Life-saving treatments can only save lives if they are accessible to patients!

RARE IMPACT Consortium sets out 7 policy solutions to improve patient access to advanced therapy medicinal products

23 November 2020, Brussels – Today EURORDIS-Rare Diseases Europe, together with the partners of RARE IMPACT initiative, sets out seven solutions to improve access to gene and cell therapies for people with rare diseases in Europe with the launch of a new RARE IMPACT report.

With the advent of advanced therapies, humanity is effectively challenging the inevitability of genetic diseases for the first time and bringing hope and opportunity to people living with a rare disease in Europe and beyond. However, we are confronted with too many barriers to true access.

The report, published today and presented at a multi-stakeholder online event, addresses challenges across four identified areas in the **accessibility, assessment, availability and affordability** of **gene and cell therapies** across the European Union. It highlights seven solutions including calls for:

- **Greater collaboration between EMA, HTA bodies and Heads of Medicines Agencies on guidance on HTA assessment of advanced therapies**, as the complexity of these therapies as much as the new type of uncertainties require long term follow up;
- **A coordinated approach on the development and use of registries serving multiple purposes** (e.g. the follow up of patients, assessment and reimbursement), as the limited set of data and today's fragmented approach needs to be addressed;
- **Greater cooperation and clarity on use of the cross border healthcare provisions**, as many advanced therapies are delivered in only a few highly specialised hospital centres across Europe and cannot be delivered in all countries;
- **More informative and technical discussions to grasp the specifics on advanced therapies' cost and value**; and
- **Payment based on outcomes and payment over time**, though innovative payment mechanisms will be pre-empted by the removal of barriers.

EURORDIS is calling on all stakeholders – patients, industry representatives, payers, HTA and regulatory bodies, policy makers at national and EU level – to take forward the solutions in a dialogue to improve access to the advanced therapies.

> **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe, said:
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> "The fact is that science is breaking through and will increasingly deliver innovative treatments that are potentially transformative or curative for people living with a rare disease. It is now our collective responsibility to turn these indescribable hopes into a reality."
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> "There is no doubt these therapies for patients can save and transform lives, so that is why, together with the Consortium, we propose solutions to patient access. Time, equity, and sustainability are all important principles in this. We hope the publication of the RARE IMPACT report, alongside national reports published earlier this year, will stimulate policy discussions on how healthcare systems can better cooperate as well as relevant stakeholders to improve access to gene and cell therapies."

What are the advanced therapy medicinal products?

Advanced therapy medicinal products are at the cutting edge of innovation and offer major hope for various diseases for which there are limited or no therapeutic options. Currently half of the ongoing clinical trials for advanced therapies are in the field of rare diseases.

These new therapies promise life-changing treatments. However, the complex nature of their development and delivery has hampered patient access to these advanced therapies due to both practical and technical challenges, such as delivery of treatments at specialist centres, and reimbursement for the high cost of these treatments.

The RARE IMPACT European report published today looks at access to advanced therapies through a European Union lens, and supplements ten detailed reports on country specific challenges and solutions, based on two years of analysis and dialogue that has allowed us to better understand the challenges to access to therapies for rare disease patients.

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About RARE IMPACT

The RARE IMPACT initiative aims to identify and validate the challenges to patients' access to gene and cell therapies through engagement with HTA agencies, regulatory bodies, payers, patient groups, clinicians, manufacturers and other experts across Europe. RARE IMPACT is a consortium of manufacturers of gene and cell therapies and umbrella organisations such as the European Federation of Pharmaceutical Industries and Associations (EFPIA), the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) and the Alliance for Regenerative Medicine (ARM). EURORDIS-Rare Diseases Europe chairs the consortium with the knowledge and support from Dolon Ltd.

About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 940 rare disease patient organisations from 73 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

About rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date affecting an estimated 30 million people in Europe and 300 million worldwide. 72% of rare diseases are genetic whilst others are the result of infections (bacterial or viral), allergies and environmental causes, or are degenerative and proliferative. 70% of those genetic rare diseases start in childhood.

Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offerings inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.