



CITIZENS' SUMMARY

EUROPEAN ACTION IN THE FIELD OF RARE DISEASES

WHAT IS A RARE DISEASE?

- A life-threatening or chronically debilitating disease affecting no more than 5 people per 10 000 (in the EU).
- That still means there are some 246 000 persons per disease in the EU. With 5 000 - 8 000 known rare diseases affecting up to 6% of the total EU population at one point in life, this equates to 29 - 36 million patients.
- Most are genetic diseases, but also included rare cancers, autoimmune diseases, congenital malformations, and toxic and infectious diseases.

WHY THIS INITIATIVE?

Rare diseases need a joint approach to effectively improve patients' quality of life and their socio-economic potential, as well as to prevent premature death.

At the moment, accurate and timely diagnosis and access to treatment are hampered by 3 main problems in the EU:



- Lack of recognition and visibility

Often rare diseases do not show up in healthcare information systems because there is no appropriate coding and classification.

- Lack of national policies

Limited resources (expertise) are available for rare diseases and these are often fragmented - it is essential to have a specific plan to concentrate and efficiently use these resources.

- Lack of effective infrastructure (healthcare, research, regulation).

There are few reference networks and poor access to care, resources and expertise (often only available in another country).

Despite the very close link between research and possibilities for diagnosis and treatment, research is highly fragmented, leading to inefficient use of limited resources.

WHY IS ACTION BEING TAKEN AT EU LEVEL?

- EU Member States need to cooperate and coordinate their efforts - having 27 separate national approaches is inefficient and ineffective. This is especially important for rare diseases, because patient numbers are low and resources are limited and scattered.
- Acting at EU level can result in economies of scale and support the efforts made by each country.
- European action is also needed to maintain solidarity and shared commitments to the health of rare diseases patients throughout the EU.



WHAT IS THE INITIATIVE (GOALS AND MEASURES)?

The European Commission issued a Communication on Rare Diseases in November 2008, with specific measures to tackle the three main problems highlighted above.

- Greater recognition and visibility

The Commission will ensure rare diseases are better codified and classified by cooperating closely with the World Health Organisation in revising its International Classification of Diseases.

- Support for national policies

The Commission has proposed an EU recommendation that governments establish specific national strategies to deal with rare diseases - and will support them in doing so.

- European cooperation, coordination and regulation

The Commission will better coordinate at EU level both national policies and programmes and EU programmes, to maximise efficient and effective use of resources.

HOW WILL THESE MEASURES HELP YOU?

- They should improve the lives of people affected by rare diseases - patients, but also the families and friends.
- A better coding and classification system will help patients and their families to understand their disease so they can discuss them with their friends, relatives, carers and doctors.
- Better access to healthcare for rare disease patients.
- More efficient research to develop more effective treatments.
- Patients will be able to access expertise from other EU countries more easily.



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