16TH EDITION

Innovation, advocacy and partnering for the orphan drug & rare disease industry

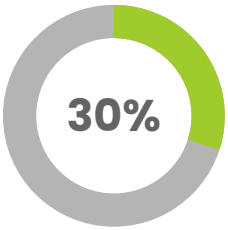
2025 POST EVENT REPORT

WORLD OrphanDrug Congress Europe 2025

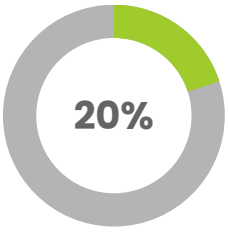
THE CONGRESS IN NUMBERS

The 16th World Orphan Drug Congress made its debut in Amsterdam, Netherlands, bringing together the most senior orphan drug and rare disease experts across the industry to participate in three days of collaboration and innovation.

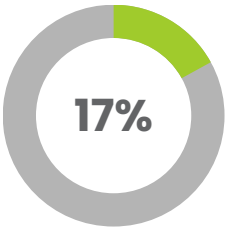
ATTENDEE BREAKDOWN



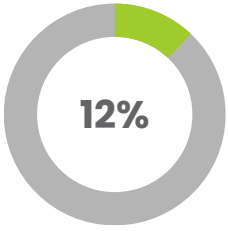
Pharmaceutical & Biotechnology



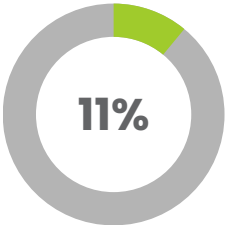
Patient Advocacy Organisations



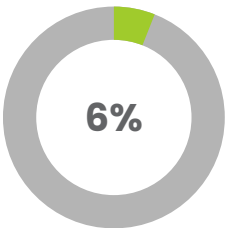
Service Providers



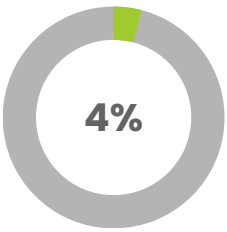
NGOs / Non-Profits / Hospitals



Consultants

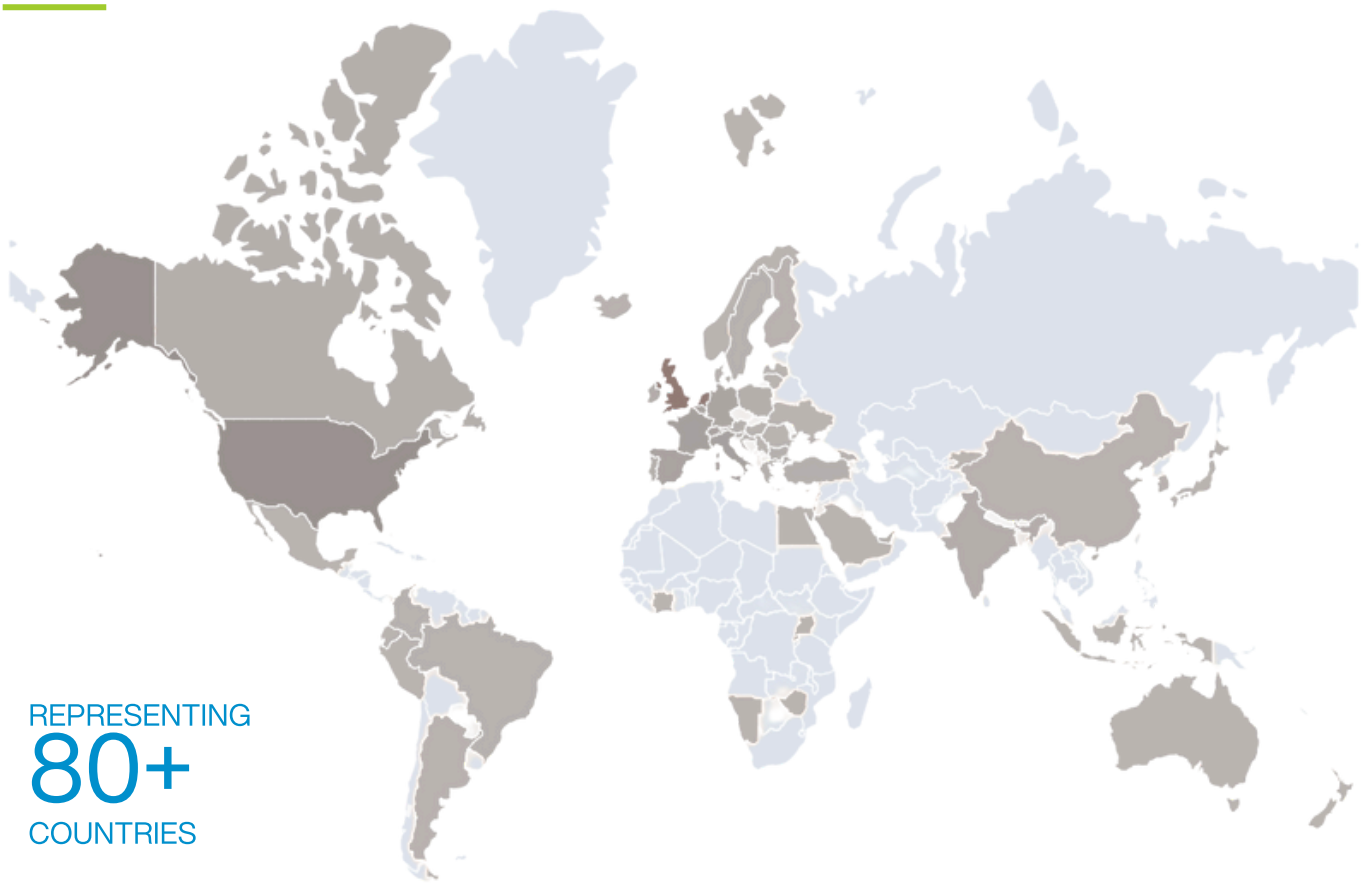


Academia



Government / Regulators

ATTENDEES ON THE MAP



REPRESENTING
80+
COUNTRIES

ATTENDEES TOP 10 COUNTRIES

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

Attendee Profiles

Founder / C Suite / President / VP / Head / Director / Senior Manager

77%

PRE-CONGRESS WORKSHOPS

Designed for in-depth insights and greater stakeholder engagement into the orphan drug value chain, the pre-congress workshops on Monday 27 October feature a multitude of panel discussions and presentations covering the full spectrum of orphan drug development, access and strategies for greater patient involvement.

ERDERA - Innovative Methodological Approaches Accelerating Rare Diseases Drug Development: Bridging Data, Innovative Design, and Decision-Making

CLINIGEN - Defeating uncertainty: Effectively Manage the Journey from Clinical Trials, OD Designation through to Patient Access

SANOFI - Public Private Partnerships in Rare Disease: Ideas to action

SCIENSUS - Accelerating a patient powered pathway from development to market in Europe with rare therapies

CHIESI - Enabling Digitally-Enhanced Care for Rare Diseases in Europe

Our pre-conference workshop was well attended and the questions raised around Orphan Drug access really stimulated healthy debate, and some good solutions to challenges.

Kieron Lewis, Director of Strategic Consulting, Clinigen

Mark your calendars for our 2026 Pre-Congress Day on 26th October.
Interested in hosting your own busy workshop next year? Please contact Steph Scanlon (steph.scanlon@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 a high-level overview of what's to come in 'Europe's Life Science Strategy' and what does it mean for Rare Disease? We had The French Ministry of Health, EU Commission, EURORDIS and industry discussing the global impact of U.S. changes and what opportunities could this mean for Europe. Following on from last year's pharma preparedness for the implementation of the EU HTA we had an esteemed panel of experts from TLV, ZIN and AEMPS discussing the current EUHTA landscape and how well your implementation has gone, moderated by the wonderful Karen Facey, Senior HTA Advisor at FIPRA and RWE4Decisions.

Day 2 didn't slow down with two more keynote panels littered with experts discussing 'Leveraging new technology for smarter decision making when in Rare Disease drug Development and Access' followed by 'New treatments on the block' covering the current 2025 gene therapy pipeline and what's imminent on the horizon.

WODC covers three action packed days of Workshops, Working Groups, Start-up Pitches, Poster Presentations, BioPharma Showcases, Panel Discussions and Stand-alone presentations that are not to be missed if attending Europe's leading Rare Disease and Orphan Drug event.

10 KEY TOPICS

Access & Pricing	Understand the dynamics governing global rare disease markets & what interventions can be made for better access.
BioPharma Showcases	Highlighting upcoming and existing orphan drug developers and what's new on the horizon. Showcasing the latest pipeline, potential partnering opportunities and highlighting the growing interest in multiple disease areas.
Cell & Gene Therapy	Find out how the latest gene therapies are transforming patients living with rare diseases, and what the future holds for the next wave of innovative medicines.
Clinical Development	Understand how to work with small patient group sizes using meaningful endpoints with patients and regulators, moving your orphan drug candidates through development phases to licensure.
Investment & Funding	Innovative funding strategies and what do investors look for before investing in rare disease. Learn about what investment models there are to overcome Orphan drug development bottlenecks.
Manufacturing	The future of manufacturing within Cell & Gene therapy and best practices to overcoming manufacturing and commercialisation obstacles.
Patient Centricity	Truly represent the patient voice and advocate for them by hearing from our partners and finding out firsthand, what has been achieved and what can be improved further.
Precision Medicine	From new gene editing technologies to new born screening, find out how precision medicine is changing the way we look and define rare disease diagnosis and treatment.
Real World Evidence	Find out how to use real world evidence should be evaluated for potential applications to increase speed of product approval, reimbursement and clinical practice adoption.
Science Strategy	Bridge the gap between science, policy and corporate strategy in order to secure commitment from all stakeholders.

KEYNOTE PANELS

DAY ONE

1. What will Europe's life science strategy hold and what does it mean for rare diseases?
2. How well has your EUHTA implementation gone?

DAY TWO

1. Leveraging new technology for smarter decision-making in rare disease drug development and access.
2. New treatments on the block – 2025's rare disease pipeline.
3. In what way can we mobilise, enable and leverage international policies around the world for PLWRDs?

"It was an honour to participate at the keynote panel and truly inspiring to engage in discussions with such leading and knowledgeable experts, contributing to an event that is advancing the dialogue around orphan drugs. The collective expertise shared during our panel highlighted the importance of our work in this field and that collaboration with all the stakeholders is a driver for future innovation around orphan medicines."

Christina Kyriakopoulou, *Senior Scientific Policy Officer*, **EU Commission**

KEYNOTE HALL OF FAME

Fabienne Bartoli
Inspector General
French Ministry of Health

Janneke van de Kamp
CEO
Norgine

Christina Kyriakopoulou
Senior Scientific Policy Officer, DG Research & Innovation
European Commission

Rosa Castro
Public Affairs Director
EURORDIS – Rare Diseases Europe

Toon Digneffe
Head Public Affairs & Public Policy
Takeda

Ray Huml
Vice President of Rare Strategy
Sciensus

Violeta Stoyanova-Beninska
Senior Scientific Specialist
EMA

Durhane Wong-Rieger
President and Chief Executive Officer
Canadian Organization for Rare Disorders

Niklas Hedberg
Chief Pharmacist
TLV

Anne-Sophie Chalandon
Head of Rare Diseases Global Public Affairs Rare Disease and ATMP Policy
Sanofi

Alexander Natz
Secretary General
EUCOPE

Jon Neal
Head of Central, South and Eastern Europe
Takeda

Diego Fernando Gil Cardozo
President
FECOER

Claire Skentelbery
Director General
EuropaBio

Christopher M de M Rudolf
Founder & CEO
Volv

Julian Isla
Director
Foundation 29

Stelios Kympouropoulos
Former Member
European Parliament

Jennifer Schranz
SVP, Global Head Rare Diseases
Ipsen

Sophie Schmitz
Managing Partner
Partners4Access

Yann Le Cam
Founder and Former CEO, EURORDIS; Consultant,
Voz Advisors

SCIENTIFIC ADVISORY BOARD

Yann Le Cam
Founder and Former CEO, EURORDIS; Consultant,
Voz Advisors

Daniel O'Connor
Director Regulatory and Early Access Policy
ABPI

Rick Thompson
CEO
Beacon for Rare Diseases

Durhane Wong-Rieger
President and CEO
Canadian Organization for Rare Disorders

Toon Digneffe
Head Public Affairs & Public Policy
Takeda

Sheela Upadhyaya
Life Science Advisor in Rare Diseases & Special Advisor Former FIPRA

Charlotte Chanson
Director, Global Diagnostics & New-born Screening
Orchard Therapeutics

Josie Godfrey
Former Associate Director, **NICE**, Rare Disease, Market Access,
JG Zebra Consulting

Anna Bucsics
Project Advisor
MoCA

Dr Alexander Natz
Secretary General
EUCOPE

Giorgio Lotti
Vice President, Global Rare Diseases R&D Pipeline Portfolio
Chiesi Group

Samantha Parker
Patient Advocacy and Communication Lead Rare Diseases Europe
Italfarmaco

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

Anne-Sophie Chalandon
Head of Global Affairs, Rare Diseases & CGT Policy Speciality Care
Sanofi

Marcus Guardian
General Manager
International Horizon Scanning Initiative

Karen Facey
Senior Research Fellow
University of Edinburgh

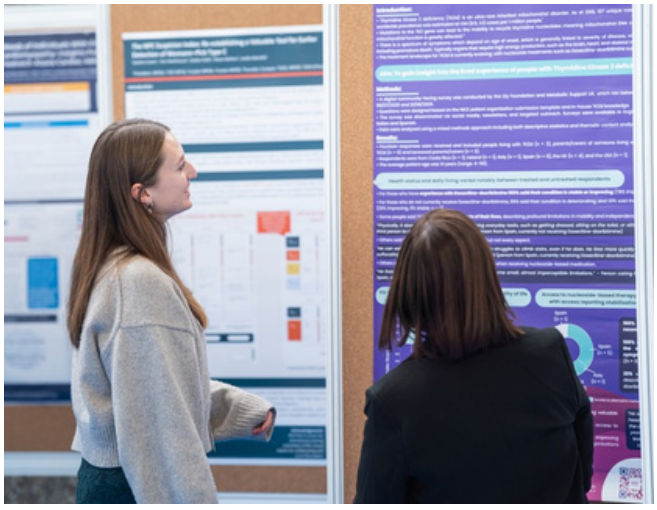
Violeta Stoyanova-Beninska
Chair of Committee for Orphan Medicinal Products
EMA

Fabienne Bartoli
Inspector General for Social Affairs
French Ministry of Health

Dr Soraya Bekkali
Senior Vice President, International Commercial Operations
Alexion, AstraZeneca Rare Diseases

EXHIBITION FLOOR

With **over 70 exhibitors** and **start-ups** this year, the exhibition floor was packed with a fantastic array of interactive and engaging booths to provoke discussion and encourage collaboration.



POSTER ZONE

60+ POSTERS

The Poster Zone was a central attraction at World Orphan Drug Congress Europe 2025.

his was a fantastic forum for individuals to share research, new innovations and rare disease awareness on the exhibition floor.

START-UP ZONE

This year our start-up zone was bigger than ever before, welcoming **24 start-ups** to participate in the congress.

2025 START-UP PITCH WINNER!

Genvade Therapeutics
recognised with the Best Start-up Pitch award at the World Orphan Drug Congress Europe 2025 for driving innovation in the rare disease ecosystem and delivering an outstanding presentation.

> This is 100% a valuable event to attend for people working in rare diseases or interested in rare diseases. We have seen like-minded people come together to talk about innovation, such as how we can give access to all patients worldwide.

Yannick Lampo
Business Development Associate
Sherwood Pharmaceuticals

Contact **alisa.forbes@terrapinn.com** to get involved and showcase your start-up.

THANKS TO OUR SPONSORS

PLATINUM SPONSORS

Sciensus

ucb Inspired by patients. Driven by science.

GOLD SPONSORS

Chiesi global rare diseases

CLINIGEN

CRA Charles River Associates

IPSEN

NORGINE

parexel

sanofi

SARTORIUS

RTI (h)(s) Health Solutions

Takeda

Worldwide Clinical Trials

volv AI-POWERED GLOBAL HEALTHC

Wemedoo Clinical Information Specialists

SILVER SPONSORS

Allucent Helping bring new therapies to light

Biogen

HEOR

Initiate.

premier research Built for Biotech

WEP With Every Patient.

BRONZE SPONSORS

25 CELEBRATING 25 YEARS. alchemist

AliraHealth Patient-Enabled Solutions

BRCR Global

caidya

costello

DENDRITE CLINICAL SYSTEMS LTD

RARE PATIENT Voice Also Non-Rare!

SGS When you need to be sure

VALID INSIGHT PART OF THE BIOSCRIPT GROUP

AND MANY MORE...

Thank you to our sponsors for helping make the World Orphan Drug Congress 2025 possible and for helping bring the rare disease and orphan drug community together. To discuss potential sponsorship opportunities for 2026, please get in touch with **steph.scanlon@terrapinn.com**

THANKS TO OUR PARTNERS

Alliance for Regenerative Medicine
ASE BIO
BARCELONA HEALTH HUB
beacon for rare diseases
biocat
BIOINSIGHTS
Bio Partner
BioPharma Asia
BIOTECH GATE GLOBAL DATABASE
BLACKSWAN FOUNDATION
CheckOrphan rare, orphan and neglected diseases
CheckRare rare disease insights
SÍNDROME DE DRAVET FUNDACION www.dravetfoundation.eu
eatris
Endo-ERN
EpiCARE
ERN ERA European Rare Diseases Research Alliance
ebn ERN | EuroBloodNet
ERN BOND EUROPEAN REFERENCE NETWORK ON RARE BONE DISEASES
EURACAN
European Reference Network ERN eUROGEN Rare Uro-Recto-Genital Diseases & Complex Conditions
ERN GUARD-Heart
ITHACA
European Reference Networks SKIN
EuropaBio
EBR European Biopharmaceutical Review
EUCOPE European Confederation of Pharmaceutical Entrepreneurs AISBL
EURORDIS RARE DISEASES EUROPE
FAMILIESCN2A FOUNDATION
SCN2A Germany e.V.
FH Europe Foundation
Gene Therapy Net www.genetherapynet.com
NL Health~Holland
hollandbio
IRDiRC INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM
Kidney Research UK
LAB A TO Z
LMNA Cardiac Diseases Network
mediaplaneta
NAVLIN BY EVERSANA
NEWS MEDICAL LIFE SCIENCES An AZoNetwork Site
patientworthy
PHARMA FOCUS EUROPE
Rare Disease 360 Powered by MedJournal360
RARE DISEASES GREECE
RARE REVOLUTION MAGAZINE
SCREEN 4CARE
touch ENDOCRINOLOGY
touch HAEMATOLOGY
touch OPHTHALMOLOGY
WORLD DUCHENNE ORGANIZATION UPPMD

Thank you to our partners for helping spread the word and raise awareness of WODC 2025. Want to support? Get in touch with **charlotte.thom@terrapinn.com** and **jasper.cameron@terrapinn.com**

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.

Our patient zone featured EURORDIS - Rare Diseases Europe, ERDERA, IRDiRC, FH Europe, Kidney Research UK, Dravet Syndrome Foundation and Rare Revolution Magazine.

This year we had **over 275 patient advocates** representing patient group organisations 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.

SHARE YOUR RARE

We featured a wall that invited attendees to share their rare journeys, giving a voice to patients and fostering awareness, connection, and empowerment.

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.

7,000 + CONNECTIONS MADE!

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

> "Excellent networking opportunities among stakeholders"
>
> **Miriam Wagner Long**, Project Mercury, **FSHD World Alliance**

SUSTAINABILITY & INCLUSIVITY

Discover how the World Orphan Drug Congress Europe is driving thoughtful eco-friendly practices, responsible partnerships, and innovative initiatives to reduce our environmental impact.

VENUE SELECTION & ACCESSIBILITY
World Orphan Drug Congress Europe 2025 took place at the RAI Convention Centre in Amsterdam.
RAI Amsterdam plays an active role in addressing the most urgent global challenges, striving to balance the three key dimensions of sustainable development – economic growth, environmental sustainability, and social inclusion.

ACCOMMODATION CHOICES
World Orphan Drug Congress Europe recommended eco-certified local hotels committed to minimizing environmental impact, ensuring a greener stay for attendees. We partnered with RAI Hotel Services to offer exclusive accommodation deals near the venue, including a 'Green hotel' filter for sustainable options.

RESPONSIBLE CONTRACTORS
World Orphan Drug Congress collaborates with contractors like Freeman, renowned for sustainability and accessibility, holding ISO 20121 and ISO 14001 certifications. Freeman is also a founding member of the Net Zero Carbon Events initiative, reinforcing its commitment to minimizing event carbon footprints. At the World Orphan Drug Congress, we are committed to using sustainable materials for our onsite signage and show-ready stands.

CARBON EMISSIONS TRACKING
World Orphan Drug Congress Europe is committed to tracking carbon emissions from attendee travel, waste, and energy use, and work toward reducing the event's carbon footprint and keeping stakeholders informed of progress.

PAPERLESS INITIATIVE
The World Orphan Drug Congress has adopted a fully paperless approach, significantly reducing paper waste, energy consumption, and transportation emissions while exploring innovative communication methods. All event information including the conference program can be found on the event app.

WELLBEING
Promoting attendee wellbeing was central to our sustainable event approach. We offered dedicated space to unwind with games or capture a great memory at our branded wall and WODC letters.

YOUR FEEDBACK

We're grateful for the positive feedback that has been offered so far, which helps us to continue improving the event for future iterations. A resounding theme from almost every comment is the value of connecting with the community in-person, which we agree is an invaluable feature of the Congress.

"Well run conference with insightful content and respected speakers."

Amanda Strickson
Co-Founder & Chief Operations Officer
Tolley Ltd

"Beyond the great conversations, panels, and scientific sessions - what touched me most was connecting with so many others from our rare disease community. Hearing these stories and shared experiences was a powerful reminder of why co-designed innovation truly matters, because we live it every day."

Thibault Krommenhoek
Strategic Partnerships Coordinator
Empowered By Us

"Attending the World Orphan Drug Congress in Amsterdam was an inspiring experience. It brought together passionate leaders, innovators, and advocates all working toward better solutions for patients with rare diseases."

Elizabeth Defendis
Government Business Development Specialist
BRCR Global

"Good sessions with current and urgent themes (such as access) and great network event."

Sylvia Macdonald
Senior Patient Advocacy Manager
Alexion Pharmaceuticals

"Well organized, excellent app, and interesting agenda."

Urbano Sbarigia
Global team rare diseases
UCB

"What really stood out for me at this congress is the huge variety of stakeholders attending from across the rare disease spectrum, from pharmaceutical companies, early-stage biotechs, regulatory and HTA agencies, to, of course, the most important attendees, the patients and their advocates."

Vanessa Buchanan
Head of health economics and HTA
Cogentia Healthcare Consulting

Thank you!

MARK YOUR CALENDAR

WORLD Orphan Drug Congress

AMERICA

9 - 11 June, 2026
Boston Convention & Exhibition Center, Boston, MA

EUROPE

26 - 28 October 2026
RAI Convention Centre, Amsterdam, NL

We hope to see you there!

WODC TEAM

As always, the event comes together because of the efforts of a fantastic team who work throughout the year to tailor it to the evolving demands of the orphan drug and rare disease industry. We're grateful that you joined us and hope to see you next year!

SPONSORSHIP & EXHIBITION

Steph Scanlon
steph.scanlon@terrapinn.com

SPEAKING OPPORTUNITIES

Abdu Kauroo
abdu.kauroo@terrapinn.com

MARKETING OPPORTUNITIES

Jasper Cameron
jasper.cameron@terrapinn.com

GENERAL ENQUIRIES

Marc Rhys- Evans
marc.rhys-evans@terrapinn.com

Wing-Yun Cheung
wing-yun.cheung@terrapinn.com

START-UP OPPORTUNITIES

Alisa Forbes
alisa.forbes@terrapinn.com