ORPHANET
Orphan Drug Services

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Orphanet
Orphan Drug Services

Expectations

- Information on orphan drugs
- Information on European policy and its regulation
- Transparency of clinical trials
Orphan Drug Services

Objectives

- Centralise available but scattered information
- Assist in the distribution of medicinal products
- Trace the stages of development of a drug
- Assist in recruitment for clinical trials
- Contribute to the transparency of clinical trials
List of Orphan Drugs and Rare Diseases

Orphan Drugs in Europe

Regulation 1999:

- 432 orphan drug designations by COMP-EMEA for more than 200 diseases
- 35 orphan drugs authorised for the European market
  - List of orphan drugs available on Orphanet
List of Orphan Drugs and Rare Diseases

Orphan Drug Regulations in the Rest of the World:
- USA (1983)
- Japan (1993)
- Australia (1997)
- Singapore (1991)

Non-orphan Drugs with Rare Disease Applications
- European market authorisation
- National market authorisation
  - French market authorisation
- Temporary authorisation for drugs with rare disease indications (ATUc)
- Assistance in medicinal product distribution
Indexing Orphan Drugs

Searching for a Drug

- Search
  - by disease
  - by laboratory
  - by drug (active substance, Brand name particular to country)

- Extension of search criteria
  - by stage of development, by designation

Indexation

- Any drug with rare disease indication
  - Name of active substance
  - Internationally used generic name
  - Name of code before marketing
  - Brand name particular to each country
  - ATC, MedDRA classification
Comprehensive Information on Orphan Drug Regulations

- Regulatory aspects around the world
- Various statuses of dispensability of orphan drugs in Europe
- Comparison of orphan drug policies around the world
- Clinical trials
Dissemination of Clinical Trials in Europe

Registration service for informing patients of current clinical trials (since 2005)

- Service available to patients
- Service available to professionals
  - patient recruitment by health providers
  - industrial professionals seeking volunteers

Participating in clinical trials
Directory of Clinical Trials in Europe

- Inventory of the development stage of a designated molecule or an orphan drug
- Most in phases II and III

Key figures
- 268 industrial sponsors for 432 designations
- 591 clinical trials
Gathering Information on Clinical Trials in Europe

- Contact with sponsors of designation for collaboration and validation
- Positive aspect of discussion and collaboration between laboratories
- Contact with academic researchers
- Contact with clinical trial investigators: role of Orphanet Europe partner network
Contribution to the Transparency of Clinical Trials in Europe

Transparency of Clinical Trials
- Clinical trials online since 2003
- European legislation in 2005

International Committee of Medical Journal Editors
- Publication of studies in a public database
- Publication of clinical trial results
Thank you for your attention