

European Strategy on Rare Diseases is Adopted by Health Ministers of the EU

A European strategy that calls upon Member States to implement national plans for rare diseases, before the end of 2013, was adopted by the Council of Health Ministers of the EU today (Tuesday, June 9, 2009).

The Council Recommendation represents an important milestone for rare disease patients here in Ireland and all over Europe. Its adoption means that patient representatives will be increasingly involved in the rare disease strategies of each Member State and will be in a position to monitor the implementation of key issues they face, namely support for the social dimension of care and funding for research and patient-led activities.

The Council Recommendation is important because it calls for concerted action at EU and national level in order to:

- Enhance **research** in the field of rare diseases
- Ensure that rare diseases are adequately **coded and classified**
- Identify **Centres of Expertise** and foster their participation into **European Reference Networks (what's a European reference network?)**
- Support the **pooling of expertise** at European level
- Share assessments on the clinical added value of **orphan drugs (what's an orphan drug?)**
- Foster **patient empowerment** by involving patients and their representatives at all stages of the decision-making process
- Ensure the **sustainability** of infrastructures developed for rare diseases.

This adoption marks the culmination of a series of legislative declaratory acts which have paved the way towards the recognition of rare diseases as a public health priority and as an area of unique European added-value for Community action.

The European Rare Disorders Organisation (EURORDIS) and the Irish national alliance the Genetic & Rare Disorders Organisation (GRDO) have been at the centre of this process, voicing the patients' demands for a European policy framework for rare diseases. Starting with the successful *Public Consultation on Rare Diseases* in November 2007 (resulting in the largest response to any such call in EU history) followed by the adoption of the *Commission Communication on Rare Diseases*, in November 2008 and today the adoption of a *Council Recommendation on a European Action in the field of Rare Diseases*, each step has demonstrated the vital importance of EU action, as well as cooperation between Member States.

In Ireland over 140,000 people are affected by rare diseases some are familiar to us such as Cystic Fibrosis, Motor Neuron Disease, Duchene Muscular

Dystrophy, and Huntingdon's disease. 50% of adult cancers and all childhood cancers are considered rare. Some conditions we have heard less about such as the chromosomal deletion disease 22q11, Sarcoidosis, Fabry Disease, Cystinosis and Retinitis Pigmentosa To be considered rare, a condition should not affect more than a limited number of people out of a whole population. In Europe, this is no more than five in 10,000.

Chairperson of GRDO Ms. Avril Daly welcomed the recommendation as a positive step towards making rare diseases a public health priority in this country.

She said: "There is a misconception that few people are affected by rare diseases in Ireland as when you take look at each condition individually the numbers are few. The reality is that 6-8% of the population is or will be affected by a rare disease in their lifetime. This gives us a figure of 140,000 people – which is a significant number by any standard. Most cancers, including all cancers affecting children, are rare diseases. 75% of rare diseases affect children and 30% of patients die before five years of age making access to a proper diagnosis and appropriate treatment a public health priority.

Mrs Judy Windle secretary of GRDO added "This recommendation has been a long time coming and the Genetic & Rare Disorders Organisation has represented the needs of Irish patients every step of the way. We hope the adoption of this recommendation today will pave the way for the establishment of centres of excellence in the treatment of rare disorders and underpin the importance of sustaining the vital research needed to produce therapies for unmet medical needs. We are encouraged to see priority given to the empowerment of patients across the Member States to ensure they play a part in decision making that will ultimately affect their quality of life."

GRDO, together with all other interested parties, will follow-up on the implementation of Council Recommendation at the European and national level.