

# Increasing the awareness for rare diseases and orphan drugs in Eastern European countries

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http://www.orpha.net/

# orphanet

<p><b><u>Maladies Rares</u></b> <b><u>Médicaments orphelins</u></b></p> <p>Serveur d'information pour tous publics</p>	<p><b><u>Rare diseases</u></b> <b><u>Orphan drugs</u></b></p> <p>Free-access website providing information</p>
<p><b><u>Seltene Krankheiten</u></b> <b><u>Medikamente</u></b> <b><u>für seltene Krankheiten</u></b></p> <p>Informationsangebot für die Öffentlichkeit</p>	<p><b><u>Malattie rare</u></b> <b><u>Farmaci orfani</u></b></p> <p>Servizio di informazioni al pubblico</p>
<p><b><u>Enfermedades raras</u></b> <b><u>Medicamentos huérfanos</u></b></p> <p>Portal de información público</p>	<p><b><u>Doenças raras</u></b> <b><u>Medicamentos orfaos</u></b></p> <p>Serviço gratuito de informação ao público</p>

ORPHANET - Inserm SC 11 - 102 rue Didot 75014 - Paris - France

# Orphanet was created in 1997 to address identified problems.....

Lack of information	→	Encyclopaedia of rare diseases
Scarce expertise	→	Directory of experts
Too few collaborations	→	Directory of research projects
Difficult recruitment	→	On-line service to register as a volunteer
	→	Directory of clinical trials
Lack of partnership	→	OrphanXchange

# Orphanet's mission.....

- Communicate and Provide Information
  - to all stakeholders
  - reliable and up-to-date
  - easy to access
  
- Information is a key issue in the field of rare diseases
  - to improve diagnosis and treatment
  - to speed up research
  - to contribute consumers' empowerment
  - to improve correct use of clinical resources

# Orphanet Funding

- Funding from European Commission
  - DG Public Health
  - DG Research
- Funding from France
  - New 3 year grant from Ministry of Health
  - INSERM: same core budget
  - LEEM (Pharma companies)
  - Telethon (AFM)
  - Groupama Foundation (insurer)

# Orphanet

www.orpha.net

## Encyclopaedia

- review article
- expert-authored
- peer-reviewed
- over 1300 diseases

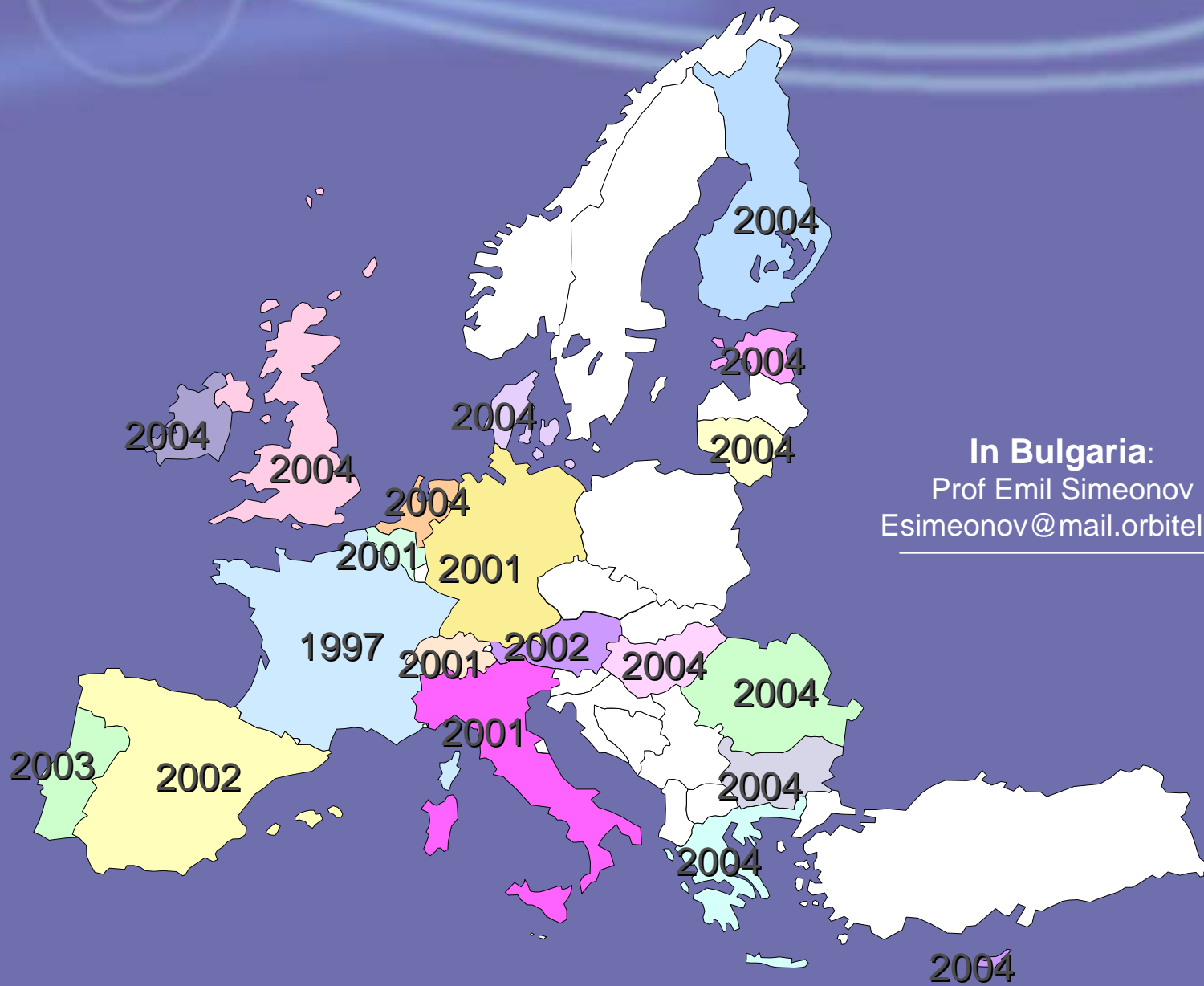
## Orphan drugs database

- Europe
- USA
- Japan
- Australia

## Directory of services

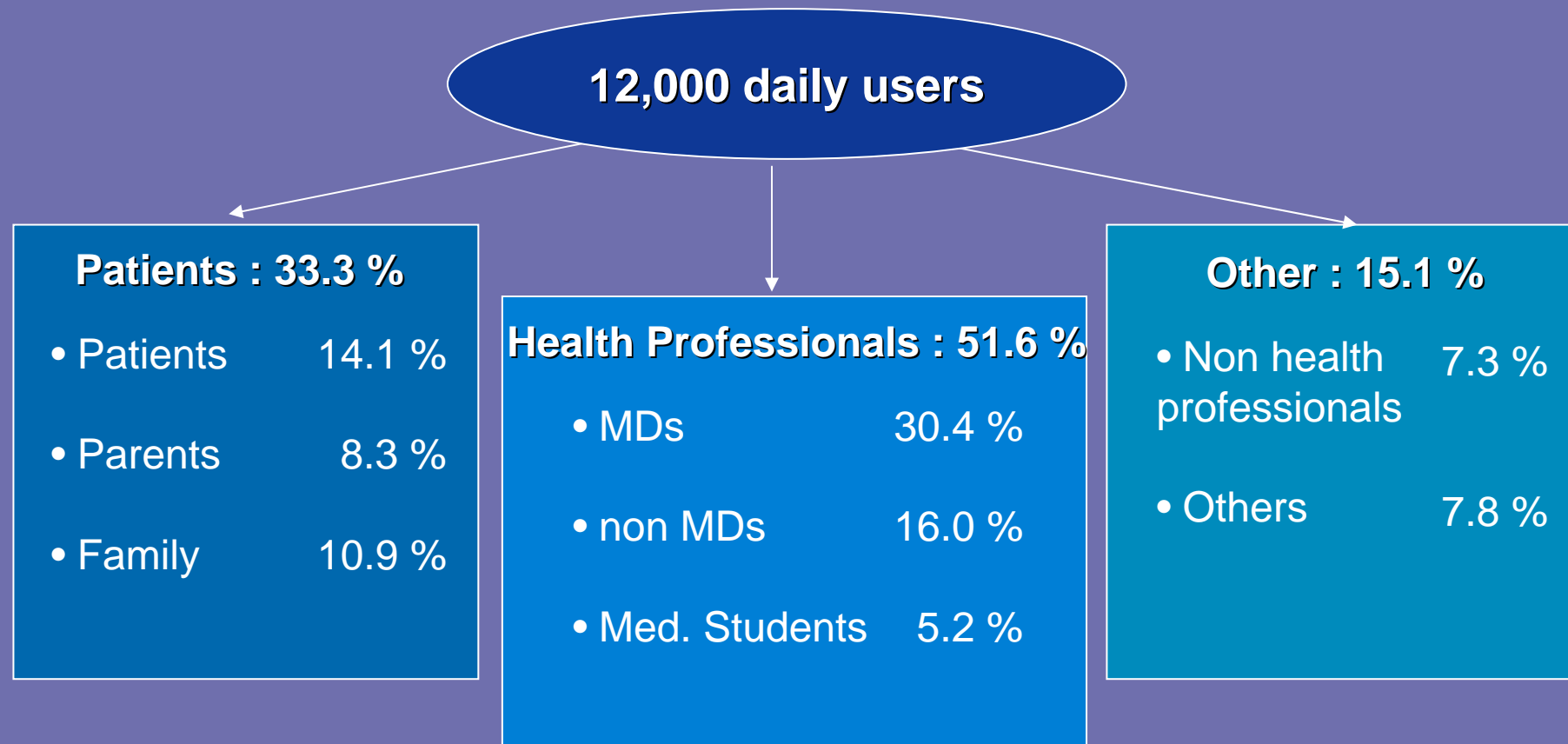
- Clinics
- Tests
- Research projects
- Support groups
- Networks
- Registries
- Clinical trials

**In 20 countries**



In Bulgaria:  
Prof Emil Simeonov  
[Esimeonov@mail.orbitel.bg](mailto:Esimeonov@mail.orbitel.bg)

# Orphanet users in March 2005





## Current content of the database

- 3.715 diseases and synonyms
- 1.361 abstracts
- 640 review articles
- 786 diagnostic labs covering 983 diseases
- 2.056 research projects covering 1.240 diseases
- 929 patients organizations covering 1.447 diseases
- 1.405 specialized clinics
- 5.067 professionals
- 4.780 other web pages

# Conclusion

- + 8% of visitors per month since the beginning
  - Serving all types of public
  - Real customers
  - High customer satisfaction
- 
- Orphanet is the most comprehensive set of review articles in the world
  - The directory of services has to be expanded to reflect the real situation in all European countries

# Extension of the network

- Principles
  - Any European country if the resources are identified and if a local professional is willing to run the project
- New country partners
  - Lebanon + Morocco + Tunisia
  - Grant for Francophone Universities
- New candidates
  - Czech Republic + Slovakia + Sweden
  - Contacts are starting with Poland and Turkey

# Orphanet in Central and Eastern Europe

- We accept new partners immediately if:
  - They have the resources to collect data
  - They accept to work with our procedures
- New application for funding next year
- ? Boundaries of Europe

# **A set of services powered by Orphanet.....**

aimed at developing information tools to address, in a comprehensive and integrated approach, the set of factors that currently affects research on rare diseases and its coordination

# Orphanet Newsletter

## OrphaNews Europe

- Monthly electronic Newsletter in English
- Content: Scientific and Political news + Events + Books
- Funding from European Commission
- Rare Diseases Task Force

Please Register  
and send information for inclusion  
[orphanews-europe@orpha.net](mailto:orphanews-europe@orpha.net)


# Services to boost R&D

- Directory of research projects in Europe
- A tool to better inform patients about clinical trials: **Eclor**
  - To speed up enrolment of patients
- A tool to boost the development of diagnostic tools and therapeutic products: **OrphanXchange**
  - To facilitate Academia-Industry partnership

# ECLOR: objectives

- To allow patients to leave their details in order to be informed about new clinical studies / clinical trials for their disease
- Expected to facilitate enrolment and speed up research



 Registration of volunteers

[← Orphanet home](#)

### Clinical studies

Welcome !  
Patient  
Parents  
Doctor  
Family or friend  
Contact us

### Orphanet

Welcome !

## Participating in clinical research [Print](#)

This service offers persons suffering from rare diseases the opportunity to volunteer for active participation in the development of therapeutic research. Registered patients are informed of ongoing and new clinical studies of interest to them. Patients remain free to ask to be recruited or not.

Recruitment must be done through the researcher responsible for the project, the principal investigator in the case of a clinical trial. A clinical trial is a medical study designed to test the effect of a new drug or of an existing drug, of a biological treatment, or of a medical device that could treat or prevent an already known rare disease. Clinical trials are carried out ethically and with care taken to protect the patients from unnecessary side effects and to allow an exhaustive and precise data collection of information about the disease. It is the clinical study investigator who is in charge of validating the applications as strict criteria must be adhered to before enrolment in such a study. Early registration on the Orphanet list does not constitute a guarantee, in any way, that the application you made would be selected should a new clinical trial take place. Neither does registration guarantee that a clinical study will be conducted in the near future if there is not an ongoing study. The registration on the Orphanet list of clinical study volunteers allows you to be quickly aware of the launch of a new clinical study and to stand for recruitment. This service has been approved by the C.N.I.L. (Commission Nationale Informatique et Liberté)..

If you wish to volunteer for participation in a clinical study, please select your status

- [Patient](#)
- [Parent or legal representative of a person suffering from a rare disease](#)
- [Doctor of a person suffering from a rare disease](#)
- [Family or friend of a person suffering from a rare disease](#)

This access is only for patients or patients' legal representatives who are already registered.

Login

Password

Case sensitive. Forgotten login or password?,  
[Please click here](#)

Welcome ! Orphanet

# ECLOR users: February - May 2005

- 472 registrations (2 to 4 a day)
- 235 distinct rare diseases
- 12 different countries

# OrphanXchange

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

- Market place of:
  - ✓ academic research projects
  - ✓ and potential orphan designations
  - ✓ of interest for Industry
- Freely accessible via a website
- To contribute to the development of diagnostics, devices and pharmaceuticals

# Identify promising projects...

- Select among academic research projects
  - ✓ As listed in Orphanet
  - ✓ As notified by departments of technology transfer
- Contact the researcher for additional information and approval
- Write up the document to be published in OXC
  - ✓ Information of interest for Industry
  - ✓ Protecting confidential information

powered by orphanet®

[print](#)

**oxc** Services

[OXC Home Page](#)  
[Search database](#)  
[Submit a project](#)

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**General Information**

[About OrphanXchange](#)  
[About Rare Diseases](#)  
[About Orphan Drugs](#)  
[OrphaNews](#)

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**Contact us**

OrphanXchange is a **market place of projects** in the field of **rare disease therapy and diagnosis**. It includes:

- ♦ a **database of molecules and technologies** of potential interest
- ♦ a **tool to network potential partners**

**OrphanXchange Content**

Research projects:	124
Last update:	25 Mar 2005

OrphanXchange is a **website freely accessible to all stakeholders but specifically designed to meet industry needs**. It aims to facilitate the **transposition of scientific developments into therapies and diagnostic tools** in the framework of the orphan drug regulation.

Unregistered users can query the database and obtain topics matching their query, if available. Registration is required to access detailed information. It is free-of-charge.

**Unregistered Users**

[Search database](#)  
and access project topics

**Registered Users**

Search database  
and access project details

**Login**

**Password**

[I forgot my password](#)

**Want to Register ?**

[Registration](#)

OrphanXchange operates in collaboration with Orphanet which is a database of rare diseases encompassing an encyclopaedia and a directory of services in twenty European countries. Orphanet is the appropriate tool to access the list of on-going research activities in the field of rare diseases, as orphanXchange only lists projects seeking partnership. OrphanXchange is supported by **INSERM** (Institut National de la Santé et de la Recherche Médicale), **LEEM** (Les Entreprises du Médicament) and by **DG Research of the European Commission** (OrphanPlatform Contract n° LSSM-CT-2004-503246)

Institut national  
de la santé et de la recherche médicale

La recherche avance, la vie progresse

LSSM-CT-2004-503246

# OrphanXchange data

- Number of projects 125
- Number of visits 3.403
- Registered users 130
  - 50% Pharma-Biotech-venture capital-consulting
  - 36% Academia
  - 7% patient support groups
  - 7% others
- Registered visiting countries 21  
Austria, Belgium, Bulgaria, Canada, Denmark, Estonia, Finland, France, Germany, Ireland, Italy, Japan, Malta, Netherlands, Pakistan, Portugal, Sweden, Switzerland, United-Kingdom, United-States

# Conclusion

- Very useful to provide accurate information on on-going research activities
- Of interest to all users
- Impact to be judged after a few years
  - True impact in France where the project originated
- Challenges: networking, partnering, optimisation of existing resources +++

# Conclusion

Gathering information requires expertise and long-term efforts

↳ Only possible at the EU level  
Networking of expert National teams

Effective research and development

↳ Only possible at the EU level  
Basic and clinical

EU organisation of health care system

↳ Network of clinical laboratories  
Reference centres  
Transborder flow of patients



**JOIN ORPHANET !**

**THANK YOU FOR YOUR ATTENTION**

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