



III Congreso Internacional de Medicamentos Huérfanos y Enfermedades Raras

Sevilla, 14 al 17 de febrero de 2007

III International Congress of Orphan Drugs and Rare Diseases

Seville, 14 to 17 of February 2007

Conclusions

1. The real needs of the patients with rare diseases are not still sufficiently covered by the Public Health Care System, mainly because the specific needs of their pathologies are not fully contemplated
2. An agreement was reached to create a general registry of patients through the launching of a programme that identifies those people affected by rare diseases at the Primary Health Centres. This register would allow both family doctors and paediatricians working at the Primary Health Care System to gain a better knowledge of the main needs of patients with rare diseases
3. Establishing European Networks of Centres of Reference (ENCR) should be preferably addressed towards the existing networks of experts, rather than centres themselves. On the other hand, the concentration of some rare diseases in centres of reference, would enable to gain experience, to reduce the variability of the results and at the end to increase the efficacy of the centre and/or the professionals.
4. Whereas technologies assessment methods are going to play a relevant role in the decision making about the adoption, dissemination and usefulness of the new technologies, the selection process for evaluating should be carefully designed.
5. Health can be neither conditioned by economic interests nor to be analysed only from a market perspective, since health is a fundamental and basic human right.
6. The informed consent given by the patient's family to participate in a clinical trial of an orphan drug, should not be only based on the hope of getting a treatment to curate or mitigate the rare disease, but to obtain a better quality of life of the patient too.
7. It was proposed to elaborate an information exchange system between primary health and specialised care units.
8. The use of orphan drugs is still a difficult field even for specialist doctors: knowing their existence, the lack of experience on their use and particularly to overcome the administra-

tive efforts for saving in pharmaceutical costs are the main obstacles to satisfactorily use orphan drugs.

9. The gene therapy still plays a basic role in the future treatment of rare diseases, although presently the research on this field is less than 25% of the total of therapeutic research.
10. Pharmacies facilitate the so call “magistral prescription” which is a very efficient way to elaborate and provide orphan drugs according to the specific needs of each patient.
11. Many rare diseases are the cause of physical disabilities that could be mitigated by physiotherapy, improving remarkably the patient’s quality of life. However, the access to these kind of services among the public health system is very limited for patients. Governments should have a much deeper concern and commitment to offer these kind of health services.
12. There are still very important inequalities between the Autonomous Communities regarding social benefits and health care they offer to the patients. These inequalities are based on different criteria used for the acknowledgment of the disability level, neonatal screening or pre-implantation diagnosis among others.
13. It was proposed to create adult units at the hospitals for specific rare diseases. It was also suggested to establish a network of “shelter houses” for these patients in those cities where there are reference centres. This housing network would support and promote specific actions in the field of educational and labour integration of the affected people and to promote the research of new therapies.
14. The Autonomous Communities (regions) are promoting and activating social and health care models on which education plays a crucial role. These models should be specifically applied to those children with special care needs without any exception. These actions should be taken by all kind of professionals and sectors involved in this area with an interdisciplinary approach.
15. The difference in the authorization of orphan drugs between Europe and USA lays primarily in the greater experience of the drug registry system in the USA as well as some differences in the designation criteria used in Europe and the USA.
16. Knowledge and treatment of the “caregiver syndrome” of patients with rare diseases should be improved
17. The aim of FEDER (Spanish Federation of Rare Diseases) is to contribute to the development and improvement of life conditions of the affected people throughout the promotion and support of medical and pharmaceutical research programmes, the encouragement of the association of patients and their families and the spread of all the existing information about these diseases
18. Private altruistic initiatives, such as “Fudación Inocente, Inocente” are collecting funds for helping those institutions that dedicate all their efforts to children with physical and psychological problems and/or discrimination and social exclusion, in order to involve and to raise awareness in the Spanish society of the problems and difficulties they are facing.

19. Rare diseases are one of the priorities of the current European Union Public Health Programme 2003-2008, throughout the promotion of the exchange of information and coordination at European Community scale.
20. It is needed to defend the rights of the patients in Spain in the same way as it is happening in neighbouring countries.
21. The feeling of loneliness of the patients with rare diseases and their caregivers finishes when they know that there are other patients with the same disease or similar problems. The creation of new associations in conjunction with the expansion of these associations to higher level organizations is an important and valuable help for patients
22. We express our support to the Rare Diseases Statement of the Spanish Senate, including the creation of a State Organization for Rare Diseases that contemplates the reorganization and improvement of the coordination of activities related with rare diseases. For this purpose it is indispensable to provide the appropriate public financing.
23. We express our hope that in the next congress of Orphan Drugs and Rare Diseases we will show and evaluate the progresses achieved in the development and implementation of the conclusions included herewith.