



# Health & Consumer Protection Directorate General

The background features three rounded rectangular panels. The left panel shows a white silhouette of a person running. The middle panel shows a green apple with a bite taken out of it, and a branch with leaves. The right panel shows a green apple with a bite taken out of it, and a shopping basket filled with various items.

**EU Public Health Programme  
and action on rare diseases**

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# Legal background

- As regards public health, the Treaty calls on the Community to act in prevention of major public health scourges, and to co-ordinate its activity in this area.
- The Treaty also includes provisions on regulation of products (pharmaceuticals) and on research.

# Action Programme 1999-2002

- The first specific action programme to co-ordinate Member State activity on rare diseases was adopted in 1999. (Decision 1295/99/EC)
- The decision text states that Community measures may contribute unique added value to the treatment of problems the scale of which in individual countries is too small to allow necessary analysis or effective intervention.

# Action Programme 1999-2002

- As a guide, the Decision took low prevalence to be less than 5 per 10,000 in the Community.
- Because of their rarity, people affected by individual rare diseases lack information about them and do not benefit from health resources and services which they need.

# Action Programme 1999-2002

- The programme aimed to improve information and understanding of rare diseases
- It aimed to foster a wider dissemination of information particularly to patients and carers
- It aimed to take a coherent approach with other EU initiatives on orphan drugs and medical research

# Action Programme 1999-2002

- A first objective was to promote the development of a European information network on rare diseases. The information was to comprise a description of the disease, prevention measures, treatments, research programmes, and a list of sources for further information.

# Action Programme 1999-2002

- A second objective was to contribute to training and refresher courses for professionals in order to improve early detection, recognition, intervention and prevention.

# Action Programme 1999-2002

- A third objective was to promote trans-national co-operation and networking between groups of persons directly or indirectly affected by the same rare conditions, or volunteers and professionals involved.



# Action Programme 1999-2002

- A fourth objective was to support at EU level the monitoring of rare diseases in the Member States and early warning systems for clusters, and promote the networking and training of experts concerned with the handling of rare diseases and with rapid response to clusters.

## Projects supported 1999-2002

- This programme ended in December 2002, having supported 24 projects in total for an amount of 6.5M€ (only 60% of the budget allocated to the programme)

# Projects supported in 2000

- EUROMUSCLENET - MUSCLE DISEASES - PROTOTYPE OF RARE AND DISABLING DISORDERS: CREATION OF A EUROPEAN INFORMATION NETWORK SPK (SCHLOSSPARK-KLINIK KG) (GERMANY)
- EDDNL - EUROPEAN DIRECTORY OF DNA LABORATORIES IPG (INSTITUT DE PATHOLOGIE ET DE GENETIQUE) (BELGIUM)
- ORPHANET 1 : A EUROPEAN DATABASE ON RARE DISEASES AND ORPHAN DRUGS (PHASE 1) INSERM (INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE) (FRANCE)
- INFORMATION NETWORK FOR IMMUNODEFICIENCIES UNIVERSITY OF TAMPERE (FINLAND)
- TEAM 1 - TRANSFER OF EXPERTISE ON RARE METABOLIC DISEASES IN ADULTS (PHASE 1) DEPARTMENT OF INTERNAL MEDICINE - UMC (UNIVERSITY MEDICAL CENTER) UTRECHT (THE NETHERLANDS)
- EAGS - CREATING A EUROPEAN ALLIANCE OF PATIENT AND PARENT GROUPS FOR GENETIC SERVICES AND INNOVATION IN MEDICINE GENETIC INTEREST GROUP (UNITED KINGDOM)
- ORPHAN MEDICINAL PRODUCTS TO THE SERVICE OF PATIENTS AFFECTED BY RARE DISORDERS (PART 1) EURORDIS (EUROPEAN ORGANISATION FOR RARE DISORDERS) (FRANCE)
- EUROCAT 1 : SURVEILLANCE OF CONGENITAL ANOMALIES IN EUROPE (PHASE 1) UNIVERSITY OF ULSTER (UNITED KINGDOM)
- NEPHIRD 1 - NETWORK OF PUBLIC HEALTH INSTITUTIONS ON RARE DISEASES (PHASE 1) CENTRO NAZIONALE MALATTIE RARE - ISTITUTO SUPERIORE DI SANITA (ITALY)

# Projects supported in 2001

- EUROCAT 2 : SURVEILLANCE OF CONGENITAL ANOMALIES IN EUROPE (PHASE 2) UNIVERSITY OF ULSTER (UNITED KINGDOM)
- SCN - EUROPEAN NETWORK ON THE EPIDEMIOLOGY, PATHOPHYSIOLOGY AND TREATMENT OF SEVERE CHRONIC NEUTROPENIA MEDIZINISCHE HOCHSCHULE HANNOVER (GERMANY)
- A EUROPEAN INFORMATION NETWORK ON PAEDIATRIC RHEUMATIC DISEASES PRINTO (PAEDIATRIC RHEUMATOLOGY INTERNATIONAL TRIALS ORGANISATION) (ITALY)
- RARE PULMONARY DISEASES - ESTABLISHMENT OF DIAGNOSTIC CRITERIA AND REFERENCE/TRAINING CENTERS INSTITUTE OF PATHOLOGY, UNIVERSITY OF GRAZ (AUSTRIA)
- NEW COMMUNICATION TECHNOLOGIES (WEB SITE) TO THE SERVICE OF THE RARE DISORDERS NETWORK AND SHARING OF GOOD PRACTICES IN DIFFERENT HELP SERVICES TO PATIENTS, ALREADY EXISTING IN EUROPE (HOT LINE) (PART 2) EURORDIS (EUROPEAN ORGANISATION FOR RARE DISORDERS) (FRANCE)
- A EUROPEAN EDUCATIONAL PROGRAMME ON RARE DISEASES ISTITUTO DI RICERCHE FARMACOLOGICHE 'MARIO NEGRI' (ITALY)
- CAUSE - CHARGE ASSOCIATION AND USHER SYNDROME IN EUROPE SENSE INTERNATIONAL (UNITED KINGDOM)
- ORPHANET 2 (PHASE 2) INSERM (INSTITUT NATIONAL DE LA SANTÉ ET DE LA RECHERCHE MÉDICALE) (FRANCE)

# Projects supported in 2002

- ORPHANET 3 (PHASE 3) INSERM (INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE) (FRANCE)
- ENERCA - EUROPEAN NETWORK FOR RARE CONGENITAL ANAEMIAS UNIDAD DE ERITROPATOLOGIA -HOSPITAL CLINIC I PROVINCIAL DE BARCELONA (SPAIN)
- RARE FORMS OF DEMENTIA ALZHEIMER EUROPE (LUXEMBOURG)
- ESTABLISHING EUROPEAN NEUROFIBROMATOSIS LAY GROUP NETWORK. HEALTH PROMOTION, IMPROVING HEALTH INFORMATION AND KNOWLEDGE FOR NEUROFIBROMATOSIS (NF) IN EUROPE NF EUROPE (EUROPEAN FEDERATION FOR NEUROFIBROMATOSIS ASSOCIATIONS) (BELGIUM)
- PAN-EUROPEAN NETWORK FOR PATIENTS INFORMATION ON RARE DISEASES AND ORPHAN DRUGS (PARD 3) EURORDIS (EUROPEAN ORGANISATION FOR RARE DISORDERS) (FRANCE)
- NEPHIRD 2 - A EUROPEAN NETWORK FOR EPIDEMIOLOGICAL AND PUBLIC HEALTH DATA COLLECTION ON RARE DISEASES (PHASE 2) CENTRO NAZIONALE MALATTIE RARE - ISTITUTO SUPERIORE DI SANITA (ITALY)
- TEAM 2 - ONGOING TRANSFER OF EXPERTISE ON PREVENTION, DIAGNOSIS AND TREATMENT OF COMMON COMPLICATIONS IN ADULTS WITH RARE METABOLIC DISEASES (PHASE 2) DEPARTMENT OF INTERNAL MEDICINE - UMC (UNIVERSITY MEDICAL CENTER) UTRECHT (THE NETHERLANDS)

# Action Programme 2003-2008

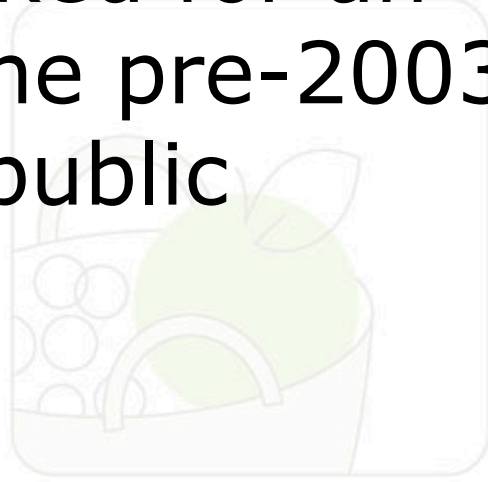
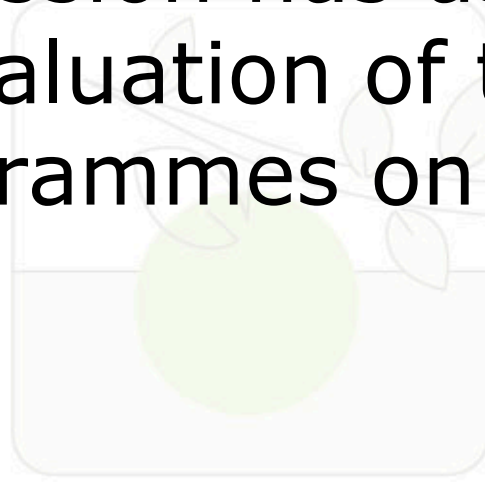
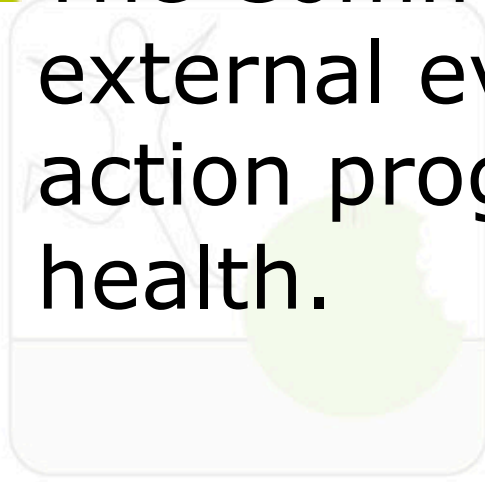
- When the Rare diseases programme came to an end in 2002, it was incorporated in a new overall Community Public Health Programme which will last from 2003-2008 with an annual budget of +/-60M€. The issue of rare diseases is covered in the health information strand.

## Projects supported in 2003-2004

- Two major rare disease projects were selected for funding in 2003, namely ORPHANET 4 and EUROCAT 3
- 3 projects were selected in 2004:
- ENERCA II Network of Rare and Congenital Anaemias (Fundació de l' Hospital Clínic i Provincial De Barcelona)
- RDTF Scientific Secretariat of Rare Disease Task Force (INSERM France)
- PARACELSUS: Policy action and information on rare diseases in Europe (Eurordis, France)

# Evaluation of EU Action Programmes

- The Commission has asked for an external evaluation of the pre-2003 action programmes on public health.





# Evaluation Recommendations

- As regards Rare Diseases the evaluation recommendations are:
  - Better define the general objective
  - To match the ambitions with financial resources
  - To encourage the Commission to further invest in rare diseases particularly because of enlargement
  - To examine the question of care and treatment
  - Continue paying particular attention to prevalence ratios and incidence figures

## Between now and 2008

- The Commission has established a task force on rare diseases to help with co-ordinating its action in this area.
- It will support a major conference in June 2005 under the Luxembourg presidency of the EU to review projects supported to date and express the political interest in continued focus on rare diseases. A political declaration is planned.
- The EU Public Health Programme will continue to support projects on rare diseases in its annual calls for proposals