EURORDIS MALTA DECLARATION

On the occasion of the Conference on Development and Access of Medicines for Rare Diseases, Malta, 21 March 2017

Rare diseases represent an area with high European added value, for which the most effective strategies are cross-border and EU-wide, with an international perspective. A strong track record of achievements on rare diseases has been established through cross-country collaboration and the support of the European Union.

People living with a rare disease face significant challenges. Merely 5% of rare diseases have therapies to date - few curative, some transformative, most improving and prolonging life. Limited, scattered patient populations and expertise exacerbate inequalities in accessing quality and appropriate medical and social care between countries and regions. Working in synergy can help mitigate these challenges.

The remarkable technological and scientific advances of the past years unlock the potential of diagnostic tools for rare diseases and present an exponential increase in novel technologies and therapies on the market. These new opportunities embody hope for patients, but in parallel bring about concerns in terms of access and of sustainability for health budgets. Recent developments in IT and infrastructures, as well as legislative and regulatory requirements that are conducive to greater data generation, allow collection, processing and standardisation of large datasets that were not previously available. Better cooperation is necessary to tap at this potential, while in parallel addressing the ethical and legal matters that are generated.

In this landscape, cross-border cooperation is needed to ensure that patients have access to the diagnosis, care and therapies they need and deserve. In order to achieve this, cooperation must be enhanced in a strategic and structured way.

Structured cooperation in healthcare for rare diseases

Care for rare diseases involves a complexity of diagnosis, treatment and management that needs to be provided by highly specialised professionals and hospital units. European Reference Networks (ERNs) create a clear governance structure for cross-border knowledge sharing and care coordination, linking nearly 1000 healthcare providers in EU countries. As a result, both health professionals and patients may have easier access to expertise on rare diseases beyond their national borders. This promotes clinical excellence by breaking isolation of patients and reducing inequity in care while in turn facilitating patients' access to better diagnosis, treatments and care pathways in their home country.

ERNs significantly impact knowledge generation through collective peer learning within each disease network. A natural environment where voluntary cooperation in the area of training can take place is created, by embedding training opportunities and exchange of best practices within the networks, with the support of the EU and Member States. Continuing education, recognition of qualifications, cross-border post-graduate exchanges and other emerging opportunities are to be applied to all workforces, from clinical specialists to nurses, paramedics to research assistants, IT specialists to managers, and centres coordinators to patient representatives.

Patient healthcare pathways need to be anchored in national health services. At the national level, support is necessary to ensure that suitable processes for the identification and designation of centres of expertise and healthcare providers are established. Adequate IT infrastructures need to be developed and connected to the IT platform developed by the European Commission in support of ERNs, adopting common interoperable standards.

Therefore, we recognise:

i. that structured cooperation is a voluntary and organised cross-border activity between actors in the healthcare sector, designed to improve patient access to high-quality diagnosis and care;

ii. that the establishment of 24 European Reference Networks on 1st March 2017 is a point in history that revolutionises specialised care by connecting patients, experts and hospitals;

iii. that a qualified health workforce often needs to be trained across borders;

iv. that full integration of ERNs into national healthcare systems is important to realise the value that EU cooperation in healthcare policy can bring to European citizens;

v. that it is crucial to ensure European cooperation on EU-wide patient registration, patient registries and data collection in the context of ERNs, to maximise the Networks' potential, to trigger new standards and clinical guidelines, to facilitate research and to boost innovation in development of therapies.

Structured cooperation in research for rare diseases

Research on rare diseases has taken massive steps over the last 10-15 years. Scientific and technological advances, including in the field of genetics, may raise societal and ethical questions. One potential issue is how to address the deepening of inequalities between regions with respect to accessing improved medical care through outcomes of scientific research.

Expertise on rare diseases is scarce and research cannot be performed in isolation. The RD-Connect project that links rare diseases databases, registries, biobanks and clinical bioinformatics data into a central resource for researchers worldwide, is a prolific example of structural support to research activities. Pooling of resources is essential to avoid duplications and also to optimise the use of funding, infrastructures and technological platforms. Initiatives such as E-Rare and IRDiRC contribute to reducing fragmentation of rare disease research resources and achieving critical mass of data and samples on a European scale. The traditional short duration of contracts for rare disease research funding hampers sustainability and the development of shared common research infrastructures.

Therefore, we recognise:

i. that cooperation in the field of research can bring about significant results that have a positive impact on the lives of people with rare diseases;

ii. that the time has come to step up collaborations with long-lasting and integrated support to rare disease research;

iii. that reinforced public funding both at the EU and national level would ensure the long-term continuity of research for rare diseases and limit the loss of precious funds;

iv. that integrated action and collaboration, at the national and European level, is needed to better coordinate both policy and funding within a strategic framework and that the potential of ERNs to embed and boost research activities should be exploited.

Structured cooperation for improved patient access to therapies and innovative technologies

The last few years have seen a sharp increase in the interest of Member States in new avenues for stronger cooperation at all levels, from horizon-scanning and joint HTA to greater information sharing and joint pricing and reimbursement negotiations. Collaboration platforms like BENELUXA have firmly demonstrated the viability and potential for success of such voluntary approaches, which can complement and bolster national competences.

In light of the specific challenges of rare diseases, posed by extremely small patient populations scattered across the continent, the often high cost of acquisition for national healthcare systems, and by the very innovative nature of the medicines, collaboration among Member States is paramount.

Therefore, we recognise:

i. that initiatives ought to be consolidated into a structured European approach, which fast-tracks R&D, brings down the costs of medicines development and increases productivity to deliver more affordable treatments;

ii. that the concept of a European approach facilitates collaborative procurement strategies, bringing together willing national competent authorities and the European Commission on issues of pricing and reimbursement, for improving access to innovative health technologies;

iii. that this unified European approach shall facilitate the negotiation of fairer prices, better related to the real value of a medicine, and to its level of clinical uncertainty; public authorities in all Member States, and most particularly the 22 Member States with a population of less than 20 million, are encouraged to give their consideration to this proposal.

We welcome the efforts of the Maltese Presidency of the EU Council to identify the mechanisms of enhancing access to improved diagnosis, care, therapies and technologies for rare diseases that have been discussed today. We hereby adopt this declaration in support of increased and strengthened structured cooperation between EU Member States to achieve the overarching objective of meeting the needs of people living with a rare disease.