WORLD OrphanDrug Congress Europe 2024

2024 POST EVENT REPORT

STRATEGY, ADVOCACY & PARTNERING FOR THE GLOBAL ORPHAN DRUG INDUSTRY

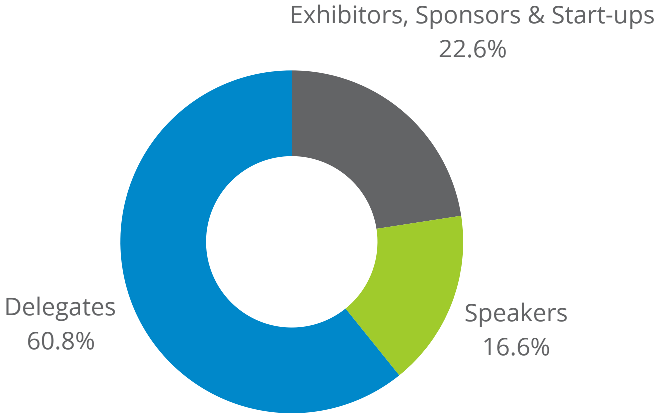
WE ARE MOVING TO AMSTERDAM IN 2025!

THE CONGRESS IN NUMBERS

THE WORLD'S LEADING ORPHAN DRUG & RARE DISEASE EVENT

The 15th World Orphan Drug Congress in Barcelona brought together the most senior orphan drug and rare disease experts across the industry to participate in four days of collaboration and innovation.

Type of Attendees



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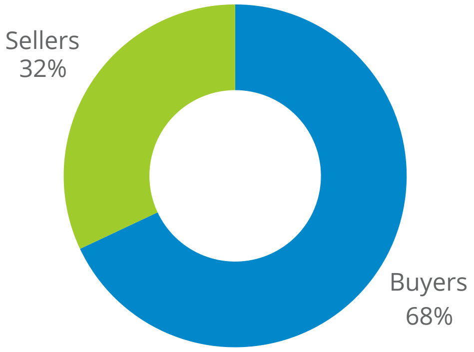
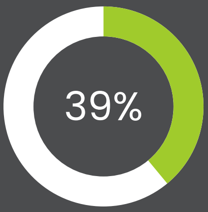
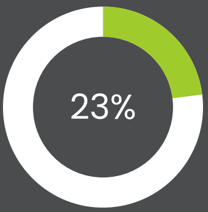


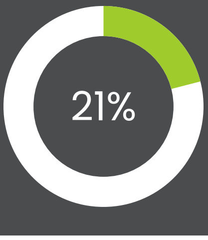
Industry Breakdown - 1653 Attendees (2000 Registered)



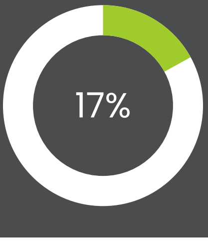
Pharmaceutical & Biotechnology



Consultants, CROs & CMO/CDMO



Patient Services, Healthcare Providers and Patient Advocates



Government, Academic & Other

REPRESENTING
80+
COUNTRIES

ATTENDEES ON THE MAP

This year's Congress brought together stakeholders from across the globe. Help us fill in the map and join us next year!

ATTENDEES FROM TOP 10 COUNTRIES

1. Spain
2. United Kingdom
3. United States
4. France
5. Switzerland
6. Germany
7. Belgium
8. Italy
9. Netherlands
10. India

Attendee Profiles

CEOs / Founder / C Suite/ VP

20%

Head / Director / Senior Manager

57%

PRE-CONGRESS WORKSHOPS

volv

CREATING REAL IMPACT FOR RARE DISEASE PATIENTS

CLINIGEN

INTEGRATING ESG, PATIENT ENGAGEMENT, AND COMMERCIAL STRATEGY FOR IMPROVED EU ACCESS

Chiesi

REDUCING THE BURDEN OF RARE DISEASES ACROSS THE PATIENT JOURNEY

sanofi

THE RARE DISEASE ACTION PLAN- HOW WILL WE IMPLEMENT THIS?

POA

NEVERMIND THE B******S, HERE'S THE EUJHTA BASICS

Mark your calendars for our 2025 Pre-Congress Day on **27th October**. Interested in hosting your own busy workshop next year?

Please contact **Kieran** at **kieran.duffin@terrapinn.com** and **Adam** at **adam.mayar@terrapinn.com** for more details.

CONFERENCE

THE START-TO-FINISH OF ORPHAN DRUGS

From cell and gene therapies to orphan drug policy to pricing and manufacturing, the Congress had it covered. There were **over 250 speakers** gathered sharing their insights and expertise.

The conference opened with insightful remarks from Soraya Bekkali of Alexion AstraZeneca Rare Disease. This set the stage for two engaging keynote panels that delved into the significance of robust pharmaceutical strategies and our preparedness for the implementation of EUHTA.

The congress continued with a variety of keynote panels, poster presentations, and dedicated conference tracks over three days, concluding with a keynote panel focusing on the role of real-world data in orphan drug development.

> **“Great networking opportunities, great panels and discussions.”**
> **- EURORDIS**

WORLD OrphanDrug
Congress Europe 2024

8 KEY TOPICS

Access & Pricing Orphan Drugs

Cell & Gene Therapy Orphan Drugs

Clinical Development Orphan Drugs

Patient Centricity Orphan Drugs

Precision Medicine Orphan Drugs

Real World Evidence Orphan Drugs

Science Strategy Orphan Drugs

Technology Showcases Orphan Drugs

4 FANTASTIC DAYS!

KEYNOTE PANELS

DAY ONE

1. How will Europe remain competitive in the C&GT and Rare Disease sector? The role of the research and innovation policies for a stronger pharmaceutical strategy in Europe

2. 12 weeks to 2025's EUHTA implementation – are we ready?

DAY TWO

1. The Rare Disease Action Plan – where are we and how to integrate this from a European to a national level for real impact?

2. How much weight does the patient voice carry when it comes to influencing clinical trials, policy and orphan drug approvals?

3. How do we reinvent the healthcare system to meet the demand of not only rare diseases, but potentially all patients in future?

DAY THREE

1. How to create a sustainable and robust pipeline for rare diseases and gene therapies

2. The use of RWD in orphan drug development and access pathways

KEYNOTE HALL OF FAME

Alison Cave
Chief Safety Officer
MHRA

Stelios Kympouropoulos
Former Member of the
European Parliament

Dr Virginie Bros-Facer
CEO
EURORDIS - Rare Diseases Europe

Sofie Alverlind
Project Leader
TLV

Claire Skentelbery
Director General
EuropaBio

Elisabetta Zanon
Director, European Policy and Advocacy
Alliance for Regenerative Medicine

Fabienne Bartoli
General Director
Haute Autorité de Santé

Daniel de Vicente
Board Member, **FEDER**,
President, **Association of Patients with ASMD**

Tim Leest
Clinical Assessor, Chair of the European Committee of Orphan Medicinal Products
Federal Agency for Medicines and Health Products (FAMHP)

Frauke Naumann-Winter
Committee for Orphan Medicinal Products
Institute for Drugs and Medical Devices

Dr Manuel Toledo Argany
Epileptologist
Vall d'Hebron Barcelona Hospital

Ella Fitzpatrick
Senior Public Involvement Adviser
NICE

Maurizio Scarpa
Director, Coordinating Center For Rare Diseases
MetabERN

Fleur Chandler
Head of Market Access UK and Ireland, **Sanofi**
& Parent Board Advisor, **Duchenne UK**

Ulrike Schwerdfeger
Legal Technical Officer
WHO

David Pearce
Chair
IRDiRC

Dr James A Levine
President
Foundation Ipsen

Dr Cesar Hernandez
General Pharmacy Director
Spanish Ministry of Health

Ella Fitzpatrick
Senior Public Involvement Officer
NICE

Matt Bolz-Johnson
Mental Health Lead & Healthcare Advisor
EURORDIS

THANK YOU TO OUR ADVISORY BOARD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Fabienne Bartoli
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French Ministry of Health


Toon Digneffe
Head Public Affairs & Public Policy
Takeda


Sheela Upadhyaya
Life Science Advisor in Rare Diseases ...
Former FIPRA

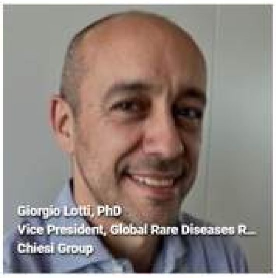

Charlotte Chanson
Director, Global Diagnostics & Newbor...
Orchard Therapeutics


Josie Godfrey
Former Associate Director of NICE
Rare Disease, Market Access, JG Zebr...


Dr Soraya Bekkali
Senior Vice President, International Co...
Alexion, AstraZeneca Rare Diseases


Anna Bucsics
Project Advisor
MoCA


Dr Alexander Natz
Secretary General
EUCOPE


Giorgio Lotti, PhD
Vice President, Global Rare Diseases R...
Chiesi Group


Samantha Parker
Patient Advocacy and Communication ...
Italfarmaco


Dr Violeta Stoyanova-Beninska
Chair of Committee for Orphan Medici...
Policy, Regulatory and International Af...


Wills Hughes-Wilson
Head of Patient Access & Commercial ...
Mereo BioPharma


Anne-Sophie Chalandon
Head of Global Affairs, Rare Diseases ...
Sanofi


Marcus Guardian
General Manager
International Horizon Scanning Initiative


Martine Zimmermann
Senior Vice President, Head of Regulat...
Ipsen


Karen Facey
Senior Research Fellow
University of Edinburgh

EXHIBITION FLOOR

This year’s exhibition featured **over 70** exhibitors and attracted **1,653** attendees, creating a vibrant atmosphere filled with interactive and engaging booths. Exhibitors had the opportunity to connect directly with their target audience, generate new sales leads, enhance their brand presence, and finalise deals with customers right on the spot.

WORKING GROUPS

This year we introduced Working Groups which fostered more intimate discussions over the key topics in the orphan drug and rare disease landscape.

POSTER ZONE

We had a record number of posters this year, with **over 70 posters** displayed.
This was a fantastic forum for individuals to share research, new innovations and rare disease awareness on the exhibition floor.

COMPANY SHOWCASES

Highlighting the different rare disease programs that companies around the world have developed. Understand what are the current ongoing efforts are, available capabilities and why you should consider partnering with them.

START-UP ZONE

Providing a platform for rare disease start-ups to meet potential partners and investors. This is the best opportunity to pitch your solution. Interested for 2025? Contact marc.rhys-evans@terrapinn.com to get involved.

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Congress Europe 2024

THANKS TO OUR SPONSORS

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ALEXION
AstraZeneca Rare Disease

DIAMOND SPONSOR

CLINIGEN

PLATINUM SPONSORS

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volv

Pfizer

ucb Inspired by patients. Driven by science.

IPSEN Innovation for patient care

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WEP With Every Patient.

AND MANY MORE...

PATIENT ZONE

Our patient zone provided the unique opportunity to engage with patient advocacy groups and ERNs who are at the heart of the rare disease community.

This year we had over 250 patient advocates representing patient groups from across the globe.

Our agenda features a Patient Centricity track to represent the patient voice and to hear first hand what has been achieved and what can be improved further.

ERDERA European Rare Diseases Research Alliance

AST Associació Síndrome de Turner de Catalunya

ERICA European Rare Disease Research Coordination and Support Action

ITHACA European Reference Network

ANSEDH Asociación Nacional Síndromes Ehlers - Danlos e Hiperlaxitud

IRDiRC International Rare Diseases Research Consortium

EURORDIS Rare Diseases Europe

Síndrome de Dravet Fundación
www.dravetfoundation.eu

Rare Diseases International

Information

Tuesday, October 22 · 7:00 AM - Friday, Octob
8:00 PM

NETWORKING

It's great to see the rare disease community come together for our event each year! This year's Congress was a unique opportunity to reconnect with colleagues and build new relationships through the networking breaks, networking drinks and around the exhibition floor.

Our dedicated networking team were also on hand to facilitate these important meetings.

15,000 + CONNECTIONS MADE!

The Terrapinn Events app offered a platform to connect and communicate with contacts throughout the Congress.

> "Great access to important colleagues, ability to connect or book time slots on Terrapinn Events App was great, varied sessions and good opportunities to network outside of the sessions."

YOUR FEEDBACK

“It is inspiring to see the **incredible stories** and **innovations** shaping the rare disease community while **connecting** with new people.”

“Always a great conference, where **key players** in the rare disease field come together and **share ideas**, and more importantly, agree on some concrete actions”

“The **leading congress** that addresses the subjects most important to patients with rare diseases and treatments by orphan drugs.”

Thank you!

MARK YOUR CALENDAR

The 16th edition of the World Orphan Drug Congress Europe will take place **27-30 October 2025** at the **RAI Amsterdam Convention Centre.**

We're excited to be moving to Amsterdam to leverage the well-established life science ecosystem.

Amsterdam is home to 400+ pharmaceutical and biotechnology companies, research institutions, government and healthcare organisations that focus on innovation in rare disease and orphan drug development.

See you there!

www.terrapinn.com/WODC

OUR TEAM

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