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

3rd Multi-Stakeholder Symposium, 13-14 February 2019

Crowne Plaza Brussels

Concept paper

Rationale & Objectives

EURORDIS-Rare Diseases Europe is organising the 3rd Multi-Stakeholder Symposium in February 2019 to build on the outcomes of the previous multi-stakeholder Symposia held in 2016 and 2017.

We will use the collaborative multi-stakeholder approach in the position paper '*Breaking the Access Deadlock to Leave No-one Behind*' written in January 2018 to develop and garner broad endorsement for a 'roadmap' document detailing a joint vision, offering solutions and stating commitments by all players in the rare disease community aimed at accelerating the development of effective therapies and guaranteeing timely and universal access to them, thereby progressively reducing existing gaps and inequalities.

The timing of this initiative is of the upmost importance as the European Parliament elections will take place in May 2019 and the related changes in the European institutions, in particular at the European Commission, will subsequently take place. The ongoing discussion around the establishment of the Multiannual Financial Framework for the period 2021 – 2027 provides additional opportunities.

Thus, it is essential for the rare disease community to have a clear and shared 'roadmap' that both highlights the critical need for a European vision and offers practical solutions that are implementable by the current and future European parliamentarians by 2025.

Building on previous work

The two previous Symposia advanced our understanding of the challenges and opportunities that shape the future of access to rare diseases therapies.

Challenges	Opportunities
→ Long-term sustainability of health systems and of collaboration initiatives → Generation of high-quality and patient-relevant data → Fragmentation and heterogeneity in national/regional health and innovation policies and in standards of clinical care and research → Duplication of processes and of collaboration initiatives → Different levels of patient engagement across countries and initiatives → Low uptake in collaborative initiatives on assessment (HTA) or procurement from some major Member States → Difficult ethical balance: in conflict of interests, data collection and data sharing → Need for a new ecosystem for payer-industry relations	→ Patients and patient groups can act as catalysts for trust and collaborators → Increased use of FAIR (Findable, Accessible, Interoperable and Reusable) data-entry → ERNs offer an unprecedented chance to reduce time to diagnosis, to create pan-European registries and to generate high-quality real world evidence → A common understanding across stakeholder groups of which elements of rare disease therapies constitute value → Pan-jurisdiction HTA collaboration can avoid duplication in generation and evaluation of clinical evidence → Exploring the expansion of existing initiatives i.e. PRimE, Scientific Advice EMA-HTA, ERNs, EUnetHTA, MoCA to increase harmonisation of processes and policies → Increased negotiating power by many Member States through joint purchasing collaboratives (e.g . BeneluxA + Ireland, Valletta Declaration) → Public-private partnerships within ERNS to generate high quality data

Desired output

The 3rd Symposium aims to put into context the current, changing landscape in healthcare decision-making and delivery, and to develop feasible strategies of how the different stakeholders in the rare disease community can contribute to sustainably improve patients' access to these.

We aim to develop a shared 'roadmap' that could *guide* European and national institutions alike to improve the development and access to therapies at all levels, that can be used to support advocacy efforts for specific actions to be taken by all levels of authority. This 'roadmap' should:

- Have a perspective for the next 5 to 10 years - where we want to be and how we can get there both at national and European levels;
- Focus on the key pillars of EURORDIS' position paper on access, namely:
    1. A new blueprint to spend efficiently and fast-track R&D;
    2. Early dialogue and cooperation between healthcare systems on the determination of value of a medicine and on patient access;
    3. A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impact;

4. A continuum approach to evidence generation linked to healthcare budget spending.

- Include outputs from the first two Symposia and take into consideration the evolution of the external policy landscape, specifically:
    - The launch and establishment of the European Reference Networks (ERNs)[i];
    - The EURORDIS Malta Declaration (March 2017)[ii] under the auspices of the Maltese Presidency of the European Union;
    - The ongoing evaluation of the Orphan Medicinal Product and Paediatric Medicines Regulations[iii];
    - The European Commission proposal for European cooperation on Health Technology Assessment (HTA) (January 2018)[iv];
    - The study on the economic impact of supplementary protection certificates, pharmaceutical incentives and rewards in Europe (May 2018)[v] ;
    - The forthcoming Rare 2030 Foresight Study[vi].

Preparatory work

In order to develop a Pan-European draft document ('roadmap') in time for the meeting in February 2019, a number of preparatory activities needs to be planned and executed. This draft needs to be advanced enough and present sufficient detail to have the capacity to *a)* garner the necessary buy-in by all constituencies and *b)* be endorsed and appropriated by European and national policy makers.

I. Prior activities

The two previous multi-stakeholder Symposia (2016[vii] and 2017[viii]) have outlined the key challenges and potential solutions to break down the existing barriers to patient access for rare disease therapies. The outcomes document of these two meetings, together with the EURORDIS' access paper, should feed into the development of a draft framework roadmap.

The drafting process will require advanced meetings and preparation with a small group of drafters, which should represent all of the various constituencies that will need to buy into the recommendations presented in the draft roadmap.

Furthermore, we should envisage to hold several webinars in January 2019 to introduce the audience to the objectives, and the progress of the draft roadmap prior to the Symposium.

II. On-site activities

The Workshop/Symposium will run for 1.5 to 2 days and will concentrate specifically on assessing the remaining challenges/areas of differing opinion in order to have the roadmap formally endorsed by representatives of all stakeholders in the rare diseases community.

Ideally, the event should consist of the following core elements:

- An opening session with high-level speakers to contextualise where the roadmap sits within the overall framework for investment in life sciences developments for rare disease therapies;
- An overview of the landscape changes, in particular the new political cycle at European level that will start with the elections for the European Parliament (May 2019) and the subsequent appointment of a new European Commission;
- Four short presentations, of 15 minutes each, to introduce each pillar of the strategic framework and outline the remaining challenges;

- Four separate, highly interactive breakout workshop sessions to agree on the final text of the roadmap. Each breakout session will feature two discussants presenting the areas of convergence and the areas where there remain divergences and a moderator to solicit views from the audience;
- Each parallel session will feature a rapporteur who will summarise the outcomes of the discussion in the plenary session;
- The final session will wrap-up the work accomplished and outline the next steps to be taken.

III. Post-event and follow up activities

Once adopted, EURORDIS will send the roadmap (accepted/agreed-upon/endorsed by all stakeholders at the Symposium) to the European Parliament ahead of the elections, 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.

The roadmap will also be disseminated to:

- All Permanent Representations of Member States in Brussels;
- All companies that are members of the EURORDIS Round Table of Companies (ERTC), as well as members of trade associations at European level (EFPIA, EUCOPE and EuropaBio);
- All members of EURORDIS, including national alliances.

Furthermore, during the next legislative term (2019 – 2024), EURORDIS will follow up at regular intervals on the commitments made at European and national levels to check whether the necessary improvements / changes have been implemented, according to the four pillars supporting the new approach.

[i] European Reference Networks (ERNs), European Commission https://ec.europa.eu/health/ern/overview_en
[ii] EURORDIS Malta Declaration | Conference on Development and Access of Medicines for Rare Diseases (March 2017) http://download.eurordis.org.s3.amazonaws.com/21March2017%20MaltaDeclaration.pdf
[iii] Evaluation of the legislation on medicines for children and rare diseases (medicines for special populations), European Commission (December 2017) https://ec.europa.eu/info/law/better-regulation/initiatives/ares-2017-6059807_en
[iv] EU cooperation on Health Technology Assessment, European Commission (January 2018) https://ec.europa.eu/info/law/better-regulation/initiatives/com-2018-51_en
[v] Study on the economic impact of supplementary protection certificates, pharmaceutical incentives and rewards in Europe (2018) https://www.copenhageneconomics.com/dyn/resources/Publication/publicationPDF/5/445/1527517171/copenhagen-economics-2018-study-on-the-economic-impact-of-spcs-pharmaceutical-incentives-and-rewards-in-europe.pdf
[vi] Rare 2030 - a participatory foresight study for policy-making rare diseases, European Commission (call for tender, http://ec.europa.eu/research/participants/portal/desktop/en/opportunities/pppa/topics/pp-1-2-2018.html)
[vii] Conclusions of the 1st Multi-stakeholder Symposium on Improving Patient Access to Rare Disease Therapies (February 2016) https://www.eurordis.org/sites/default/files/symposium-feb2016-conclusions.pdf
[viii] Proceedings of the 2nd Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies (February 2017) http://download2.eurordis.org.s3.amazonaws.com/ertc/Proceedings_Multistakeholder_Symposium_2017.pdf