

[www.eurordis.org](http://www.eurordis.org)

- 9 • EURORDIS' Orphan Drug Task Force:  
Regular dissemination of information to a network of volunteers affected by rare diseases and supporting orphan drugs.

The role played by Eurordis is financially independent from the pharmaceutical industry. All orphan drug activities are made possible through the work of Eurordis' volunteers and the financial support from its members, AFM-Téléthon and the EMEA.



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- 450 orphan drugs designated since 2000
- 37 orphan drugs with marketing authorisation since 2000
- 2 million EU citizens potentially benefiting from these drugs

#### SOURCES OF INFORMATION ON ORPHAN DRUGS:

Full list of designated orphan drugs available at  
<http://ec.europa.eu/enterprise/pharmaceuticals/register/alforphreg.htm>

Eurordis' website and electronic monthly newsletter  
[www.eurordis.org](http://www.eurordis.org)

European Medicines Agency (EMA)  
<http://emea.europa.eu>



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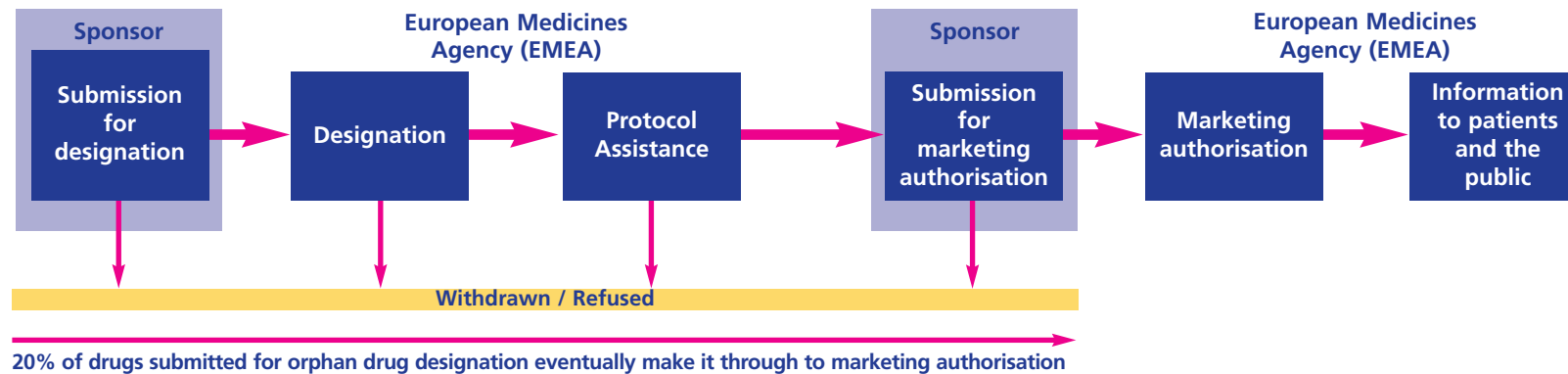


## ORPHAN DRUGS : THE ROLE PLAYED BY EURORDIS



# ORPHAN DRUGS • THE ROLE PLAYED BY EURORDIS

All orphan drugs undergo a single centralised procedure at European level



- 1** EURORDIS was instrumental in the development and adoption of the EU Regulation on Orphan Medicinal Products in 1999.
- 2** EURORDIS plays an important role in the orphan drug development process through its participation in the Committee for Orphan Medicinal Products (COMP) at the EMA (European Medicines Agency). EURORDIS has 2 full members and 1 observer in the COMP.
- 3** EURORDIS successfully advocated for the implementation of incentives in the development of orphan drugs:
  - Fee waiver for orphan designation and reduced fees for marketing authorisation, inspections, variations and protocol assistance;
  - 2 year extension of market exclusivity for orphan drugs for children (12 years in total instead of 10 for other orphan drugs).
- 4** EURORDIS currently advocates for:
  - Parallel EU-US submission and designation of orphan drugs to speed up development and access to new drugs based on a single dossier;
  - Creation by the Commission, within FP7, of a Clinical Research Programme for orphan drugs in support of designated products;
  - National incentives such as research grants and tax credits.
- 5** EURORDIS collaborates closely with the EMA for the production of quality information on orphan drugs for patients:
  - At the time of designation, Eurordis reviews all Public Summaries of COMP opinion and liaises with concerned patient groups;
  - At the time of marketing authorisation, Eurordis facilitates the reviewing of EPARs (European Public Assessment Reports) by rare disease patients.
- 6** EURORDIS identifies and supports rare disease patient representatives for participation in:
  - Protocol assistance;
  - Meetings of the Scientific Advice Working Party;
  - Other meetings such as discussions on guidelines and risk management programmes.
- 7** EURORDIS is instrumental in the inclusion of over 60 rare disease patient representatives in the various activities linked to the orphan drug development process.
- 8** EURORDIS advocates for patient access to authorised orphan drugs.
  - Regular survey to assess and compare real availability of orphan drugs for patients in the various Member States;
  - Promotion of European common policy and criteria for orphan drug access.