friends of europe

SUMMER 2019

Equal access to care for rare diseases

EVENT REPORT

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Co-funded by the
Europe for Citizens Programme
of the European Union

Co-organised with

Takeda

EURORDIS
RARE DISEASES EUROPE

Introduction

Rare diseases, by their very nature, are experienced by a small proportion of the population. In the European Union (EU), a disease is considered rare if it affects up to 5 in 10,000 people. Today there are between 6,000 and 8,000 rare diseases, with new conditions being identified every year. An estimated 30 million Europeans have a rare disease.

Huge progress has been achieved following the entry into force of the EU Orphan Regulation in January 2000 and over 150 orphan products – medicines to tackle rare diseases – have now been approved. But timely and equal access country-wide to these medicines is not guaranteed, participants at Friends of Europe's 'Café Crossfire' lunch debate on 26 June heard.

Introducing the Brussels meeting, moderator and Friends of Europe Senior Fellow **Tamsin Rose** emphasised that rare diseases are perhaps the most compelling reason for the EU to address health. With 75% of rare diseases affecting children and diagnoses taking up to five years, "if you are a parent of a kid with a rare disease struggling to find a diagnosis, only to find there are few if any treatments available, it is very clear that Europe needs to do more."

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> **Rute Fernandes**, Group Vice-President and Head of Rare Diseases for Europe and Canada at global pharmaceutical company Takeda

Indeed, with only 5% of rare diseases getting appropriate treatment, **Rute Fernandes**, Group Vice-President and Head of Rare Diseases for Europe and Canada at global pharmaceutical company Takeda, said, "We need a new approach to accelerate access and use real-world evidence to establish the value of these treatments. Industry and regulators need to be in dialogue early in the process."

Reference networks and disease registries

The European Reference Networks (ERNs), launched in 2017, are potential game changers. **Till Voigtländer**, Austrian Representative of the Board of Member States of ERNs, told the meeting that patients can benefit from these virtual networks that bring together more than 300 hospitals and 900 specialised teams.

If these ERNs are fully integrated into member states, they will reduce diagnosis time, **Martin Seychell**, European Commission Deputy Director General for Health and

Food Safety, told the meeting. However, this integration is harder in some countries than others – notably federal states like Austria.

But ERNs are not the only answer. Most EU countries now have a national plan for rare diseases. Part of the answer lies in collecting the right data and, more importantly, using it correctly. Meanwhile, "the data revolution is already upon us," Seychell said. "Other sectors are far more digitalised than health, which is a decade behind. We generate large amounts of high-quality data, but we only use a bit of it."

Registries of patients with rare diseases are valuable assets and a European clearing house for rare diseases data could be useful. However, the European Organisation for Rare Diseases (EURORDIS) Chief Executive Officer **Yann Le Cam** sounded a note of warning. There are too many calls for disease registries without criteria about whether they would work. Registries will only be possible for up to 500 diseases, not the 6,000 or so rare diseases.

The access challenge

The good news is that treatments are being developed and authorised. However, there are still huge access problems. A EURORDIS survey revealed 25% of them had no access to adequate therapy or products. In Poland, Romania and Ireland, the figure was 40%.

One problem is insufficient or delayed reimbursement procedures. In 10 out of 19 European countries sampled in the EURORDIS Rare Barometer survey, more than 20% of patients say that insufficient reimbursement is a major obstacle in accessing treatments. The situation could worsen with the advancement of new cell and gene therapies as the system of reimbursement is not adapted to the latest scientific developments.

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> **Yann Le Cam**, Chief Executive Officer of the European Organisation for Rare Diseases (EURORDIS)

No patient should be left without a pathway. For that reason, incentives for innovation must be linked to improving access for more patients. There are good news stories, panellists said. Since October 2018, Scotland has decided that all medicines targeting ultra-rare diseases that the Scottish Medicines Consortium (SMC) decides are clinically effective will be reimbursed for a minimum of three years.

1. **Till Voigtländer**, Austrian Representative of the Board of Member States of ERNs
2. **Tamsin Rose**, Senior Fellow at Friends of Europe
3. **Rute Fernandes**, Group Vice-President and Head of Rare Diseases for Europe and Canada at global pharmaceutical company Takeda
4. **Yann Le Cam**, Chief Executive Officer of the European Organisation for Rare Diseases (EURORDIS)

Europe must not be second choice

Le Cam and Seychell warned of a recent worrying trend, wherein the United States, not Europe, is leading the way in developing new drugs to fight rare diseases. One explanation for this is that American regulations in this area are different and less constricting.

“With the Orphan Regulation, from what I see today, honestly, we are second choice,” Le Cam said. “If we are not playing in the top league, products will be developed in very different settings, where the ethical and legal principles are very different.”

> “It is always a challenge to develop a drug, There are many in the pipeline and only a few will end up as marketed products

Till Voigtländer, Austrian Representative of the Board of Member States of ERNs

In short: “We can either be at the table or on the menu,” Seychell argued, adding “and, as Yann says, a product that does not reach the patient is not working.”

China should also be recognised as a competitor, Rose said. Le Cam agreed that China, and India too, “should be on our radar”, noting the Commission-funded EURORDIS Rare 2030 Foresight study, launched January 2019, would look at this.

Such tensions only exacerbate a situation where “it is always a challenge to develop a drug,” Austria’s ERN Representative Voigtländer highlighted. “There are many in the pipeline and only a few will end up as marketed products.”

Rare diseases are also not a “homogeneous group”, he continued. Some conditions affect many people, but others are exceedingly rare. This means finding the money to justify treating such a small population is a challenge. “In Austria, we developed a drug only relevant for a couple of patients, but industry said there was no market for it.”

Conclusion

There are many positive developments in the fight to combat rare diseases, notably the ERNs and patient registries, the meeting heard.

The Orphan Regulation has been particularly successful. We started with only 8 orphan medicinal products and now more than 150 cover over 90 rare diseases. In addition, there has been an 88% increase in research annually between 2006 and 2016 – with Europe leading the globe in new clinical trials. But despite all this, rare diseases are still an unmet need.

Voigtländer added “We also need a think tank at European level to develop a vision and project what is good,” in fighting rare diseases.

The next five years will be absolutely crucial in deciding how to use expertise where it can make a difference. We have to solve the big paradox where we have some of the best, well-organised healthcare systems in the world, but are no longer the automatic first or even second choice where innovation happens.

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Martin Seychell, European Commission Deputy Director General for Health and Food Safety

The Commission will adopt a new rare disease policy in the future, informed by two studies to be published in late 2019: a 2030 Foresight study on future policy needs of the rare disease community and a study on the functioning of the EU Orphan Drug Regulation.

“With all the knowledge available today, it should be possible to provide a diagnosis six months after seeing a doctor, and bring a high standard of care to a broader percentage of the population,” Seychell said.

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1. **Martin Seychell**, European Commission Deputy Director General for Health and Food Safety

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