Roadmap on the European Pharmaceutical Strategy

A response from EURORDIS Rare Diseases Europe

EURORDIS Rare Diseases Europe (www.eurordis.org), a unique, non-profit alliance of 924 rare disease patient organisations from 72 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe, welcomes the European Commission's publication of the roadmap for the adoption of a Pharmaceutical Strategy for Europe. We particularly welcome the intention to build a 'holistic, patient-centred strategy that covers the whole life-cycle of pharmaceutical products'.

Each of the over 6000 identified rare diseases affects a very small population, but altogether they affect nearly 30 million people in the EU, or 5% of the population of Europe [1]. The negative impact of the combination of rarity and scarcity of knowledge cannot be underestimated: the lack of knowledge means that many rare diseases are not diagnosed, that disabilities and other symptoms are not recognised, and that in turn persons with rare diseases do not receive the same support as those with more common and well-known conditions. Ultimately, this effectively bars persons with rare diseases from genuine integration into society [2].

Results on access to treatments from our latest Rare Barometer quantitative survey a of 7.500 respondents across EU and World, presented at our European Conference on Rare Diseases and Orphan Drugs in May 2020 [3] revealed that69% of respondents have already experienced a treatment, but only 5% have already experienced a centrally approved curative treatment. 31% have never experienced any treatment (because there is no treatment, or they could not take part in the clinical trial, or the treatment is not affordable). Great variation of the number of treatments the patient could access depends on the disease area. Many treatments experienced were not centrally approved (e.g. off label use therapies, compassionate use).

Individual countries cannot face alone issues related to rare diseases, notably those related to the access of treatments and the unmet needs of a significant part of the population, as emerged in abovementioned survey. A strong track record of achievements on rare diseases has been established through cross-country collaboration and the support of the European Institutions. European expertise and innovative technologies have been developed and can potentially benefit people living with a rare disease. Most Member States now have national rare disease strategies and specialised centres of care, thanks to the establishment of European Reference Networks (ERNs). Rare disease research is stronger and recognised for its excellence and true multistakeholder collaboration in the European Joint Programme on Rare Disease (EJP RD).

Still, there is a lot left to do. 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 safety, long term efficacy, access and of sustainability for health budgets. New scientific developments, the promise of many new therapies and new diagnostic landscape raised hope, but new challenges in pricing and access of therapies show how existing models are not sustainable. Major difficulties remain in access to the right diagnosis and treatments, which are often too costly for the patients, as well as discrepancies and inequalities amongst Members States. A treatment that is unavailable to patients who need it loses its value.

We also observe discrepancies and inequalities amongst rare diseases with some having benefitted from the specific legislations for orphan drugs, from the recommendations issued by the International Rare Diseases Research Consortium and subsequent research grants, and many others remaining totally disregarded with not even a minimal available body of knowledge. There is a need to keep structuring the field, to sustain the existing networks and initiatives (ERNs, EJP RD) and to build their capacities in terms of Research & Development as it is

the case in the US with the RD-Clinical Research Networks. Rare diseases represent an area with high European added value, for which the most effective strategies are cross-border and EU-wide. With the development of the Pharmaceutical Strategy, Europe has the opportunity to develop a new ecosystem, a framework based on a global approach to innovation for unmet medical needs and on sustainability for healthcare systems as well as financial attractiveness to developers and investors [4]. We have a collective responsibility to shape a new approach which will accelerate the transfer of major scientific advancements into new therapies, in a predictable and sustainable way for society.

Additionally, the COVID-19 crisis showed us the crucial necessity of collaboration at international level, including in terms international regulatory cooperation, in order to align and maximise the resources but also to adapt to new way of developing medicines and delivering care to patients with the help of remote and digital tools.

Alongside the intent stated in the roadmap, we propose some recommendations for reflection and inclusion in the development of the Pharmaceutical Strategy, with specific regards to therapies for people living with a rare disease.

- Ensure that patient perspective is taken into account in the research, development but also regulatory, assessment and appraisal processes for rare diseases therapies. The patient perspective across all stakeholders (researchers, clinicians, industry, regulators and policy makers) needs to be balanced as this voice is crucial in the discovery, development and delivery of innovative treatments. At the end what patients need, is access to effective treatments.

- Reinforce the EU centralised processes of the Regulation on Orphan Medicinal Products together with the regulation on Paediatric Medicines in a structured and seamless way for all rare disease therapies as they do require a "continuum of evidence generation", all through their life cycle, from scientific guidance and assessments until after the moment of marketing authorisation, as well as early patient access to address the unmet needs of conditions which are frequently life-threatening and debilitating. In this sense, the Pharmaceutical strategy should include proposals to coordinate data collection as regulatory tool in the area of Advanced Therapies Medicinal Products (ATMPs), an area which holds a lot of transformative potential but that needs tools that will help surveillance and follow up of the pioneering patients that undergo the innovative treatments [8].

- Support actions on the socio economic burden of rare diseases that would accelerate the research on the new therapies. Accurate data regarding prevalence, impact on individuals and families affected, and cost burden to the Member States' economies is often lacking. This hinders development of new treatments, and the planning and implementation of clinical treatment trials. Thus there is a need for a stronger evidence base to support research, advocacy, care pathways and value for money to regulatory bodies for treatments.

- Consider a reflection process at EU decision making level on how to address the challenges related to patient access to ATMPs in terms of assessment, affordability, availability and accessibility. This reflection shall encompass among others the issues around funding models, sustainability of healthcare systems, education of patients and other stakeholders on the existing treatments, incentives for the EU specialised centers and its workforce [8].

- Strengthen European cooperation in pricing and negotiations, as mechanisms of voluntary cooperation between EU Member States have been gaining in strength over the last few years with the emergence of multi-country platforms of discussions on negotiations of pricing and reimbursement of medicines. Such efforts need consolidating and unifying while respecting current treaty competences, by exploring the feasibility of establishing a European Table of Negotiations, supported by joint procurement initiatives [4].

- Consider initiative to shift from the European Reference Price system currently in use towards a European Transactional Price and use of differential pricing i.e. to develop a common methodology to set a fair price based on a transparent, dynamic, mutually constructed approach to incentivise value and healthcare

priorities, starting from a cost-based price, which would then be adjusted as a factor of the agreed determination of the value of the product (with a view to rewarding high-risk investments as much as genuine healthcare innovation), and of bonus/malus to incentivise private investments in the specific directions called for by healthcare systems [4].

- Ensure legal clarity to facilitate access to treatment across borders for people for rare diseases to approved treatment when not available in their country of origin, as indicated in existing legislation on patients' rights in cross border healthcare; specifically, the Pharmaceutical Strategy should include a roadmap to fulfil the commitments taken with the European Court of Auditors recommendations to assess current rare disease strategy by 2022 [5].

- Consider the initiation of action towards the creation of a "European Fund" to support the generation of additional real-world evidence data in the years following marketing authorisation for selected, innovative and transformative medicines for complex and low prevalence diseases, with true cross-border value. In the suggested Fund, the EU would contribute to a certain percentage (30% to 50%) of the actual cost of the medicinal products so to reduce costs for Member States, whilst maintaining the attractiveness of the EU for investment, research and innovation in life sciences [4].

- Work towards the establishment of an appropriate regulatory framework for repurposed therapies as well as compassionate use at European level, as well as looking at the robustness of the regulation for ATMPs [6].

- Accelerate the adoption of the proposal on EU cooperation on HTA, with the crucial inclusion of patients at all relevant level of decision making [7].

References

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