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# Europe's leadership in life sciences needs strong frameworks and partnerships

POLITICO Studio speaks with Ricardo Marek, President Europe and Canada at Takeda Pharmaceuticals, on why rare diseases need to be a public health priority, and how Europe can remain a leader in life sciences.



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BY POLITICO STUDIO

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Life sciences are integral to the European economy and its people. Over two years ago



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ecosystem had to rapidly come together, forging strong partnerships to deploy treatments at unprecedented scale.

This precedent should continue and be considered to aid people suffering from rare diseases. “Around 30 million people across Europe live with a rare disease<sup>1</sup>. On average, it takes six to seven years to obtain a correct diagnosis<sup>2</sup> and over 40% of rare disease patients are initially misdiagnosed<sup>3 4</sup>,” says Ricardo Marek, President Europe & Canada at Takeda.

A strong policy framework incentivizing investments in the health care ecosystem and partnerships is critical. The region has strengthened its position through lessons learnt from the pandemic. But if Europe is to continue tackling the rising burden of diseases, it is critical that this momentum is maintained.

Marek adds: “To reinforce its global competitiveness, Europe needs to continually evolve its regulatory framework, adopting advances in technology, utilizing real-world evidence (RWE) and data analytics in a dynamic and iterative decision-making process.”

In conversation with POLITICO Studio, Ricardo offers valuable insights as to how this can be achieved — and the potential benefits that may ensue.



Takeda's footprint in the Europe and Canada Business Unit,  
where Ricardo Marek is President

**POLITICO Studio: What are some lessons learned and partnerships forged during the COVID-19 pandemic?**

Ricardo Marek: The pandemic uncovered some of the components necessary to drive positive change. Multistakeholder partnerships were swiftly forged, with data and expertise shared to accelerate vaccine development and production<sup>5</sup>.

Takeda is proudly leading or participating in more than 100 research and development public-private partnerships across more than 75 countries<sup>6</sup>, dedicating the expertise of our scientists and our data, including [H2O \(Health Outcomes Observatory\)](#)<sup>7</sup>, [ARDAT \(Accelerating Research & Development for Advance Therapies\)](#)<sup>8</sup> and [Screen4Care](#)<sup>9</sup>.

These allow us and other stakeholders to go further to address challenges that could not be pursued alone and help accelerate progress toward delivering transformative medicines to patients.

**PS: While rare diseases are, by definition, rare, the absolute numbers are still significant. What are some of the innovations Europe has achieved/is working on in this area?**

RM: Around 30 million people across Europe live with a rare disease<sup>1</sup>. On average, it takes six to eight years to obtain a correct diagnosis, and over 40 percent of rare disease patients are initially misdiagnosed<sup>2</sup>.

It is critical for Europe to maintain a strong focus on innovation in medicines and health care infrastructure to improve diagnosis and deliver health care management and treatment options to these patients. Alongside raising awareness, Europe has developed a vibrant research and development community dedicated to improving the lives of rare-disease patients.

Some benefits have been reported for patients suffering from rare diseases. The European Commission has already authorized more than 200 orphan medicinal products (OMP), since the adoption of the EU Orphan Drug regulation in 2000<sup>10</sup>. Moreover, the frameworks for addressing rare-disease patients' needs in Europe have been significantly upgraded in the past 20 years, transforming care and innovation standards<sup>11</sup>.

Partnerships have been critical to the evolution of Europe's rare disease ecosystem and remain essential for addressing unmet needs. Similarly, value-based partnerships unlock inno

exert positive impact by delivering better outcomes for patients<sup>12</sup>.

**PS: The OMP Framework has increased innovation in Europe, but disparities in access to medicines remain. How do you think people living with rare diseases can be supported better?**

RM: Rare disease patients across Europe do not have equitable access to therapies<sup>13</sup>. Innovative payment models and early evidence dialogues may support broader and faster access to medicines.

Multiple challenges remain during patient journeys, ranging from diagnosis delays to inequitable access to treatment. These include slow regulatory processes, late initiation of market access assessment, duplicative evidence requirements, reimbursement delays and local formulary decisions. As the root causes are multifactorial, they can only be solved by different stakeholders working together<sup>14</sup>.

At Takeda, we have developed strong partnerships with trade associations such as the [European Federation of Pharmaceutical Industries and Associations \(EFPIA\)](#), [EuropaBio](#) and [European Confederation of Pharmaceutical Entrepreneurs \(EUCOPE\)](#) and support the call from patients by the [European Organisation for Rare Diseases \(EURORDIS\)](#), for a European action plan on rare diseases and to make rare diseases a health priority. Alongside an evolved incentive framework, partnerships can effectively address barriers across the OMP lifecycle.

**PS: There is increasing demand for treatment options for rare diseases. Patients who previously have not had access to approved therapies. Considering this, how does Takeda build and maintain access to treatments across the whole lifecycle, from the initial clinical trials, to delivering affordable medicines to end-users?**

RM: Access starts with science. A good example is next-generation sequencing (NGS) which has enabled rapid sequencing of the base pairs in DNA or RNA samples. This has opened the way for new technologies — and hence for developing innovative therapies for rare diseases.

Despite significant improvements, unmet needs persist among rare disease patients. An obvious unmet need includes the 95 percent of rare diseases that today have no authorized treatment<sup>11</sup>. Prevalence estimates suggest that these 95 percent affect a small fraction of the rare disease patient population<sup>1</sup>.

If we can determine if a medicine is going to succeed early in its development process, we can avoid placing patients, caregivers and health care providers in unnecessary stress. This requires companies and regulatory bodies to partner around a common goal of advancing promising therapies and terminating those that are not.

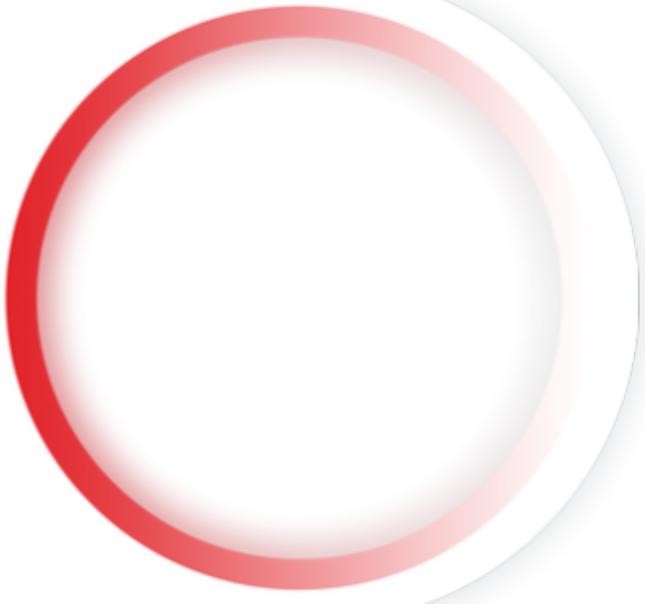
Furthermore, we need to rethink how we improve patient access in Europe. This requires companies, disease experts, patients and payers to come together and find creative solutions that allow for equitable access. We need to look at setting up partnerships between stakeholders early in the development process and explore ways to leverage digital technologies and alternative data sources where randomized clinical trial data is not available for ethical or other reasons.

**PS: What part does data play in this pipeline? And how is Takeda leveraging its data across the medicine lifecycle?**

RM: Data and evidence are crucial to medicine development and innovation. Along the value chain, data shapes research and development and clinical trial design, measuring and demonstrating the efficacy, safety and benefits of a product in development.

Randomized controlled trials are often not feasible for rare diseases due to small patient populations and variable disease courses, thus challenging the ability to produce sufficient evidence. However, real-world data is increasingly being collected through various alternative sources, allowing R&D developers to better understand disease lifecycles over time, explore unmet medical needs and study treatment outcomes.

## RWE usage in three steps of the development path



One way in which Takeda is unlocking such potential is through a partnership with RWE4Decisions — a payer-led, multistakeholder learning network exploring RWE<sup>15</sup>. It highlights the need for robust RWE and accelerated development pathways to foster highly innovative technologies. Through this partnership, Takeda has contributed to the European Health Data Space initiative<sup>16</sup> aimed at creating a harmonized healthcare data space which delivers for patients<sup>6</sup>.

Commitments such as this from major stakeholders whether in the European Commission, industry, academia, or health care systems will help to foster innovation in health care in Europe and beyond to deliver better outcomes for all, through standardization and patient-reported outcomes<sup>17</sup>.

## About Takeda

*Takeda is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to discover and deliver life-transforming treatments, guided by our commitment to patients, our people, and the planet. Takeda focuses its R&D efforts on four therapeutic areas: Oncology, Rare Genetics and Hematology, Neuroscience, and Gastroenterology. We also make targeted R&D investments in plasma-derived therapies and vaccines. <https://www.takeda.com/>*

## C-APROM/EUC/CORP/0001 /October 2022

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