

Public consultation Rare diseases: Europe's challenges

General remarks

The Dutch Genetic Alliance VSOP, is the national umbrella organisation for rare, congenital and genetic diseases and conditions in The Netherlands, with 55 patient organisations as their members. VSOP fully supports the initiative of DG Sanco to propose a Commission Communication and a Council recommendation on rare diseases. More European coordination and priority setting is urgently needed in this field.

At a national level, VSOP is a member of the Dutch Forum for Biotechnology and Genetics and supports their reaction to this consultation. At a European level, VSOP is a member of both the European organisation for rare disorders, Eurordis, and the European Genetic Alliances' Network, EGAN, and supports their contributions as well. As both organisations already sufficiently dealt with the questions in the consultation paper, VSOP would like to give her view on the priorities that are required in the rare disease field.

Preventive Priorities

Most of the rare diseases originate from genetic and congenital causes. It is therefore of utmost importance that perinatal education and screening programs, especially in the preconceptional and neonatal phase, are stimulated and implemented in both national and European programs. There is no justification for the current huge differences in educational and preventive services between the different EU member states.

Research Priorities

Research priority should be given to the setting up of patient registries, data- and biobanking and the stimulation of more cooperation in this field. A pre-competitive data/biobank infrastructure is absolutely necessary to attract more attention from science and the biopharmaceutical industry for rare diseases. Patient organisations should have a role in the governance of the data/biobanks and may contribute to their development and success by involving their members. Adequate care is strongly connected to adequate research.

However, funding for research as well as care for rare diseases is currently fully inadequate in most of the EU member states.

Health Care Priorities

With regard to the health care for people with rare diseases, priority should be given to the setting up of multidisciplinary centres and networks of excellence that are linked with ongoing international scientific research on the one side, as well as with primary health care on the other side. Within the national systems, these expert teams should have access to structural funding, instead of being dependent on the incidental project funding by governments, funds or charities.

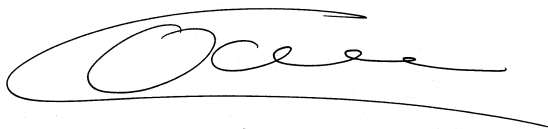
Priority for Patient Organisations

The added value of more patient participation and governance in research and health care policies for rare diseases has been proven to be essential and of major importance. However, this is still not fully recognised by biopharmaceutical science and industry, governments, regulators etc. Therefore, in all policy and funding programs for rare diseases, patient involvement should be structurally included.

European and international priorities

Priority should be given to support for those EU member states with a poorly developed health infrastructure for rare diseases. North-Western Europe should be more active in working together with, supporting and networking with other European regions. In addition, in international developmental health support programs, care and prevention for rare diseases must be included from the beginning.

On behalf of VSOP Dutch Genetic Alliance,

A handwritten signature in black ink, appearing to read 'Cor Oosterwijk', with a long horizontal flourish extending to the right.

Cor Oosterwijk PhD
director