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RARE DISEASES: ENSURING EUROPE REMAINS AT THE FOREFRONT OF INNOVATION

EVENT REPORT
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In the past two decades, a combination of scientific breakthroughs coupled with strong incentive systems has seen considerable progress for rare disease patients, but this innovation has tapered off in recent years.

Given that 95% of rare diseases conditions remain without treatment, the need for science and innovation to tackle rare diseases is far from decreasing.

The Orphan Medicinal Products (OMPs) regulation, introduced in 2000, has been instrumental in stimulating research and development, bringing new treatments to patients and incentivising companies to invest in OMPs.

In the lead up to the publication of the OMP and Paediatric Regulations evaluation, EUCOPE, Europe's trade body for small to medium-sized innovative companies working in the field of pharmaceuticals and medical technologies, brought together a multi-stakeholder dialogue to explore the ways in which Europe can remain at the forefront of innovation to the benefit of patients with rare diseases.

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Data sharing critical to close 'innovation blackhole' for rare diseases

By Natasha Foote | EURACTIV.com

Alexander Natz, secretary general of EUCOPE, said that "competitiveness in Europe is decreasing which might lead to postpone innovation, which will be detrimental to patients, especially those living with a rare disease". [SHUTTERSTOCK]

Access to data and 'real-world evidence' obtained from observational data is needed to help push research and innovation on rare diseases, stakeholders have said.

Speaking at a recent webinar, stakeholders emphasised the need to "break silos" on data and enable access to data to drive forward innovation in this area, highlighting that this is particularly important for developing treatments for rare diseases.

Earlier this year, the European Commission put forward plans to establish nine common EU data spaces, including across sectors including healthcare, as part of its EU data strategy.

Europe's trade body for SME companies, EUCOPE, working in the field of pharmaceuticals and medical technologies, recently highlighted the ways in which Europe can remain at the forefront of innovation to the benefit of patients with rare diseases.

In the past two decades, a combination of scientific breakthrough coupled with strong incentives systems has seen considerable progress for rare disease patients.

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The EU Regulation on Orphan Medicinal Products (OMPs) was introduced in 2000 specifically to address the challenge of regulating medicines that treat patients with rare diseases.

Marking the 20th anniversary of the regulation coming into force, and in the lead-up to the OMP evaluation, EUCOPE commissioned a study which concluded that regulation had successfully incentivised companies to invest in the development of OMPs.

Notably, it found that the number of medicines approved in Europe to treat rare diseases since 2000 has increased from 8 to 167, while the number of clinical trials focused on rare diseases have grown by 88% in a decade.

However, the initial progress seen since the introduction of the regulation has tapered off in recent years, with a decline in a number of designations and approvals in the EU over the past 4 years. Currently, 95% of rare diseases conditions remain without treatment.

Introducing the event, Alexander Natz, secretary-general of EUCOPE, said that "while the regulation has done a great deal to foster the development of OMPs, competitiveness in Europe is decreasing which might lead to postponing innovation, which will be detrimental to patients, especially those living with a rare disease.

He added that the EU "simply cannot afford this innovation blackhole."

But participants stressed that there are still significant hurdles in speeding up from science to treatment and fully capturing the value of what new OMPs can bring.

One of these hurdles is access to data and the collection of 'real-world evidence' (RWE), with Natz stressing that coordination on this is needed to close data gaps and represents a "critical step for driving forward research in the area of orphan drugs".

RWE is evidence obtained from observational data obtained outside the context of randomised controlled trials and generated during routine clinical practice, otherwise known as real-world data.

This is particularly pertinent in light of the COVID-19 crisis, which has thrown a number of existing challenges faced by those living with rare diseases into sharp relief, but which has also shown that the potential of digital technologies and data sharing, something EUCOPE says must be capitalised on for rare diseases post-crisis.

Using data in this way allows a more accurate assessment of patient outcomes and to ensure that patients get a treatment that is right for them, panellists highlighted.

Dimitrios Athanasiou, a board member of patients' association World Duchenne Organisation, said that the EU needs "new processes and new methodologies," stressing that we "can't do anything without data".

"Patients reported outcomes and RWE supported by patients is a must, I don't see any other way. We need to gather more data," he emphasised.

RWE is especially important in the case of rare diseases, according to Thomas Bols, head of government affairs and public policy at pharmaceutical company PTC Therapeutics, who said that the standard approach to clinical trials is "challenging, if not impossible" for many rare diseases.

This is because the numbers are not available to produce "meaningful" data given that the number of patients is so small.

However, he highlighted that there are "lots of challenges" involved with using RWE in this way, citing the fact that there is "no real framework and no consensus on criteria and how to generate the data".

As such, he said that work is required to "harmonise this field and make it more predictable," in order to improve the quality of this data in the field of orphan medicines.

Data must be stored in the form of electronic health records, databases, or registries, but EUCOPE's Natz highlighted that this represents a hurdle for small and medium-sized companies, saying that the setting up of registries can be "burdensome".

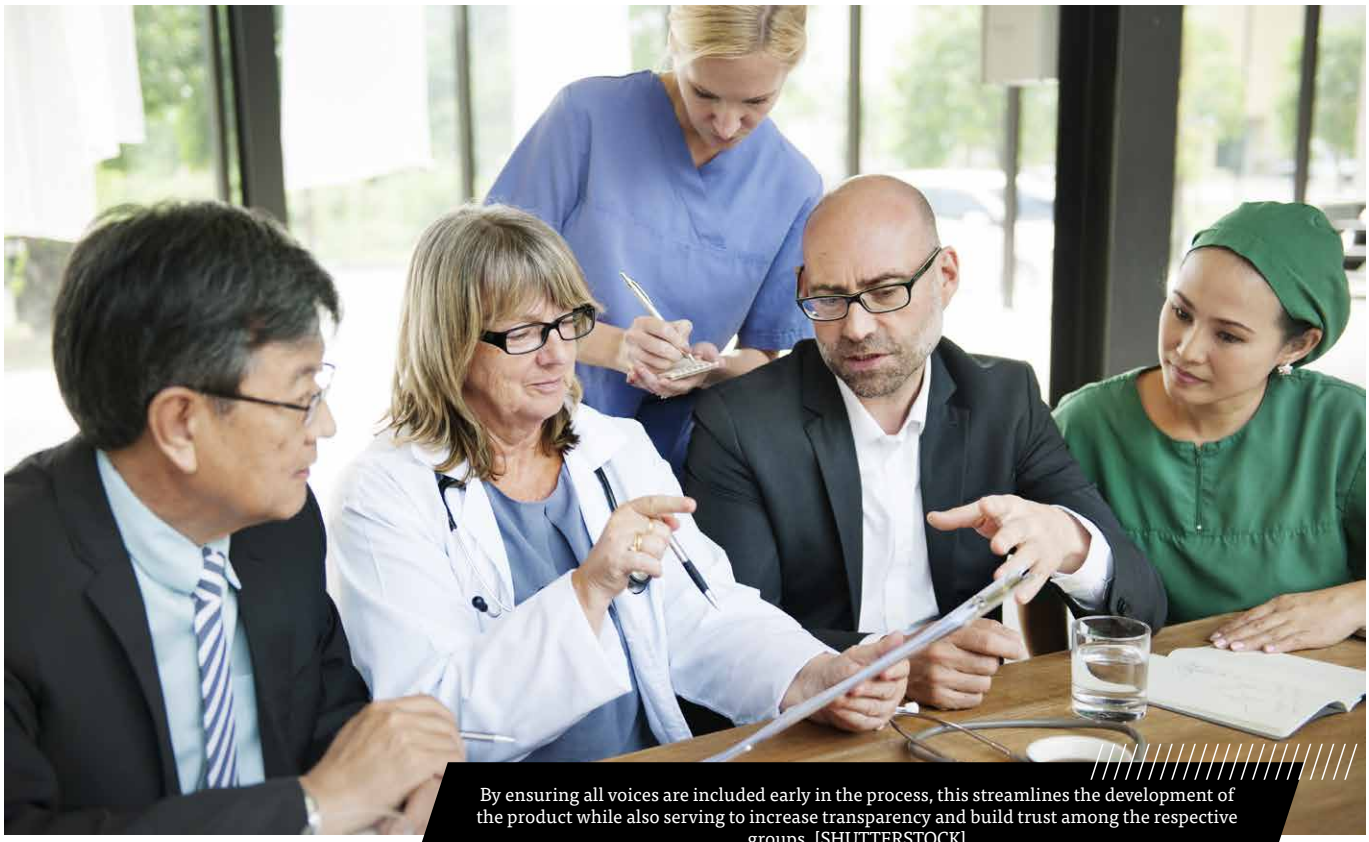
The study also concluded that the cost of generating RWE post-launch, which is particularly relevant for OMPs marketed under conditional approval, can be prohibitive.

Olga Soloman, head of medicines policy, authorisation and monitoring at DG SANTE, highlighted that there is currently a lot of work underway to encourage the use of RWE, but that there is a need to "learn from this and build a system that integrates RWE".

She added that there are questions pertaining to the quality of the data collecting and the best way to use this, highlighting that although registries already exist, there are differences between types of registries, and more work needs to be done to "create the right conditions for RWE".

Early stakeholder dialogues crucial for success of rare orphan medicines

By Natasha Foote | EURACTIV.com



By ensuring all voices are included early in the process, this streamlines the development of the product while also serving to increase transparency and build trust among the respective groups. [SHUTTERSTOCK]

Early dialogue between stakeholders is crucial for the effective development and success of rare orphan drugs, used to treat patients with rare diseases, Anna Bucsics, project adviser at the mechanism of coordinated access to orphan medicinal products (MoCA), told EURACTIV in an interview.

"Nobody wants to develop a drug that is not able to be used or to be sold. The earlier you find out what is needed, the easier it is for everyone to make important decisions and to ensure that the drug is fit for purpose," she said, adding that dialogue also serves to increase transparency and build trust among the respective groups.

Around 246,000 people suffer from rare diseases across the EU.

MoCA is a voluntary coordinated mechanism which works to facilitate dialogue between key stakeholders, including member states, orphan medicine developers, payers and patients, to evaluate the technical

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aspects of an orphan medicinal product, including distribution, logistics and pricing.

By working to support this exchange of information, MoCA aims to enable informed decisions on pricing and reimbursement at the member state level.

It is particularly important to facilitate this kind of discussion early on in the process of developing an orphan drug, Bucsics stressed.

"Bringing all involved parties around the table to discuss a new orphan product in the early stages of development is crucial given that these treatments often pose more varied problems than others and can be particularly difficult to deal with," she said.

She added that this is also important for developing treatments for other diseases given that these orphan products often serve as a "canary in the coalmine" for other diseases.

This is because orphan medicinal products (OMPs) are usually at the cutting edge of innovation, and can therefore offer insights into problems that other medicines may encounter later in development.

Bucsics added that OMPs also often serve as a model for other new treatments, such as new cancer drugs.

PATIENTS FEW AND FAR BETWEEN

But bringing all involved parties is particularly challenging for patients, Bucsics stressed.

"It is very valuable to have patients' input and support early in the discussions, as this allows companies to take their concerns into consideration," she said, adding that patients that are involved early on in the procedure are also more likely to engage with patient-reported outcomes later.

Patient-reported outcomes are defined as any report of the status of a patient's health condition that comes directly from the patient, without interpretation by a clinician or anyone else, and these are a key tool to measure the effectiveness of a drug.

But getting enough patient input is a challenge in the case of rare orphan drugs given that the pool of patients is often very small and patients are often spread across Europe.

PAYER PARTICIPATION A 'CHALLENGE'

Equally, ensuring payers participation in these dialogues presents a major challenge, but those are "vital voices" in the conversation which ensure discussions about pricing and reimbursement happen at an earlier point in time.

But there is currently a reluctance to get involved in such discussions.

One of the reasons is that there are often concerns that payers will be held to what they say, according to Buscics, driving concerns that early constructive dialogue could later turn into constraints.

But Buscics stressed that these early dialogues are not binding and are "completely informal", adding that this is something that must be made clearer to participants.

"People are worried that in these discussions, they will be held to whatever they say, and that this could have repercussions later down the road. But the European Medicines Agency makes it very clear that all advice is not binding," she emphasised.

Another reason for a low level of participation among payers is because they often do not have enough resources to participate, as this is technically "outside their remit".

One way this could be alleviated is by holding discussions virtually, making it easier, cheaper and less time-consuming for everyone to be able to attend discussions and thus encouraging a wider variety of voices to the table.

PROMOTED CONTENT / OPINION

DISCLAIMER: All opinions in this column reflect the views of the author(s), not of EURACTIV Media network.

Europe at the crossroad of Rare Diseases innovation: lessons from the past and future outlooks

By Alexander Natz | European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

Competitiveness in Europe is decreasing which might lead to postponing innovation, which will be detrimental to patients. [EUCOPE]

In shaping the future of rare diseases policies all stakeholders should cooperate to create an environment that actively supports research and clinical development of orphan medicinal products (OMPs). This entails building synergies all across the innovation ecosystem to address the many hurdles related to prevention, treatment and care of rare diseases.

Alexander Natz is the Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE).

COMPANIES WORKING ON RARE DISEASE – A MULTIFACED REALITY IN CONTINUOUS EVOLUTION

At EUCOPE, with half of our 130 members active on rare diseases, we know that when it comes to rare diseases one size truly does not fit all. The upcoming publication of the OMP and Paediatric Regulations evaluation offers ground for reflection: before the OMP Regulation (141/2000) came into force there was hardly any focus on rare diseases. Since 2000, the number of authorised orphan medicines increased from 8 to 169. The Regulation

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is among the factors that allowed a whole innovation ecosystem to thrive, spanning from global pharmaceutical corporations to smaller companies, start-ups and research centres. As highlighted by a study EUCOPE commissioned to the UK Office of Health Economics, more than a third of granted marketing authorisations for OMPs are held by OMP-focused developers. Many of these companies make little or no revenue yet, mostly due to enormous upfront investment in research and development. For these companies, incentives are vital because they enable them to invest (and reinvest!), often building on the knowledge, expertise and the long process of trial and error that leads to scientific breakthrough.

UNMET MEDICAL NEED IN RARE DISEASE – WHAT'S IN A NAME?

Despite increasing use of the term unmet medical need, there is no agreed common definition of this concept. It can vary significantly depending on the intended purpose and different stakeholders. When it comes to rare diseases, we face a staggering 95% of conditions without any therapeutic options. However, we believe that, while crucial, absence of any therapeutic option is hardly the only unmet need to take into account. Diseases severity, patients, families and carers quality of life are also essential elements of the equation. The OMP regulation allows a product to demonstrate a significant benefit compared to the existing standards of care and thus to be granted the OMP designation in the same indication. Not all patients respond to the same treatments in the same way, therefore the improvements brought by new products in the same indication should not be underestimated.

Furthermore, when addressing unmet needs of people with rare diseases, including improving their quality of life, the OMP Regulation is only one piece of the puzzle. For instance, timely diagnosis of a rare disease and the availability of expert centres in the EU, which are now fostered by the European Reference Networks (ERNs), are key elements too.

PLAYING DEVIL'S ADVOCATE: THE INCENTIVES TO DEVELOP THERAPEUTIC OPTIONS FOR RARE DISEASES HAVE DONE PLENTY BUT THERE ARE MORE ELEMENTS TO CONSIDER FOR FUTURE SUCCESS.

While the OMP regulation has done a great deal to foster the development of OMPs, competitiveness in Europe is decreasing which might lead to postponing innovation, which will be detrimental to patients. As highlighted in an event recently hosted by EUCOPE there are still significant hurdles in speeding up from science to treatment and fully capturing the value of what new OMPs can bring.

Multifaced issues concerning rare diseases need equally articulated responses. Breaking it down is no easy task but it is also a duty that the whole rare disease community shares.

EUCOPE believes that any future success in rare disease policy should include these three elements:

- **Global cooperation**: Rare diseases is not a field where Europe can operate alone. Next to strong incentives' ecosystems, global regulatory cooperation is a reality that needs further attention.
- **The power of data**: We cannot miss out on the opportunity to break down the data silos barriers, make the most of real-world evidence to strengthen our network of knowledge, improve disease registries for rare disease and also find common standard to make the most of it throughout the lifecycle of medicines.
- **Innovative pricing models**: All this innovation has little value if it does not reach the patients, this is why innovative pricing solutions to bring rare diseases therapies to the market will be key.

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Contact us

Teresa Dominguez
EU Affairs Manager
teresa.dominguez@euractiv.com
tel. +32 (0)2 788 36 93

Natasha Foote
Reporter, Agriculture & Health
natasha.foote@euractiv.com
tel. +33 (0) 7 69 69 51 36