A STUDY ON TWENTY-SEVEN RARE DISEASE NETWORKS IN EUROPE

identified through EU reports and scientific articles

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OBJECTIVE

To review the existing and the formerly established, but no longer functioning, rare disease Networks in Europe, collecting relevant data regarding their set-up and functional status, with a view to establishing European Reference Networks, in the framework of EUROPLAN (WP4 of EUCERD Joint Action). EUROPLAN (European Project for Rare Disease National Plans Development) was launched in 2008 and foresaw 2 implementation phases: phase 1 (2008-2011) to build the consensus definition of operational tools (recommendations and indicators) and the phase 2 (2012-2015), embedded in the EUCERD Joint Action, with the main goal of establishing an interactive network of stakeholders to support Rare Disease National Plans through scientific and technical assistance.

METHOD

Networks have been a) identified through the analysis of institutional documents, b) described using a table listing their characteristics for every network, c) validated by their Coordinators (each compiled table was sent to the Coordinator for data validation).

RESULTS

Twenty-seven networks have been identified through several documents such as EU reports and scientific articles (e.g. "EUCERD Report: Preliminary analysis of pilot European Reference Networks for Rare Diseases", May 2011) *[Fig. 1. Ongoing and ended Networks by starting year]*.

Data has been a) collected through research reviewing the Networks' websites, the European Commission website and related documents, and b) reported in individual tables for each Network. Every table included the following items: a) Full name & acronym of the Network; b) Name of the Scientific Coordinator/Project Leader; c) Funding sources (past and present); d) Objectives of the Network; e) Year when Network was established; f) Phases; g) Is the Network still operational? h) List of partners participating in the Network; i) List of Countries participating in the Network; j) Rare disease or groups of rare diseases covered by the Network; k) Characteristics of Centres participating in Network activities; l) Website address; m) Other sources of information (for the purpose of this review); n) Any other comments. Twenty-three single tables out of the 27 Networks have been validated by the Coordinators or Project Managers: 1. CHD PLATFORM; 2. DYSCERNE; 3. ECORN CF; 4. ENCE CF-LAM-LTX; 5. ENERCA; 6. EN-RBD; 7. E-Pilepsy; 8. ESDN; 9. EUHANET; 10. EUROCARE CF; 11. EuroCYST Initiative; 12. EUROGLYCANET; 13. EURO HISTIO NET; 14. EUROSCA; 15. EUROWILSON; 16. EXPO-r-NeT; 17. GENESKIN; 18. LEUKOTREAT; 19. PAAIR; 20. Paediatric Hodgkin Lymphoma Network; 21. PRINTO; 22. TAG; 23. TREAT-NMD.

Tables of four Networks (CARE NMD, EPNET, EUROCRAN and NEUROPED) have been not validated. Of the 23 established Networks, 12 were operational by September 2015. Data analysis of the 23 Networks highlighted relevant information on diseases & group of diseases in which each of the 23 Networks specialised, their geographical coverage, and their setting-up under the leadership of institutions belonging to 8 Countries, 7 of which are EU Member States.

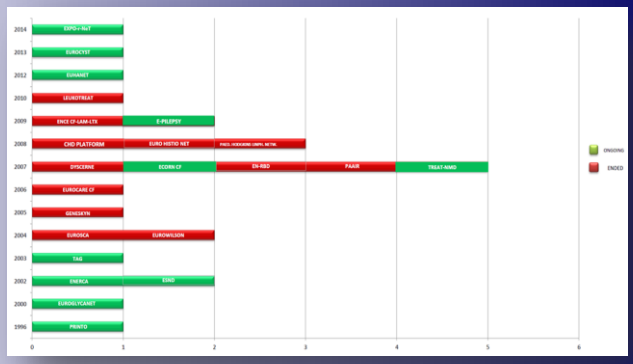

Fig. 1. Ongoing and ended Networks by starting year.

CONCLUSION

Member States and European Commission began exploring the necessity for European Reference Networks in 2003. A process started that culminated with the adoption of the Directive 2011/24/EU on patients' right in cross-border healthcare that provides the legal basis for establishing a system of European Reference Networks of highly specialised healthcare providers.

The issue regarding the ERNs has been treated in several documents including European directives, reports, recommendations, scientific papers and conference presentations. The present study intends to be a starting point for a panel discussion to identify the areas of strength and the critical issues for Networks in Europe. There is a fair track record in establishing rare diseases Networks in Europe dating since 1996. The experience thus far gained is interesting despite some lights and shadows, both valuable at a time when important decisions need to be taken. More so, when such decisions are to be taken in the face of mounting needs and scarce resources to be invested into a system that must necessarily be effective, efficient and sustainable.

There are successful and unsuccessful stories regarding the maintenance of Networks and both must be further studied to understand which are the success factors enabling Networks to survive and thrive, and what prevents this in order to optimise the first and minimise the second. So far, 12 out of 23 Networks were still operating by September 2015: a few have been newly established and others have survived the initial investment while others have stopped operating most probably for luck of funds as declared by the Coordinator. Of the 12 still active, a few have been at risk of not surviving the project phase. If a lesson can be learned from this experience, it is that sustainability not only financial but also in terms of other resources (skilled personnel, technology…) is one of the key elements of the guiding principles in the establishment of a Network.

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