Let's make a pact to ensure patients' sustainable access to rare disease therapies

3rd EURORDIS Multi-Stakeholder Symposium on Improving Patients' Access to Rare Disease Therapies, 13-14 February 2019

Draft Roadmap Document

Introduction

Since the 1990s, rare diseases have been a policy priority at both European Union (EU) and Member State (MS) level. A number of countries led the way in the decade leading up to the first European legislative text concerning rare diseases -the Orphan Medicinal Product Regulation of 16 December 1999 - and the subsequent Commission Communication (2008) and Council Recommendation (2009).

In the last decade, the European rare disease scene has changed significantly, with many notable successes. There are many reasons to celebrate the progress of Europe's rare disease community:

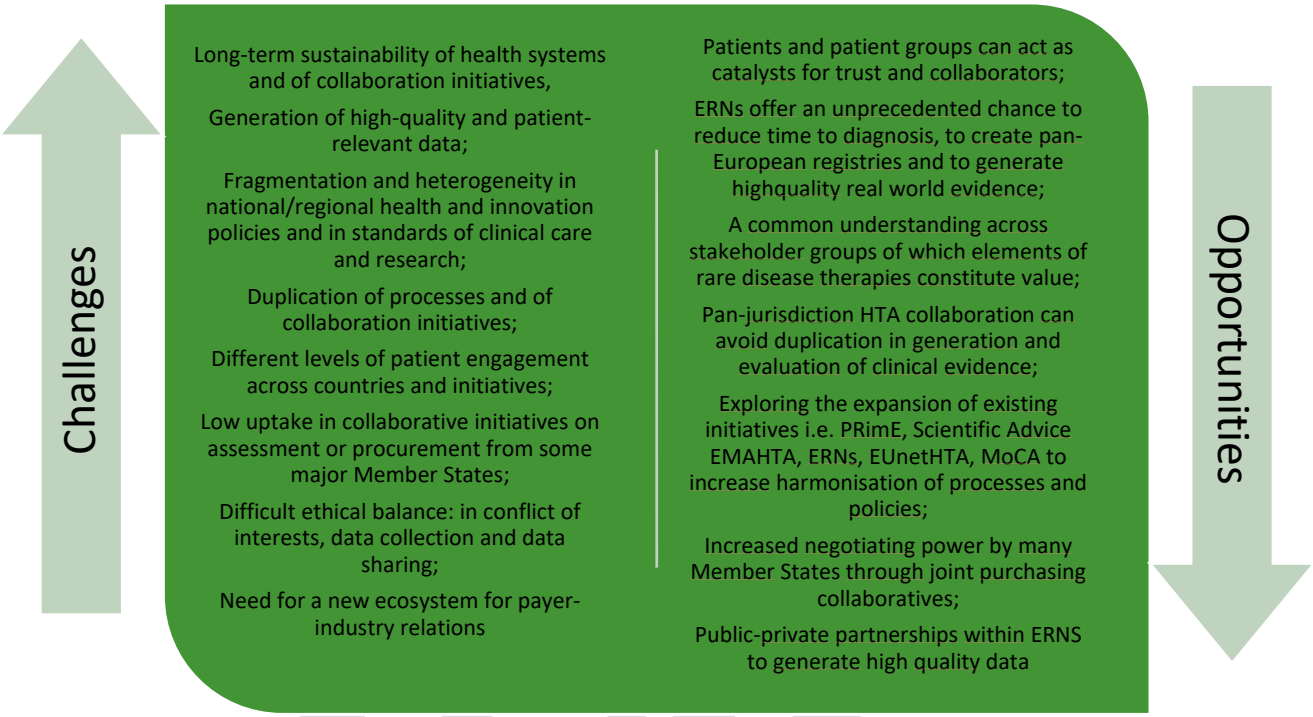
- 25 EU Member States have now adopted a national plan or strategy for rare diseases, compared to only 4 in 2008. The focus has also shifted from 'adopting' to actually implementing and evaluating the success of these first (and in some cases second) plans.
- 2016 saw the approval and official 'birth' of the long-awaited European Reference Networks for rare diseases – 24 ERNs are now operational, an unprecedented success, and opportunities for these Networks to add value in the rare disease field continue to emerge;
- Collaboration between the 'healthcare' and 'research' domains is increasing, and will continue to grow (bolstered by the imminent European Joint Programme Co-Fund for Rare Disease research).

However, alongside these successes, the rare disease community is now facing a number of challenges that have the potential to halt the rate of progress and threaten the continued advancement of diagnostics, treatment and care for people with rare diseases in Europe. In particular, despite these achievements, there is still much to be done to ensure that people suffering from a rare disease can obtain the best possible treatment throughout the EU.

The EU Regulation on Orphan Medicinal Products 1999 enshrined in the European health regulation the right for people suffering from rare conditions to be entitled to the same quality of treatment as other patients and it has been a success in fulfilling its primary purpose - to attract investment to the development of therapies for life-threatening or debilitating diseases for millions of people who today have either no treatment at all or no satisfactory treatment. However, despite successful progress, the original ambitions remain far from being fully achieved, especially when considering patient access to approved orphan medicinal products across Europe.

A Multi-Stakeholder Approach to Break the Access Deadlock

The two previous EURORDIS Multi-Stakeholder Symposia (in 2016 and 2017) advanced our understanding of the challenges and opportunities, as well as the areas of agreement and divergence, that shape the future of development and access to rare diseases therapies.



This Third Symposium puts these challenges and solutions into context of the changing landscape in healthcare decision-making and delivery of innovative therapies for rare diseases, and challenges us again to develop feasible strategies of how the different stakeholders in the rare disease community can contribute to sustainably improving patients' access in this context. This draft 'roadmap' will serve to *guide* European and national institutions alike to improve the development of, and access to, therapies at all levels.

A Roadmap for the Future

This draft roadmap is focused on the four pillars highlighted in the EURORDIS Position Paper 'Breaking the Access Deadlock to Leave No-one Behind' and is based on discussions held during the breakout sessions at the Third EURORDIS Multi-Stakeholder Symposium in February 2018.

1. A New Blueprint to Cut Costs and Fast Track R&D

Rare diseases R&D in Europe can be improved to overcome fragmentation, leading to more effective use of data and resources, and faster scientific progress to improve the lives of people living with a rare disease. In this specific context, it is timely to maximize the potential of already funded tools, networks, projects and programmes, by further supporting them, scaling them up, linking them, and most importantly, adapting them to the needs of end-users through implementation tests in real settings.

Suggested recommendations :

- Public funders, the European Commission and Member State competent authorities in research policy should all support a much greater integration of care and research.
- EU public authorities need to ensure greater understanding and consistent appreciation of innovative methods with an alignment across decision makers.
- Product developers should consider the specific methods for clinical trials in small populations as the first and preferred approach, while seeking parallel European scientific advice from the EMA and HTA bodies at an early stage.
- European research infrastructure servicing across all ERNs, and clinical research networks per disease or per group of diseases, should be developed.
- All patients who receive a consultation through an ERN at any point should be registered in order to accelerate patient identification and clinical trial recruitment, and to initiate more disease registries and facilitate more upstream therapy development.

2. Improving Multi-Stakeholder Early Dialogues to Optimise Determination of Value

Early dialogue and scientific advice initiatives have traditionally provided authoritative advice to medicine developers on specific questions about their confirmatory clinical trial(s). Over the years, advice from regulators and scientific experts has evolved to be more of a two-way dialogue that can include patients and a range of

agencies such as HTA/payers and to consider issues beyond the confirmatory trial. Such dialogue is essential in the development of treatments for rare diseases where traditional clinical development programmes may not be possible and where wider evidence sources may be needed to determine the value of a treatment. Patients' views need to be incorporated to focus on outcomes that matter and ensure study feasibility.

Suggested recommendations :

- Early dialogue at the European level should be encouraged for a much greater number of rare disease therapies under development, with the due involvement of patients and medical specialists.
- Relevant authorities (national and European) must ensure that all existing platforms for early dialogue can, and should, be coordinated with one another.
- EMA scientific advice and protocol assistance should be proactively promoted to the attention of developers of designated drugs, in an open dialogue on all non-conventional and adaptive methods.
- Parallel EMA-HTA scientific advice should become the preferred procedure for all product developers in the field of rare diseases as soon as possible.

3. A Transparent European Cooperation Framework for the Determination of Fair Prices and Sustainable Healthcare Budget Impacts

Full and quick access to therapies remains difficult across Europe for people living with a rare disease. Difficulties in negotiating the monetisation of the value of these therapies is one of the key issues to address, one where increased collaboration at European level could bear fruit. In light of the specific challenges of rare diseases, that affect extremely small patient populations scattered across the continent, the often high cost of acquisition for national healthcare systems, and because of the very innovative nature of the medicines, collaboration among Member States is paramount.

Suggested recommendations :

- Dialogue between payers and product developers at the European level (such as the MoCA pilots) should be encouraged. Scaling up the experiments that have taken place to date requires political encouragement and financial support to lead to a truly European collaborative effort, within the opportunities provided by the current European legislation.
- Member States should consider on the basis of MoCA experience as well as on the existing multi-country collaboration the establishment of a common European "Table of Negotiation", and consider committing to develop, in an open multi-stakeholder format, the innovative approach to lay out a more transparent pathway to the construction of prices.
- EU Member States on board the "Table of Negotiation" should commit to considering discounts for uncertainties, payments based on outcomes, and all other appropriate modalities or techniques so as to provide early access for patients' to medicines approved under exceptional circumstances, under conditional approval, at the end of stage 2, or in any other situation when uncertainties are high or significant

4. A Continuum Approach to Evidence Generation Linked to Healthcare Budget Spending

Orphan medicines pose many different challenges to competent national authorities for pricing and reimbursement, not least as they increasingly tend to arrive to the time-point of marketing authorisation with higher levels of uncertainty on efficacy and effectiveness. With increasing available data set at European and national level that allow for better understanding of diseases and their treatments, their effectiveness, there is the possibility now to feedback into budgetary considerations, in post-assessment and post-marketing authorisation whilst focusing on health outcomes that are relevant for people living with a rare disease.

Suggested recommendations :

- National HTA bodies and national competent authorities for pricing and reimbursement to more openly consider modalities according to which the price of a medicine coming to marketing authorisation with a high level of uncertainty should not remain fixed and "set in stone", but rather fluctuate upwards or downwards according to the evidence collected from real-life use.
- HTA authorities and experts from all EU Member States should consider new approaches for the health technology assessment of orphan medicines particularly in application to innovative therapies with a high level of uncertainty at the time of marketing authorisation.
- European Commission and all EU Member States should consider proposals for a dedicated "European Fund", the resources of which could help finance the generation of evidence for high uncertainty orphan medicines from the time point of marketing authorisation up to the first reassessment of their value.

Post-event next steps

Once reviewed and adopted by you as participants at this 3rd Multi-Stakeholder Symposium, EURORDIS will share this roadmap with the new European Parliament following the elections in May, European Commission and Council of European Health Ministers calling upon them to sign-up to the roadmap, implement the content and or continue the dialogue in the appropriate fora at European and national level.

References

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