

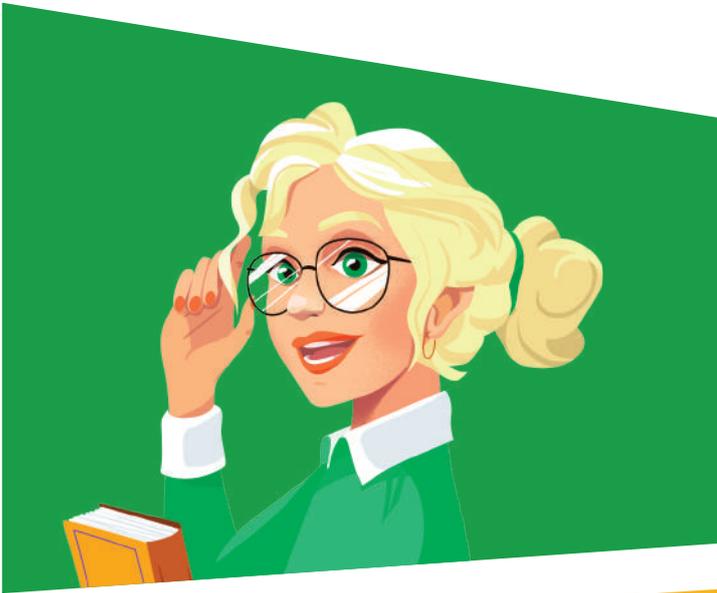
THE EUROPEAN CONFERENCE



ON RARE DISEASES AND ORPHAN PRODUCTS

# 12 LABOURS OF ECRD

**ACTION WITHIN REACH:  
A RARE DISEASE JOURNEY  
IN EUROPE**



# General Introduction

In Europe, four champions – Emilia, Camilo, Rosalind and Damiano – tackle rare diseases, driven by a shared mission. From Poland to Germany, the Netherlands to Italy, they unite, forming a mosaic of hope. Their journey delves into European health policy and innovation, marking milestones in the pursuit of equity in healthcare. Join them through twelve labours, a modern odyssey of unity, advocacy, and unwavering hope for the rare disease community.

In the heart of Europe, where history whispers and innovation pulses...



**Emilia** 🇵🇱

Driven by my journey, I fight for all of us.

A fervent patient advocate from Poland.



**Damiano** 🇮🇹

Healing begins with understanding and compassion.

A compassionate clinician from Italy.



**Camilo** 🇵🇹

**Rosalind** 🇳🇱

By working together, we establish the rules for the future.

A policy expert from the Netherlands.



Seeking answers in science, for hope beyond the unknown.

An innovative researcher from Portugal.



Driven by a mission to transform the rare disease landscape.

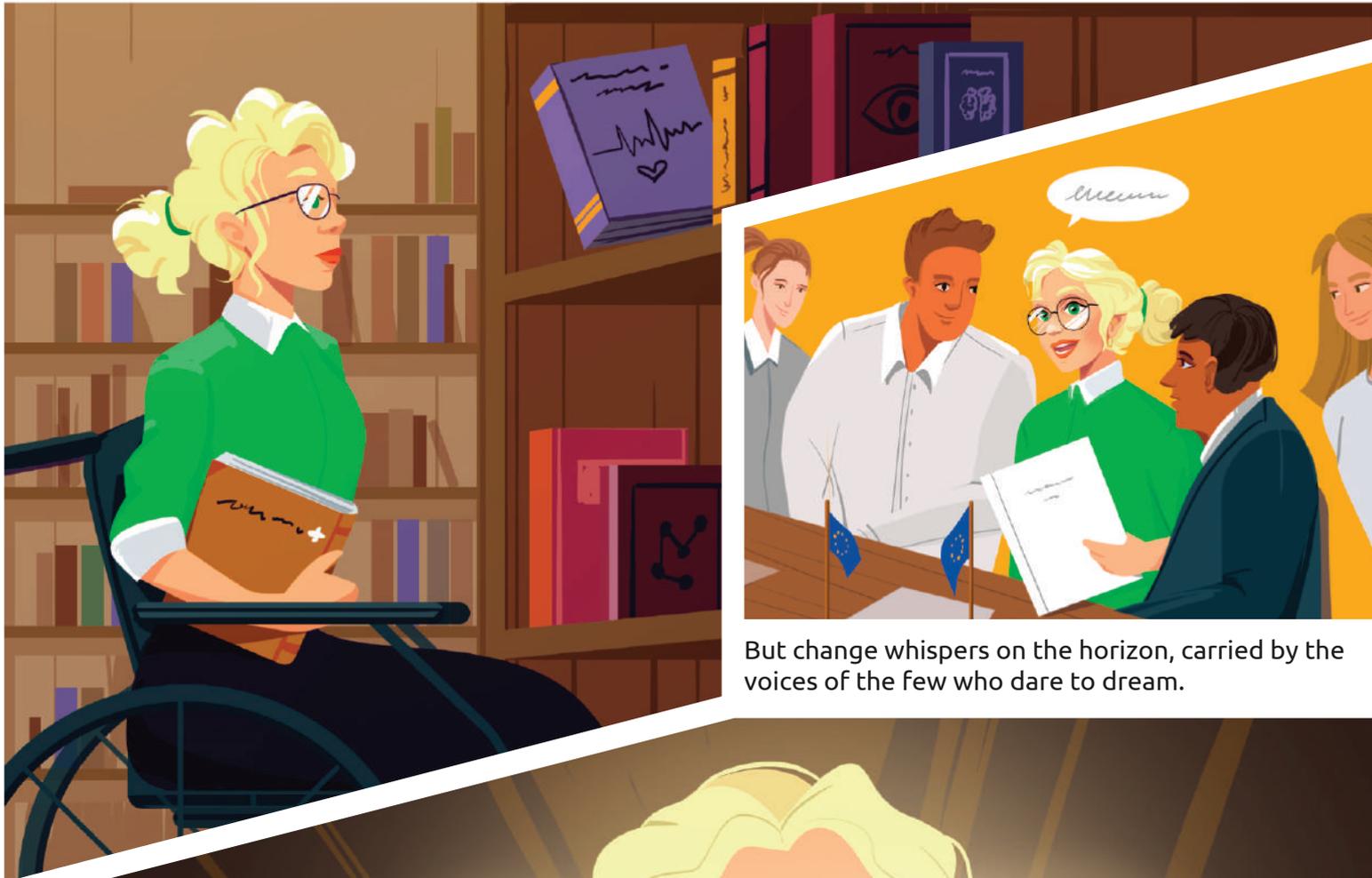


Together, we embark on a journey of advocacy, innovation and inclusiveness. Care to join us?

# The Beginning: The Orphan Medicinal Products Regulation

Moving forward, we'll delve into the twelve labours that mirror the historical and ongoing efforts to address rare diseases. We begin in 2001 with the implementation of the EU Orphan Medicinal Products Regulation, a pivotal moment that catalysed the development of treatments for rare diseases.

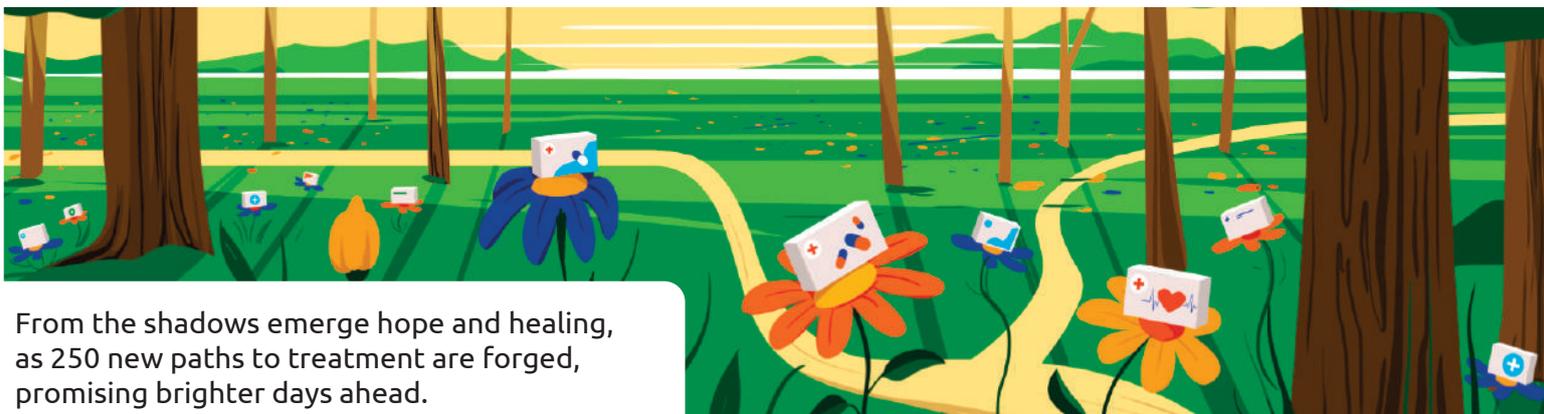
For too long, rare diseases have lingered in the shadows, their mysteries locked away without hope for cures.



But change whispers on the horizon, carried by the voices of the few who dare to dream.



With unity and perseverance, the rare disease community comes to the fore, marking a pivotal moment.



From the shadows emerge hope and healing, as 250 new paths to treatment are forged, promising brighter days ahead.

# The First Overarching Strategy: The EU Council Recommendation on Rare Diseases

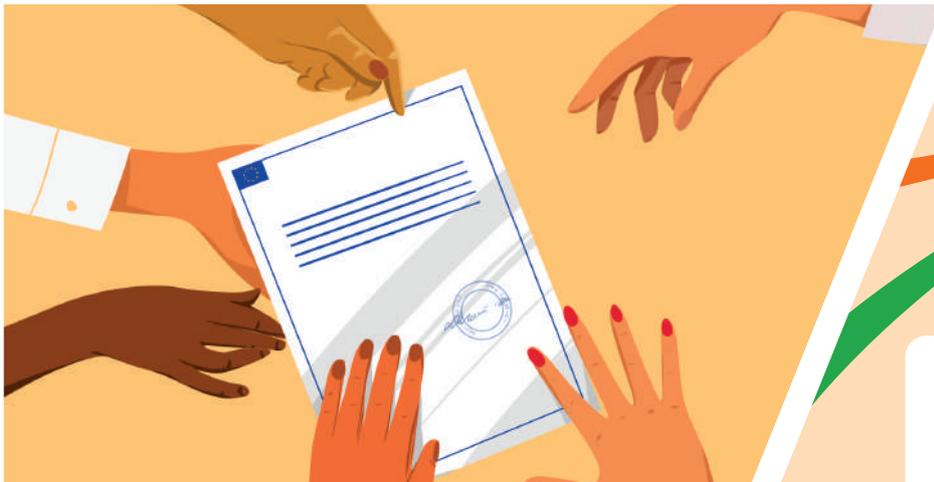
Continuing to the second significant achievement, we'll cover the adoption of the EU Council Recommendation on an Action in the Field of Rare Diseases in 2009. This recommendation was a crucial step toward encouraging EU member states to develop and implement national plans for rare diseases, aiming to improve diagnosis, treatment, and overall care.

Across Europe, the rare disease community faces a labyrinth of challenges, disparate in care and disconnected in approach.



Rosalind, with unwavering resolve, calls upon the nations of Europe to unite, weaving a stronger fabric of support for rare diseases.

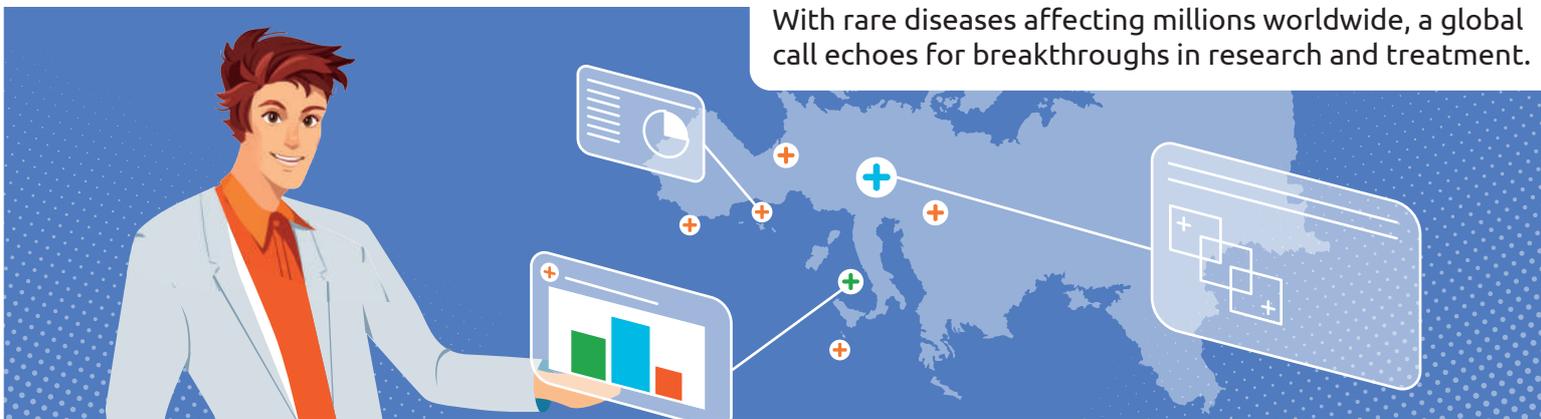
In a landmark moment, unity prevails. The Recommendation is adopted, setting a new course toward collaborative care.



The seeds of change take root, promising a future, where no rare disease patient navigates their journey alone.

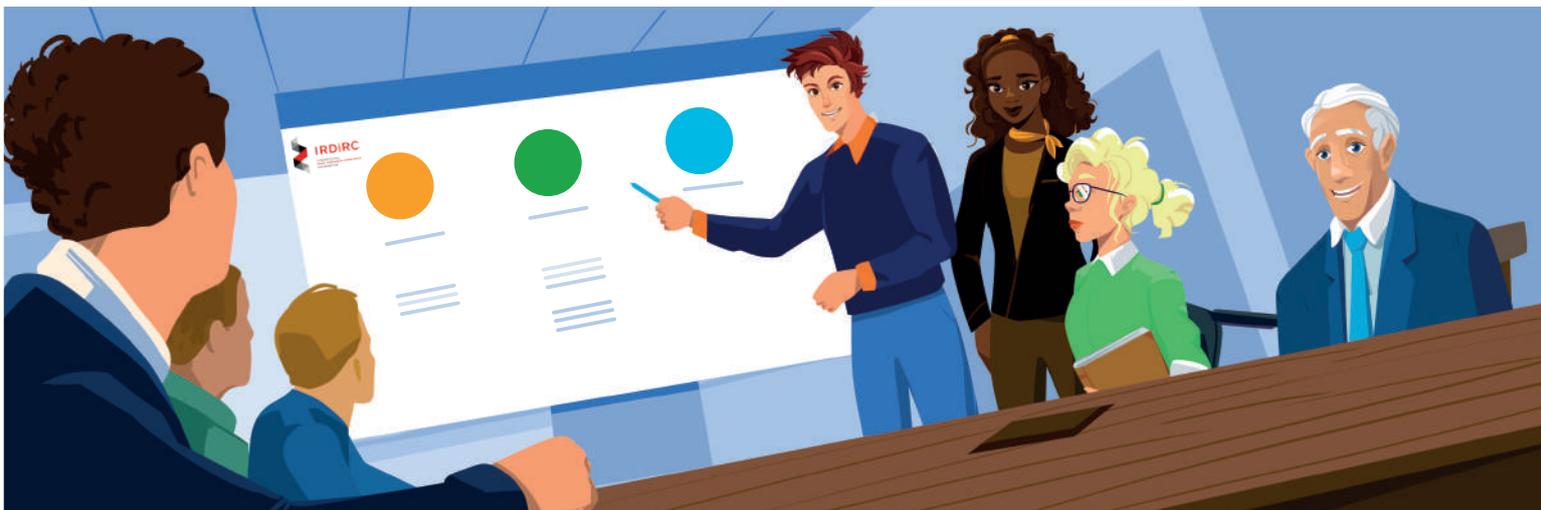
# IRDiRC's New Decade Vision

Transitioning to the third significant achievement, we spotlight the new goals and vision set forth by the International Rare Diseases Research Consortium (IRDiRC). IRDiRC's ambitious objectives aim to accelerate global research on rare diseases, with the ultimate goal of developing new therapies and diagnostic tools.



With rare diseases affecting millions worldwide, a global call echoes for breakthroughs in research and treatment.

Camilo spearheads the charge, rallying the world's best to commit to IRDiRC's bold objectives for the next decade.



From laboratories to patient bedside, IRDiRC's vision ignites a coordinated, worldwide assault on rare diseases.

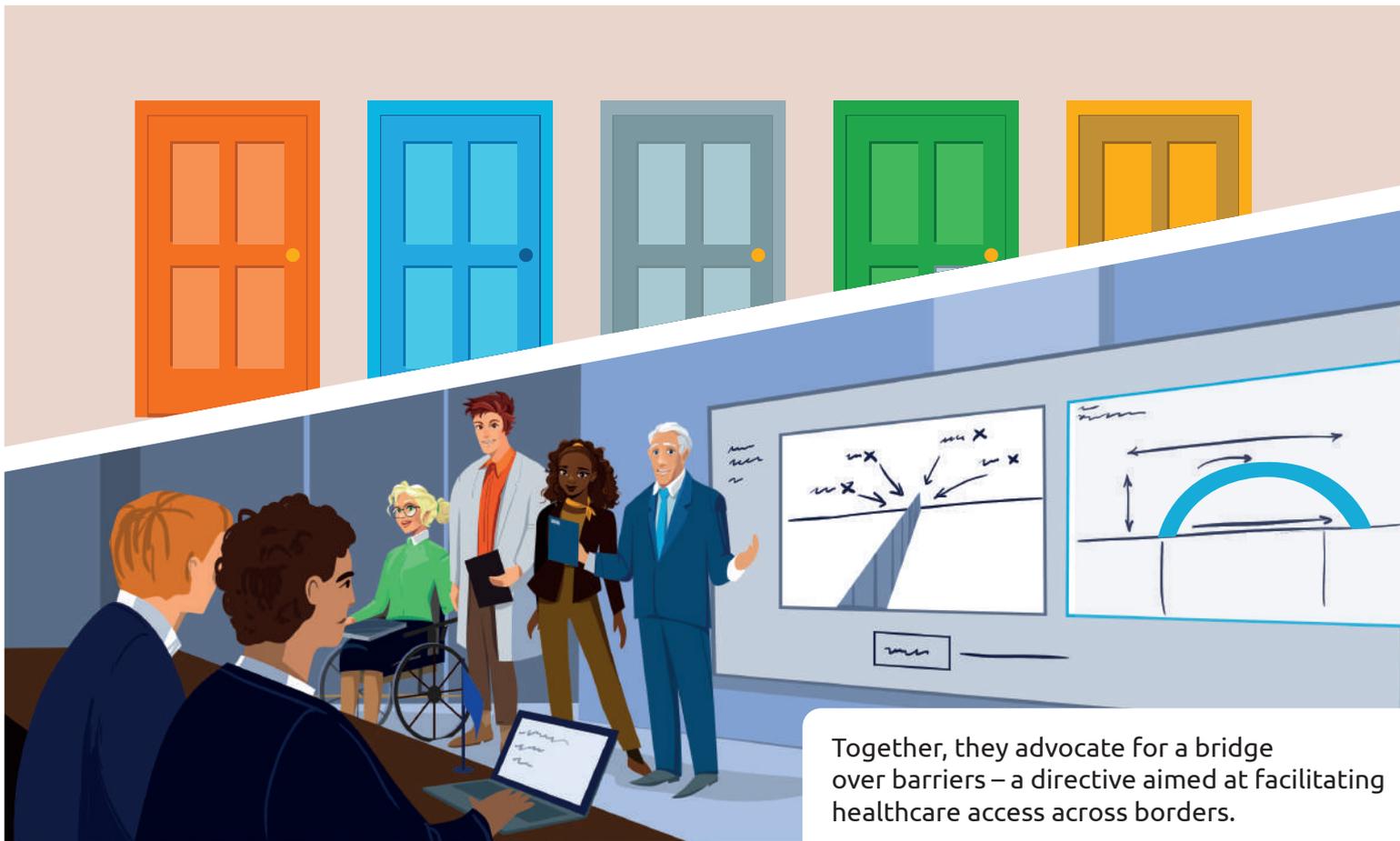


Under IRDiRC's guiding stars, the next decade promises advancements once deemed impossible, offering hope to millions.

# The Cross-Border Healthcare Directive

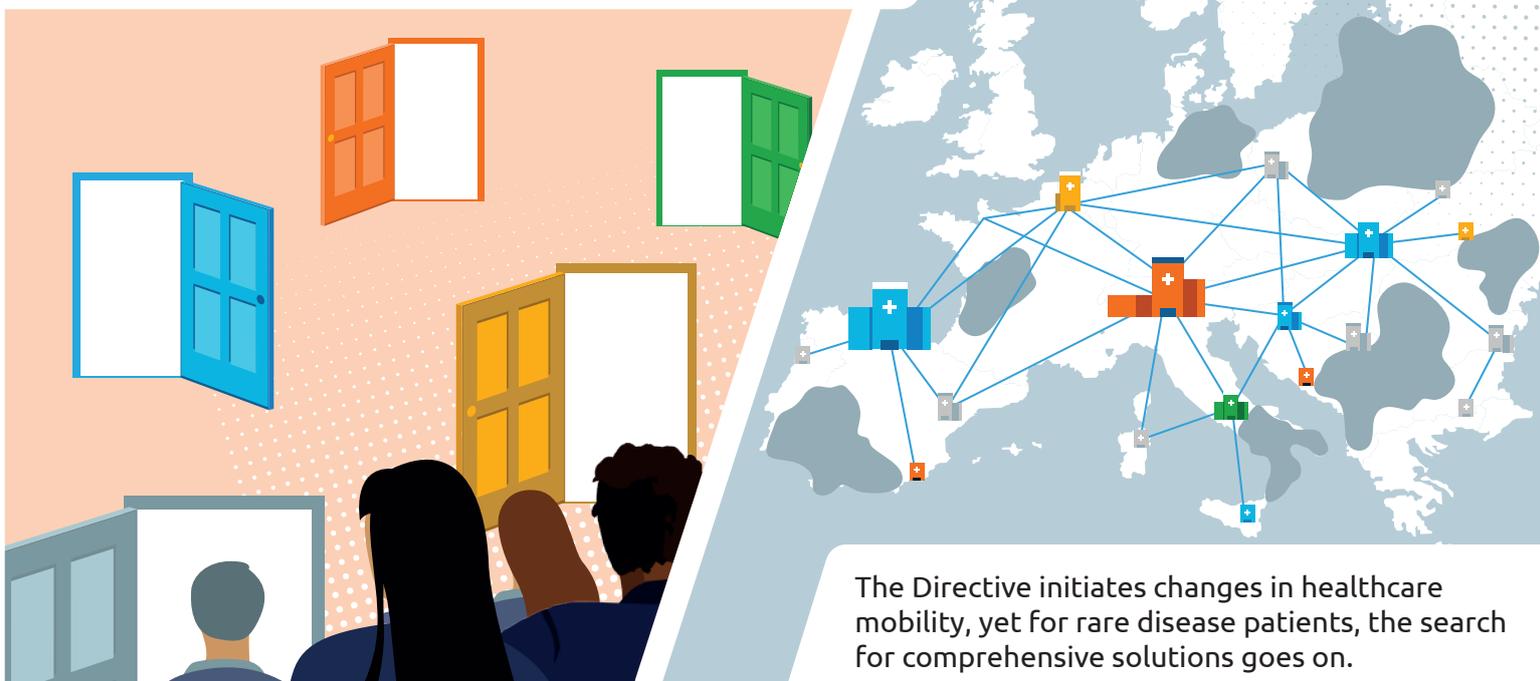
Moving on to the fourth significant achievement, we'll focus on the enactment of the Cross-Border Healthcare Directive in 2013. This directive was a pivotal moment for rare disease patients across Europe, promising access to specialised healthcare services beyond national borders, so they receive the best possible treatment available within the EU.

Rare disease patients face invisible walls within Europe, their access to treatments halted by national borders.



Together, they advocate for a bridge over barriers – a directive aimed at facilitating healthcare access across borders.

The Directive is approved, but its effectiveness still varies. For rare diseases, the journey for adequate treatment continues.

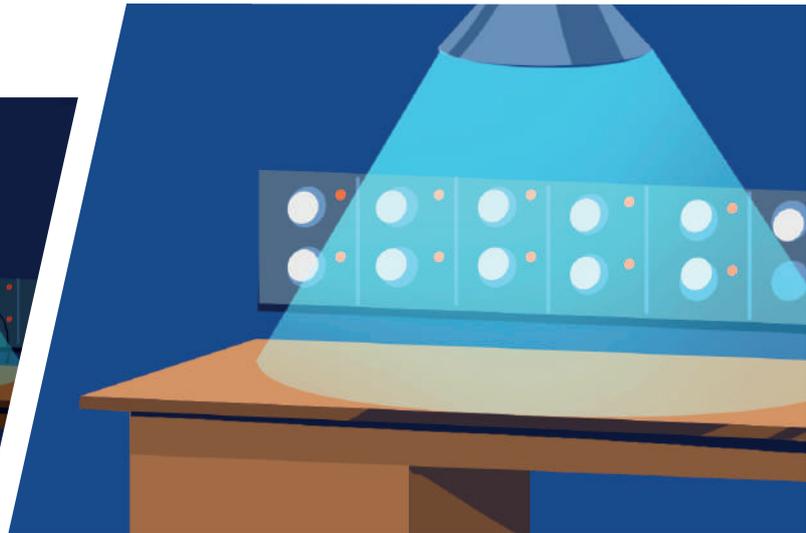


The Directive initiates changes in healthcare mobility, yet for rare disease patients, the search for comprehensive solutions goes on.

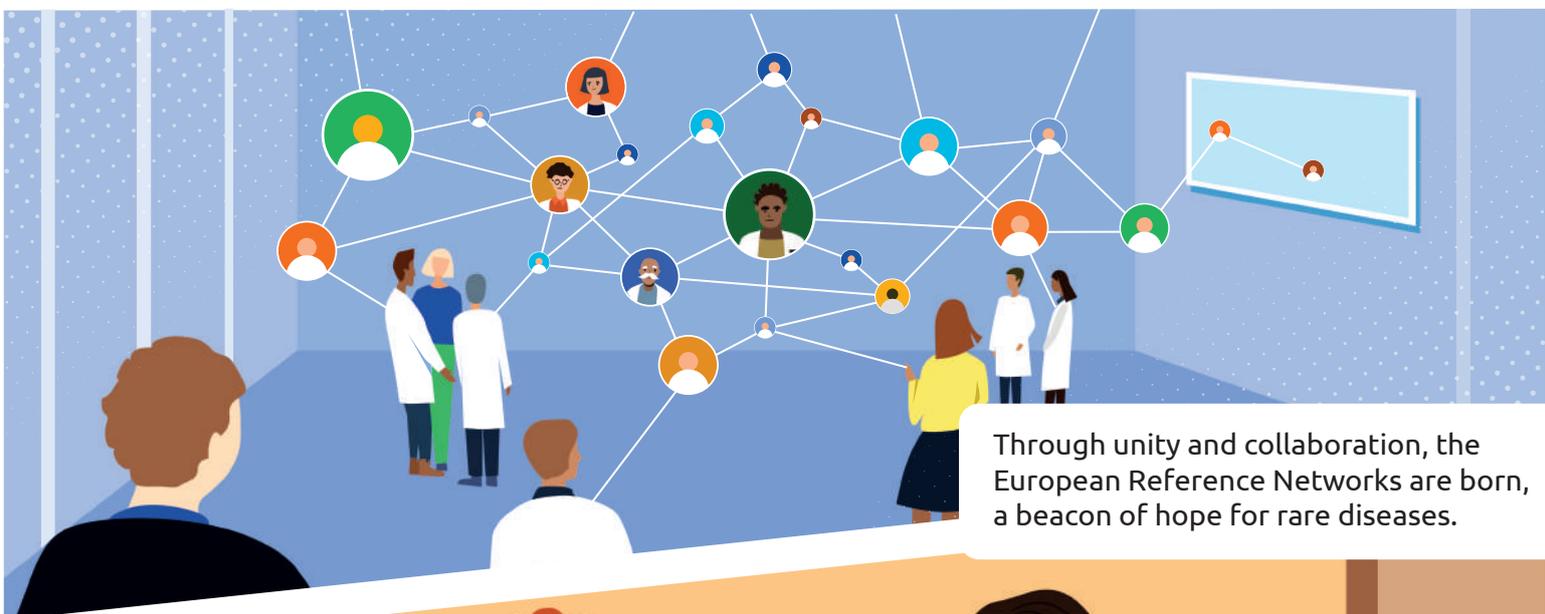
# European Reference Networks: Share, Care, Cure

Advancing to the fifth significant milestone, we turn our focus to the launch of the European Reference Networks (ERNs) in 2017. ERNs represent a major innovation in health-care for rare diseases, facilitating unprecedented collaboration across EU member states by connecting healthcare providers and experts to discuss complex or rare diseases, sharing knowledge and resources to improve patient care.

In a sea of fragmented care, rare disease expertise lies isolated, hidden within national confines.



Camilo envisions a future where knowledge flows freely, connecting the dots across Europe.



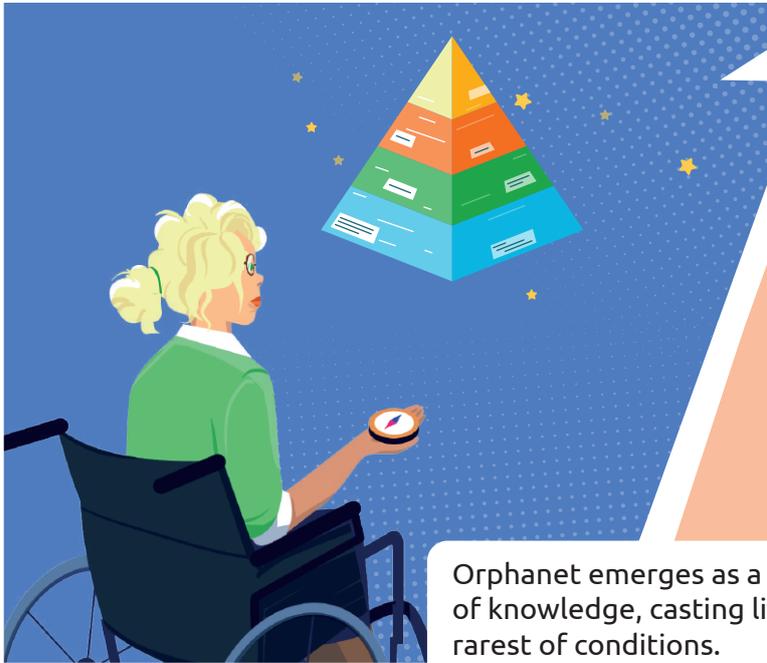
Through unity and collaboration, the European Reference Networks are born, a beacon of hope for rare diseases.



ERNs herald a new era, where every patient has access to the best minds in rare disease care, regardless of borders.

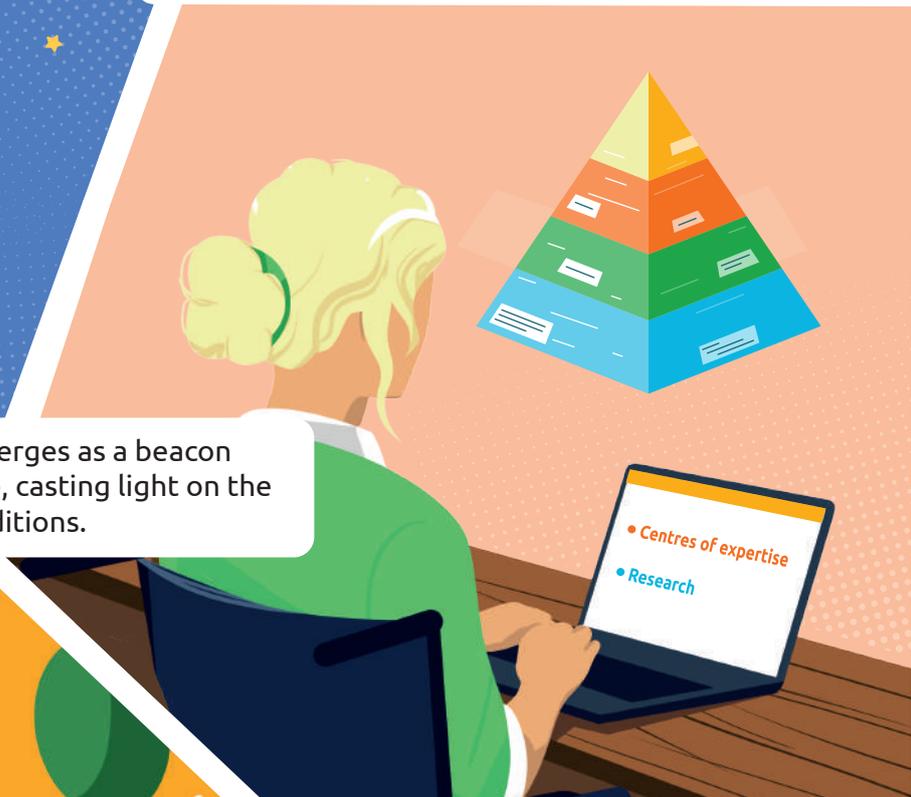
# Orphanet's 20th Anniversary

For the sixth significant achievement, we'll focus on the celebration of Orphanet's 20th anniversary in 2017. Orphanet has been an invaluable resource for patients, families, healthcare providers, and policymakers, offering a comprehensive database of information on rare diseases and orphan drugs, aiding in diagnosis, treatment, and research. Orphanet created the ORPHAcodes, a codification system that is crucial for making rare disease patients visible in hospitals and registries.



Navigating the rare disease landscape has long been a journey through the unknown, with information as scattered as the stars.

Orphanet emerges as a beacon of knowledge, casting light on the rarest of conditions.



In 2017, Orphanet celebrates two decades of illuminating the path for the rare disease community.



Orphanet's legacy is empowerment and visibility, ensuring that the quest for understanding and treatment continues with renewed vigour.



# Launching the European Joint Programme on Rare Diseases

Progressing to the seventh milestone, we'll explore the launch of the European Joint Programme on Rare Diseases (EJP RD) in 2019. This initiative represents a significant investment in collaborative rare disease research across Europe, aiming to streamline efforts, share resources, and accelerate the development of diagnostics and treatments.

Rare disease research, rich in potential, yet fragmented across Europe, struggles to find a unified pace.



In 2019, the dream crystallises into action; the European Joint Programme on Rare Diseases is launched.

Camilo imagines a Europe connected in its quest for answers, a joint force against rare diseases.



Pooling resources and minds, EJP RD promises to accelerate the path to discovery, turning hope into tangible outcomes.



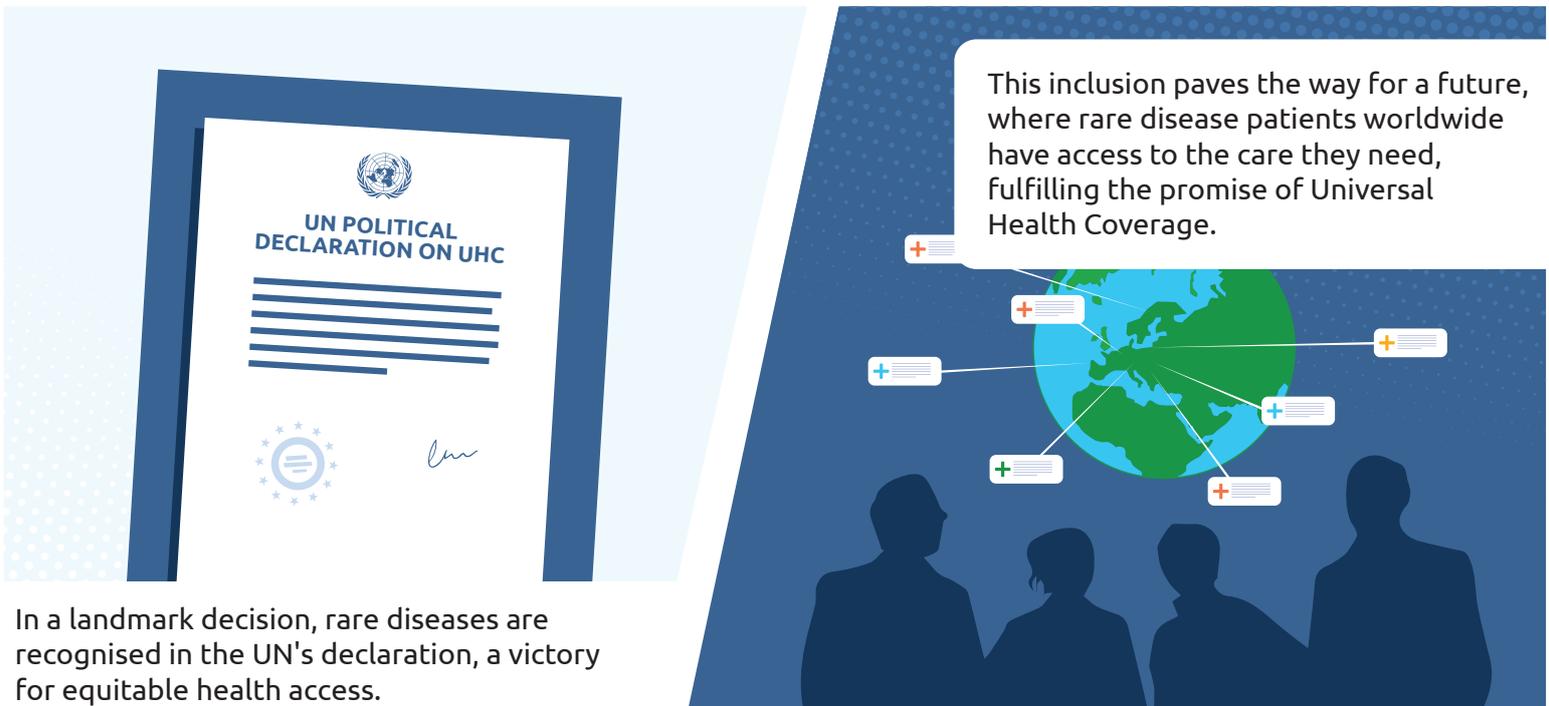
# Rare Diseases and the UN Political Declaration on UHC

Advancing to the eighth achievement, we'll focus on the inclusion of rare diseases in the United Nations' Political Declaration on Universal Health Coverage (UHC) in 2019. This inclusion marks a significant global acknowledgment of rare diseases, advocating for their integration into health systems worldwide to ensure equitable access to care and treatment for all.

Rare diseases are a global challenge, demanding a universal health coverage that ensures access to essential services without being pushed into poverty by the cost of healthcare.



Armed with evidence and unity, Rosalind presents a compelling case to the world's leaders.



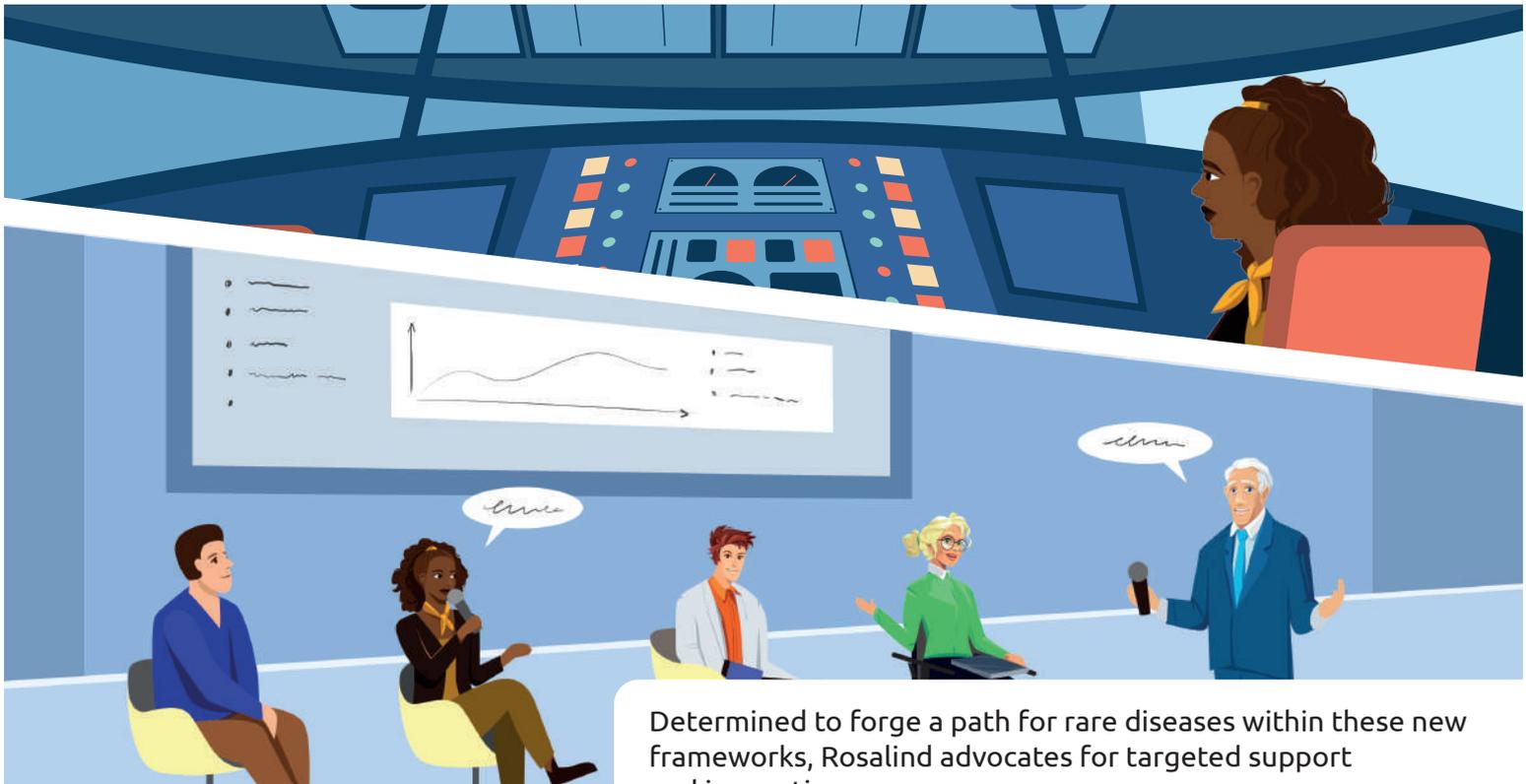
This inclusion paves the way for a future, where rare disease patients worldwide have access to the care they need, fulfilling the promise of Universal Health Coverage.

In a landmark decision, rare diseases are recognised in the UN's declaration, a victory for equitable health access.

# The New Funding Programmes: EU4Health & Horizon Europe

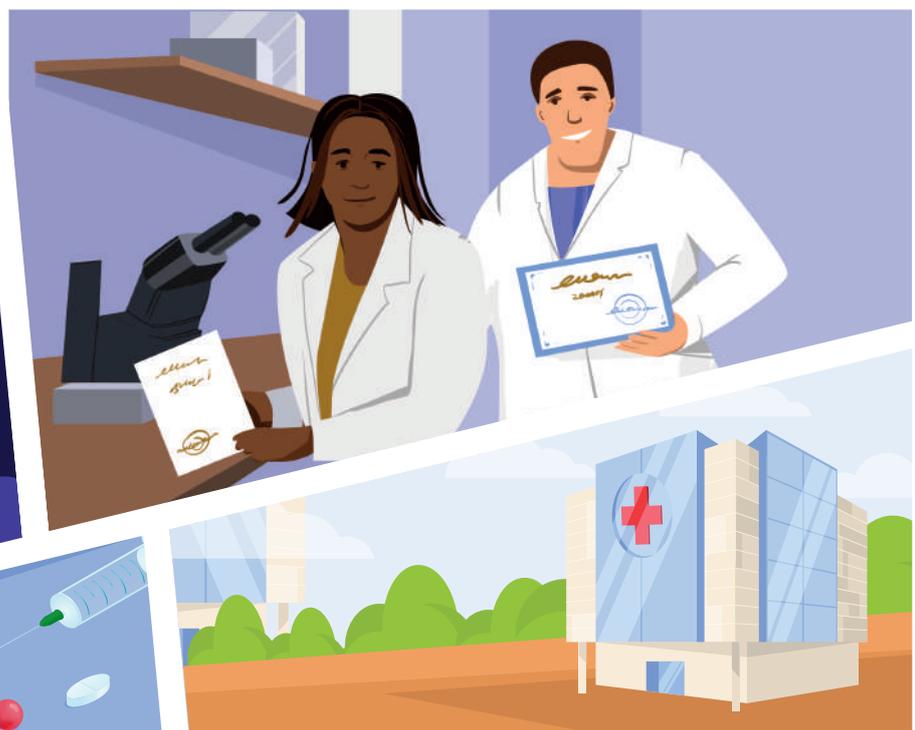
Moving on to the ninth milestone, we explore the initiation of the EU4Health Programme for 2021–2027. This ambitious health initiative aims to bolster the EU’s health systems, making them more resilient to future health threats, and includes specific provisions to support rare disease patients, research, and infrastructure.

In the aftermath of global health crises, the vision for a resilient and inclusive health system in Europe takes shape.



Determined to forge a path for rare diseases within these new frameworks, Rosalind advocates for targeted support and innovation.

With today’s launch, EU4Health and Horizon Europe promise to revolutionise healthcare in Europe, integrating rare diseases into its core mission.



Now, rare disease care receive a significant boost, marking the dawn of a new era in European healthcare.



**EU4HEALTH  
& HORIZON EUROPE:  
BUILDING A HEALTHIER  
FUTURE FOR EUROPE.**

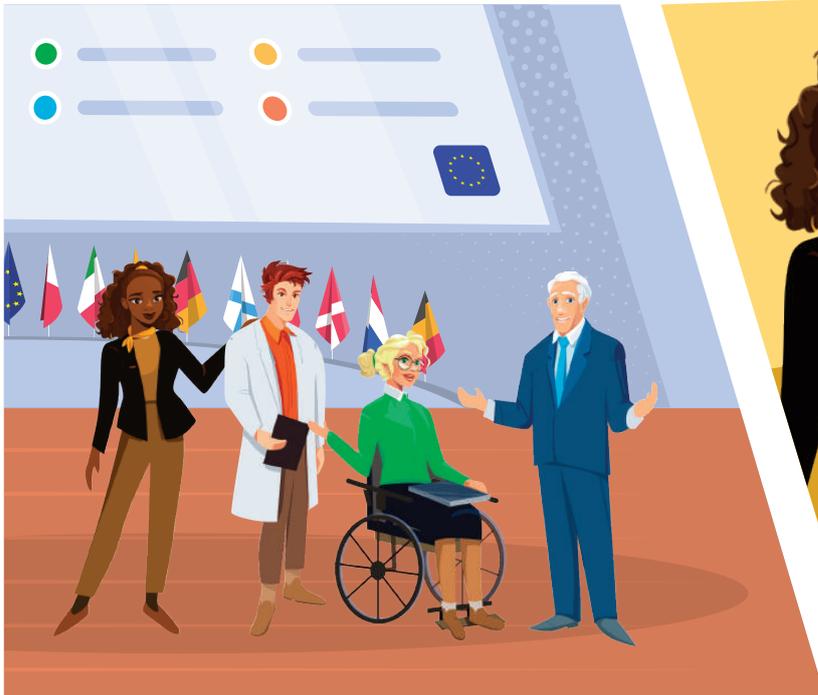
# Rare 2030 Foresight Study: A Call for a European Action Plan

Advancing to the tenth milestone, we focus on the Rare 2030 Foresight Study's recommendations, published in 2021. This pivotal report provided a comprehensive roadmap for the future of rare disease policy in Europe, outlining strategic recommendations to enhance research, diagnosis, treatment, and care for rare disease patients over the next decade.

Navigating the future of rare diseases requires foresight, a vision to guide us through the complexities ahead.



Together, they listen, learn, and compile a wealth of knowledge, shaping the Rare 2030 Foresight Study.



The Rare 2030 recommendations, which include a call for a comprehensive, revamped strategy for rare diseases in Europe, offer clear directives for the next decade.



With this blueprint and its call for a unified strategy, Europe is poised to transform the future of rare disease care and research.

# The UN Resolution for Rare Diseases

Moving to the eleventh significant milestone, we examine the adoption of the first-ever United Nations Resolution on 'Addressing the Challenges of Persons Living with a Rare Disease and their Families' in 2021. This groundbreaking resolution recognised the unique challenges faced by the rare disease community on a global scale, urging member states to integrate rare diseases into their national health plans and strategies, thereby ensuring comprehensive support and care.

The rare disease community's voice, long echoing in the corridors of power, now commands the global stage.



Together, they advocate for recognition, support, and action, sharing stories and data that weave a compelling narrative for change.



In a landmark decision, the resolution is adopted, marking a historic moment of global solidarity for rare diseases.

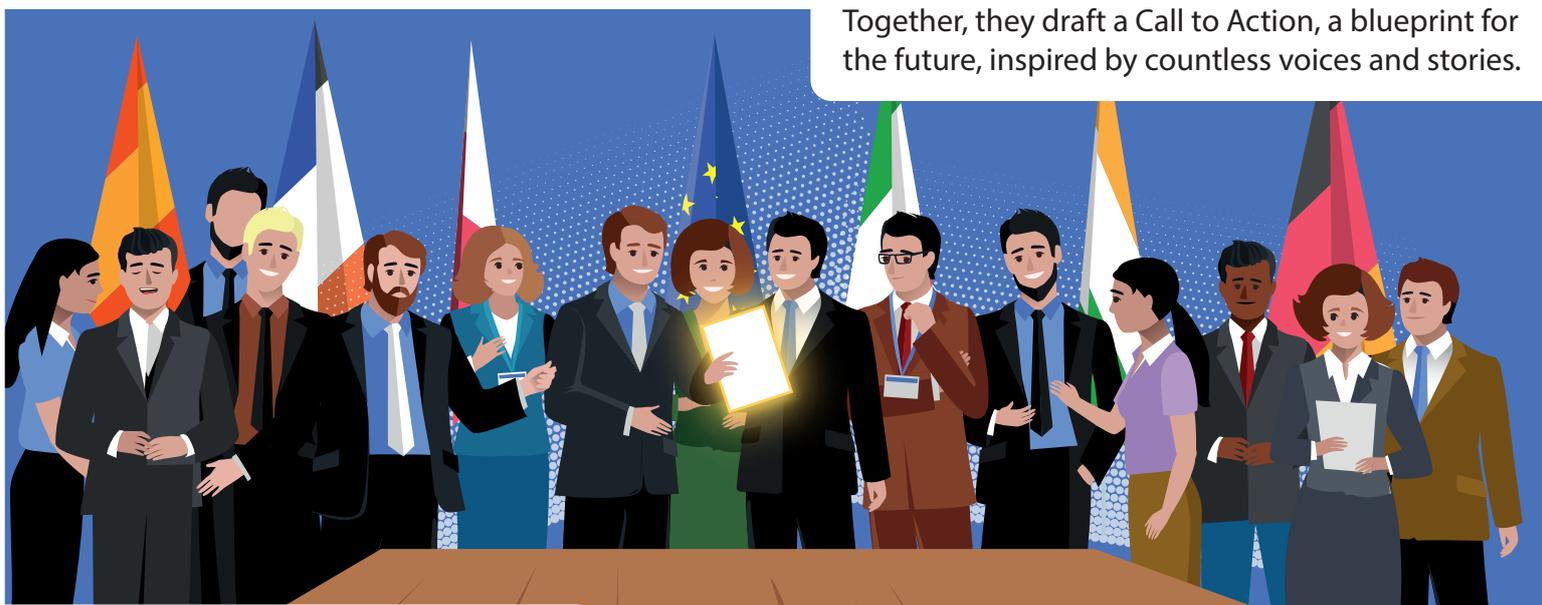
This resolution lights the path forward, promising a future, where no one with a rare disease is left behind, regardless of where they are in the world.



# The Call to Action on Rare Diseases

Transitioning to the twelfth and final significant achievement, we spotlight the endorsement by 21 Member States of the Czech EU Presidency's Call to Action on rare diseases in 2022. This collective endorsement represents a significant commitment from a majority of EU countries to enhance cooperation, support, and resource allocation for rare disease research, care, and policy development, further solidifying the EU's role as a leader in the global rare disease community.

The journey of rare diseases in Europe reaches a pivotal juncture, as 21 nations acknowledge the need for unity and action.



Together, they draft a Call to Action, a blueprint for the future, inspired by countless voices and stories.

In a show of solidarity, 21 Member States endorse the Call to Action, pledging to transform the landscape of rare diseases.

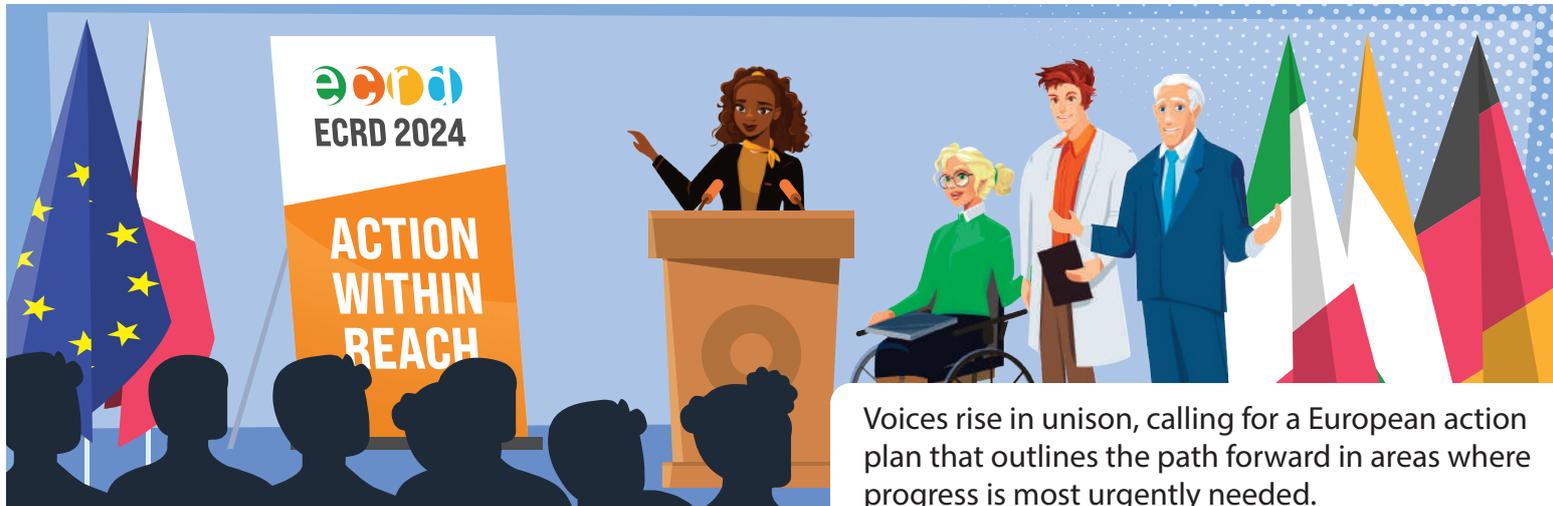


This commitment weaves a stronger Europe, where rare diseases are met with unprecedented support, cooperation, and hope.

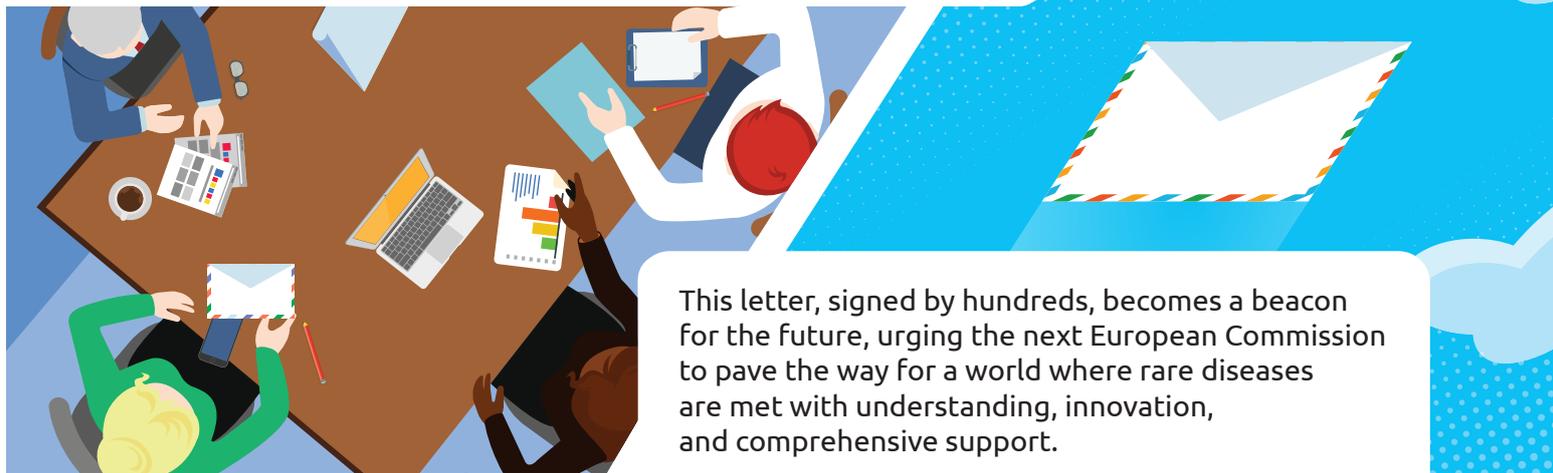
# An Open Letter to the Future

Now we arrive at a moment of collective action at the ECRD 2024. Here, our champions and the broader rare disease community seize the opportunity to influence future policies directly by addressing the next European Commission.

At ECRD 2024, the rare disease community converges, ready to spark a new wave of advocacy and action.



Together, they draft an open letter to the future European Commission, a manifesto for change rooted in unity and shared vision.



As our narrative concludes at the ECRD 2024, Emilia, Camilo, Rosalind, and Damiano, alongside the rare disease community, have cast a powerful message into the future. Their journey through the twelve labours, culminating in the open letter to the European Commission, symbolises not just the achievements of the past but a steadfast commitment to the progress yet to come. The unity, resilience, and collective voice they have showcased are a testament to the strength of the rare disease community. The impact of the actions of our heroes continues to resonate, promising a brighter, more inclusive future for people with rare diseases across Europe and beyond.

In the heart of Europe, where history and innovation intertwine, a compelling narrative unfolds – one that transcends borders and bridges the gap between hope and reality for those living with rare diseases. This is the story of four champions: Emilia, Camilo, Rosalind, and Damiano.

Each, touched by the challenge of rare diseases, whether personally or professionally, embarks on a transformative journey. They are driven by a shared mission: to catalyse change, break down barriers, and illuminate the path toward a future where rare diseases are no longer an odyssey of the unknown but a journey met with understanding, support, and cutting-edge care.

From the cobblestone streets of Poland to the academic halls of Portugal, from the policymaking corridors in the Netherlands to the vibrant heart of Italy, our champions gather their strengths, expertise, and experiences. Together, they form a mosaic of hope – each piece a testament to the resilience and determination of the rare disease community.

As we delve into their journey, we traverse the landscape of European health policy and innovation, witnessing firsthand the triumphs and challenges that have shaped the rare disease community over the past two decades. Each 'labour'—a milestone achievement in the realm of rare diseases – marks a step forward in the relentless pursuit of equity, access, and excellence in healthcare.

Join us as we journey with Emilia, Camilo, Rosalind, and Damiano through their twelve labours, a modern odyssey that mirrors the heroic quests of old, yet grounded in the real-world victories and ongoing battles of the rare disease community in Europe. Their story is one of unity, advocacy, and unwavering hope – a beacon for all those navigating the complex waters of rare diseases.

**In Europe, four champions – Emilia, Camilo, Rosalind and Damiano – tackle rare diseases, driven by a shared mission.**

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2024