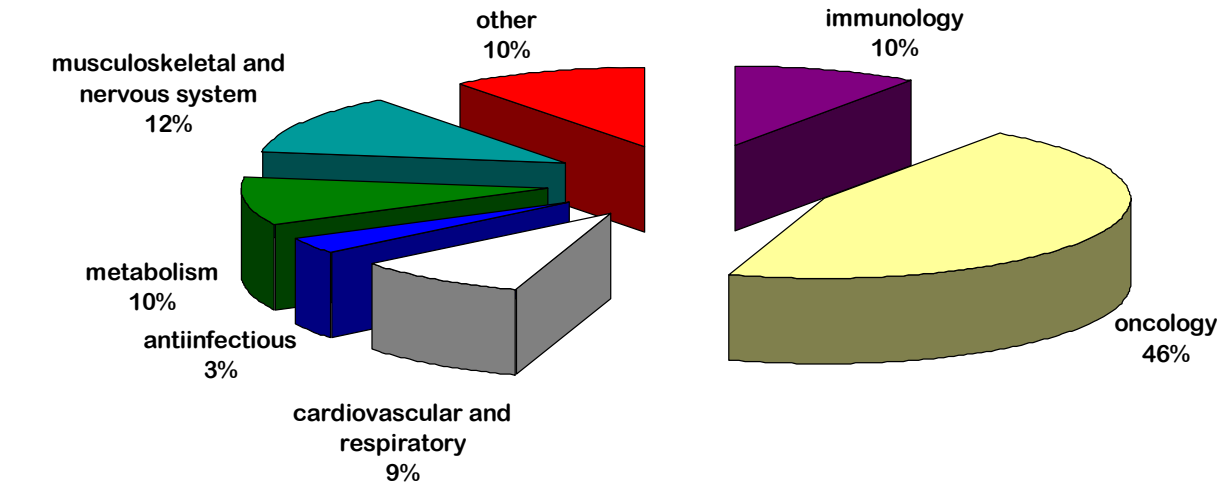


Withdrawals per therapeutic area

- 269 withdrawals
- most withdrawals within oncology (such as malignant melanoma, pancreatic cancer)

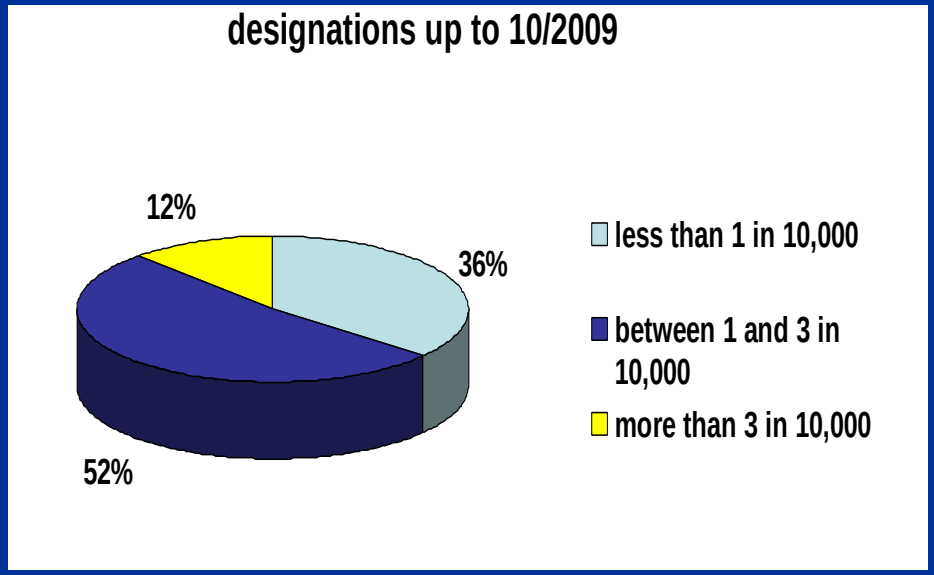


Opinions per therapeutic area

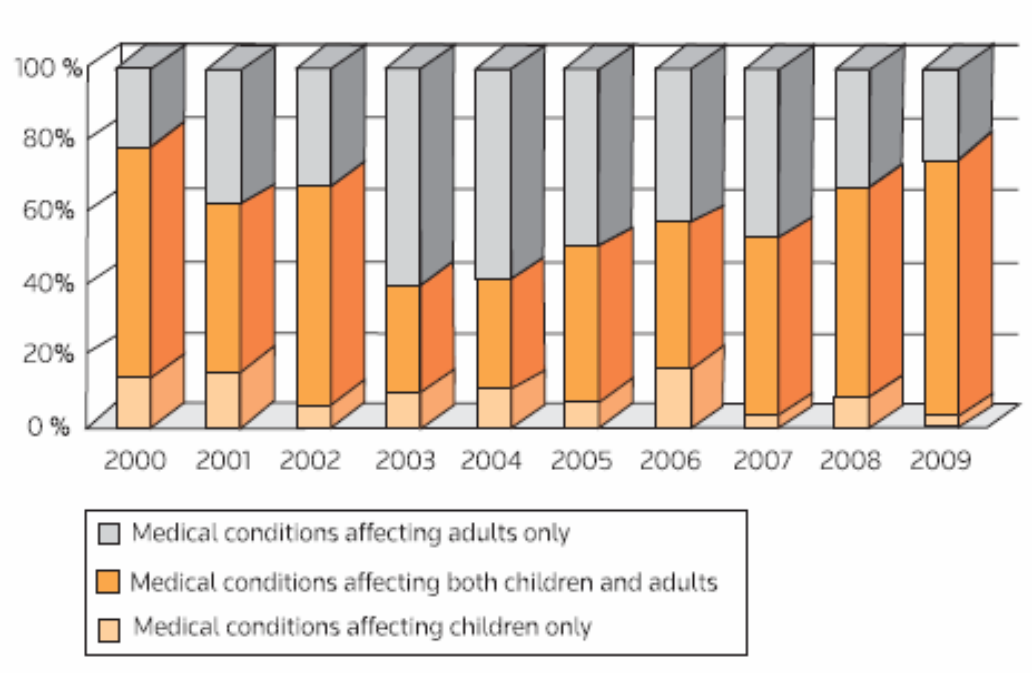


Prevalence of designated applications

- More than a third affect less than 1 in 10,000 in the EU
- Would attract little interest per se without OD incentives



Target populations (age)

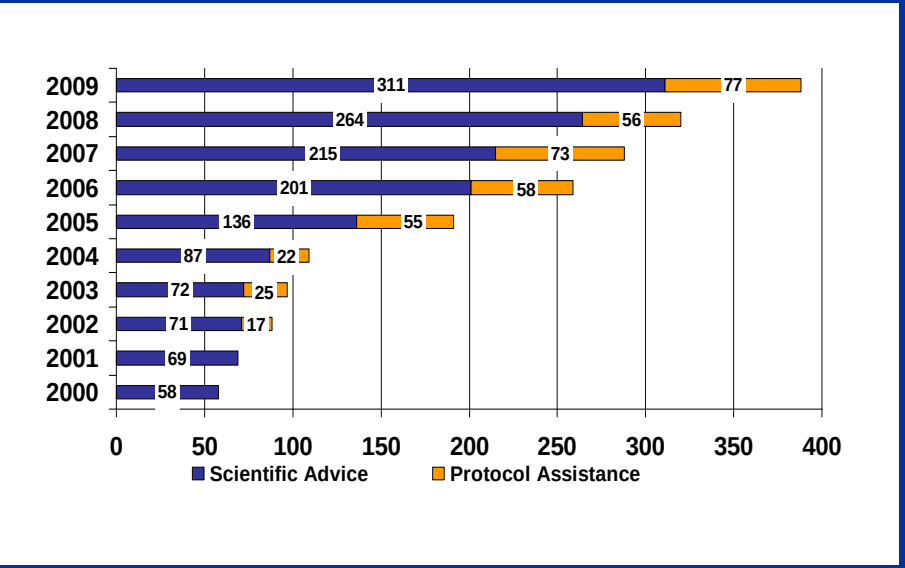


The story so far…

- A simple framework that works
- 1113 applications submitted
- More than two thirds positive opinions
- 724 designated orphan products

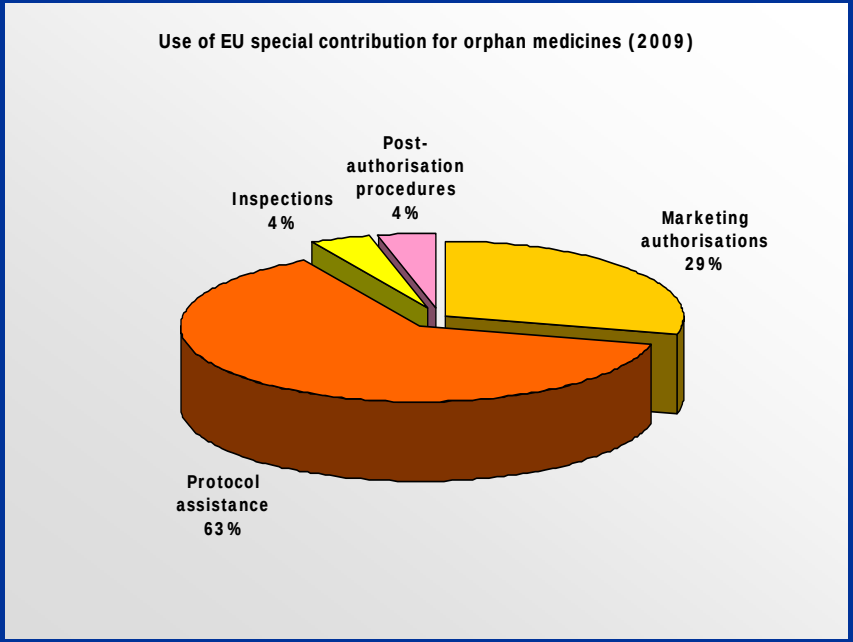
Protocol Assistance

- Protocol assistance is free scientific advice
- Yearly increase in the number of SA/PA procedures
- Links to success in marketing authorisation



Use of EU special contribution for OD

- Protocol assistance is one of the most utilized incentives
- Contrast to post-authorization procedures and inspections



OD Marketing Authorisations

2001	**Fabrazyme** for Fabry disease
	Replagal for Fabry disease
	Glivec for chronic myeloid leukaemia
2002	**Tracleer** for pulmonary arterial hypertension
	Trisenox for acute promyelocytic leukaemia
	Somavert for acromegaly
	Zavesca for Gaucher disease
2003	**Carbaglu** for hyperammonaemia
	Aldurazyme for Mucopolysaccharidosis
	Busilvex for haematopoietic progenitor cell transplantation
	Ventavis for pulmonary arterial hypertension
	Onsenal for Familial Adenomatous Polyposis
2004	**Litak** for Hairy cell leukaemia
	Lysodren for adrenal cortical carcinoma
	Pedea for Patent Ductus Arteriosus
	Photobarr for Barret's oesophagus
	Wilzin for Wilson's disease
	Xagrid for Thrombocythaemia

OD Marketing Authorisations

2005	**Orfadin** for Hereditary tyrosinemia type 1
	Prialt for chronic pain requiring intrathecal (IT) 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 leukaemia
	Nexavar for advanced renal cell carcinoma
	Sutent for gastrointestinal stromal tumour and metastatic renall 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 leukaemia and chronic myeloid leukaemia
	Diacomit for severe myoclonic epilepsy in infancy
	Elaprase for mucopolysaccharidosis type II (Hunter syndrome)
	Inovelon for Lennox-Gastaut syndrome
	Cystadane for homocystinuria

OD Marketing Authorisations

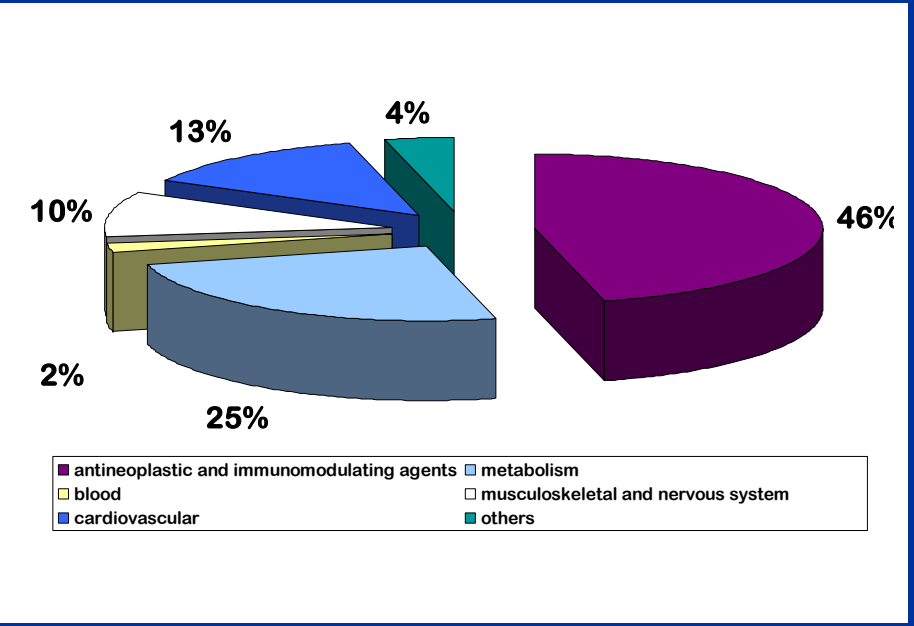
2007	**Revlimid** for multiple myeloma
	Soliris for paroxysmal nocturnal haemoglobinuria
	Siklos for sickle cell syndrome
	Atriance for acute lymphoblastic leukaemia
	Increlex for primary insulin-like growth factor-1 deficiency due to molecular or genetic defects
	Gliolan for Intra-operative photodynamic diagnosis of residual glioma
	Yondelis for soft tissue sarcoma
	Tasigna for chronic myeloid leukaemia
	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 leukaemia
	Kuvan for hyperphenylalaninaemia
	Mepact for osteosarcoma
	Vidaza for acute myeloid leukaemia and myelodysplastic syndromes

OD Marketing Authorisations

2009	**Nymusa** for primary apnoea in premature newborns
	Afinitor for renal cell carcinoma
	Mozobil for mobilization of progenitor cells prior to stem cell transplantation
	Cayston for gram negative bacterial lung infection in cystic fibrosis
	Arcalyst for Cryopyrin-Associated Periodic Syndromes (CAPS), including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS)
	Ilaris for Cryopyrin-Associated Periodic Syndromes (CAPS), including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS)
	Nplate for idiopathic thrombocytopenic purpura (ITP)
	Firdapse for Lambert-Eaton Myasthenic Syndrome
2010	**Revolade** for idiopathic thrombocytopenic purpura
	Tepadina for conditioning prior haematopoietic progenitor cell transplantation
	Arzerra for chronic lymphocytic leukemia

Distribution of Marketing authorizations per therapeutic area

- 62 orphan drugs received marketing approval so far
- More than one third antineoplastic and immunomodulating, followed by agents for metabolic diseases



The story so far…

- A Simple Framework that has produced measurable output:
- 724 designated orphan products across the entire spectrum of human disease
- 62 Orphan Medicinal Products have received Marketing Authorisation

Thank you