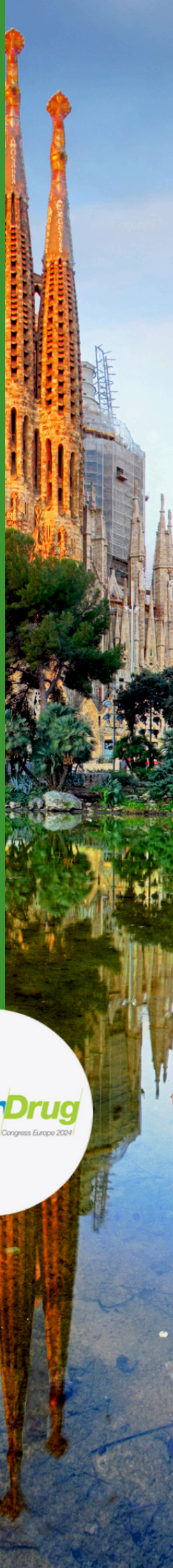
WODC

key takeaways

IN 60 SECONDS

Day Three at WODC Europe 2024



Remap

KEYNOTE 1

HOW TO CREATE A SUSTAINABLE AND ROBUST PIPELINE FOR RARE DISEASES AND GENE THERAPIES

SPEAKERS: Paolo Morgese, Victor Maertens, Daria Julkowska, Francis Pang, Thomas Butt

INTRODUCTION

Gene therapies are reshaping the future of rare disease treatments, holding the potential to address previously untreatable conditions. However, developing a sustainable and scalable pipeline for these therapies involves a range of scientific, regulatory, and commercial challenges. This keynote panel explored the hurdles and opportunities ahead, covering topics from scalability differences between therapy types to the need for updated regulatory policies and early stakeholder engagement.

THE DISCUSSION

How will Gene Therapies Impact Future Pipelines?

Gene therapies offer transformative potential, but their development requires navigating distinct challenges, depending on whether they are in vivo or ex vivo. Francis explained the key differences in business models for in vivo versus ex vivo therapies. In vivo therapies, which target cells within the patient's body, are more scalable, while ex vivo therapies, which involve manipulating cells outside the body before reintroducing them, are complex and must be tailored to each individual. This patient-specific approach requires specialised treatment centres, which are typically located in major cities and are not accessible to all patients.

Francis highlighted the importance of newborn screening for ultra-rare diseases. Early detection and intervention significantly improve treatment success, but it can take years for rare diseases to be added to newborn screening panels. To speed up this process, it is critical to engage with policymakers and key stakeholders early on.

Remaining Scientific and Commercial Challenges

Tom highlighted three key factors for gene therapy development: planning, flexibility, and expectation management. He advised companies to have a strong evidence base at the time of launch, as relying on post-market evidence gathering can hinder commercial success. Flexibility is essential, he noted, as each country may interpret evidence differently, even for the same therapy. Therefore, companies need to adjust their launch expectations and adopt country-specific payment models.

Tom also pointed out the distinct difference between the US and EU regulatory environments. Companies can expect stricter scrutiny on evidence in the EU, which underscores the need for tailored launch expectations and strategies for each market.

Daria stressed the importance of strong public-private sector relationships to ensure patients receive timely access to gene therapies. She argued that companies must keep patient needs at the forefront of their engagements with stakeholders, as successful collaborations across sectors will be essential for advancing gene therapies.

Regulatory Science and Policy Adaptation for Innovative Medicines

The future success of gene therapies relies on a supportive regulatory and policy framework. Victor called for a proactive approach in developing policies that will accommodate the rapid advancements in gene therapy. The EU, in particular, has the potential to lead by setting an example in creating a comprehensive and favourable regulatory landscape for gene therapies.

Tom noted a potential challenge with joint clinical assessments (JCAs). If these assessments are conducted at the time of launch, advanced medicinal therapy products (AMTPs) and gene therapies may receive negative evaluations due to a lack of long-term evidence.

Therefore, a structured approach to post-market evidence collection is necessary to support these therapies over time.

The panel also highlighted the importance of upskilling regulatory professionals. Gene therapies for rare diseases are complex, and regulators need specialised knowledge to understand the nuances of these treatments. Open, ongoing communication between regulators and developers is essential to facilitate the development of therapies for multiple indications without excessive regulatory pushback.

Looking Ahead: Changes for a more Sustainable Gene Therapy Pipeline

The panel outlined several changes they hope to see in the next three years to make gene therapies more viable and accessible:

- Updated legislation and a comprehensive rare disease action plan to support the development of gene therapies
- Incentives to launch therapies in the EU to attract manufacturers and boost local investment in gene therapy development
- Expanded newborn screening panels for early detection of rare diseases, enabling pre-symptomatic intervention where possible
- Stronger public-private collaborations, which are essential to pool resources and expertise
- Early engagement with stakeholders to increase the chances of regulatory and commercial success

Platform Technologies and Future Business Models

One potential solution to scalability and efficiency issues in gene therapy is platform technologies. The European Medicines Agency (EMA) has shown interest in platform technologies, but current policies and incentives may not fully support their development. The panel noted that a shift in policy direction could foster innovation and enable the use of platform technologies across multiple rare diseases, making gene therapy development more efficient.

HERE ARE OUR 5 TAKEAWAYS

1. **Scalability challenges differ for in vivo vs. ex vivo therapies:** in vivo therapies are more scalable, while ex vivo treatments require patient-specific customisation and specialised centres

2. **Importance of early and robust evidence:** companies need a solid evidence base at launch, as relying on post-launch evidence can delay market access

3. **Flexibility and adaptation are key to market success:** with varying regulatory expectations across countries, companies need adaptable strategies and payment models tailored to each market

4. **Policy and regulatory advancements in the EU are crucial:** updated legislation, HTA improvements, and attractive incentives are needed to position the EU as a leader in gene therapy

5. **Early stakeholder engagement and public-private collaboration:** involving stakeholders early and fostering strong partnerships will increase the likelihood of successful development and patient access to gene therapies

KEYNOTE 2

USE OF REAL-WORLD DATA IN ORPHAN DRUG DEVELOPMENT AND ACCESS PATHWAYS

SPEAKERS: Dan O'Connor, Alison Cave, Frauke Naumann-Winter, Dr Annika Jodicke, Sicily Mburu

INTRODUCTION

Real-World Data (RWD) has become a critical tool in orphan drug development, offering valuable insights into rare disease progression, patient outcomes, and treatment effectiveness outside traditional clinical trials. This keynote panel explored the potential of RWD to overcome barriers in the development of orphan drugs, improve patient access, and adapt regulatory pathways. The panellists discussed challenges in data quality, the importance of patient-centric outcomes, and advancements in data standardisation and infrastructure.

THE DISCUSSION

Overcoming Barriers in Real-World Data Collection

Real-world data in rare diseases presents both challenges and opportunities. High variability in rare disease data complicates analysis, and panellists stressed the importance of data quality, especially around patient-relevant endpoints and the length of follow-up periods. Learning from retrospective data, they noted, is essential to develop successful prospective data collection frameworks, as retrospective insights reveal trends in disease progression and treatment response.

For rare and ultra-rare diseases, traditional randomised controlled trials (RCTs) are often impractical. Regulatory agencies are increasingly turning to RWD as an alternative, including the use of historical controls. However, this shift necessitates robust safety and risk-benefit assessments tailored to specific healthcare systems.

Challenges in Data Standardisation and Quality

The panellists highlighted that the diversity of datasets in rare diseases can hinder analysis and interpretation. Dan O'Connor and Alison Cave explained that standardisation is vital but requires significant academic expertise and manual processing to ensure reliable results. Automated data transmission and standardised models could alleviate some of these issues, yet until these solutions are widely implemented, collaboration across sectors remains crucial.

Panellists noted the importance of high and consistent capture rates for patient-relevant endpoints, particularly those related to mental health and quality of life. Rare disease patients are experts in their conditions, but clinical trials often overlook these patient-focused outcomes. Incorporating frameworks to measure and consistently capture these endpoints could significantly improve the quality of RWD in rare diseases.

Importance of Patient-Centric Outcomes

Panellists underscored the need for trials to consider patient-centric outcomes, as these often provide a more accurate measure of treatment success. Functional and mental health endpoints are particularly relevant, but they are frequently overlooked or inconsistently collected. To align clinical trials with patient needs, early patient engagement is essential to identify and measure these outcomes consistently across studies. The panel advocated for designing trials around a clear rationale for collecting mental health and quality-of-life data, as well as reliable methods for capturing these insights.

Advancements in Methodology and Data Linkage

Emerging methodologies are advancing the field of RWD, supporting the development of datasets with linked economic and patient-reported outcomes. Collaborations between academia and industry are accelerating these developments, particularly in defining and validating new endpoints. Data linkage efforts, such as those in the UK, Nordic countries, and Catalonia, offer more comprehensive views of patients' journeys by connecting primary and secondary care data.

The panel discussed innovations in genomics and early diagnosis. Advances such as the NHS baby screening study suggest a shift towards pre-symptomatic interventions that could transform clinical pathways. These methods allow for a better understanding of disease progression and can lead to earlier and potentially more effective treatments.

Building Sustainable Infrastructure and Logistics for RWD

The panellists endorsed a “collect once, use often” approach to reduce the data burden on healthcare providers by consolidating data collection into single registries, improving efficiency. Centres of excellence were highlighted as essential for integrating specialist expertise and patient access.

They also noted the need for greater funding and integration of healthcare and social care data to provide a fuller view of patient needs, despite logistical and financial hurdles.

HERE ARE OUR 5 TAKEAWAYS

1. **Standardising RWD is essential yet challenging:** Rare disease data requires collaboration and, for now, significant manual processing until automated solutions mature
2. **Early patient engagement is critical: i**dentifying relevant outcomes upfront helps align trials with patient needs, particularly for mental health and quality-of-life measures
3. **Genomics and data linkages enable early diagnosis: i**nnovations in genomics and RWD support pre-symptomatic intervention, especially for ultra-rare diseases
4. **Centralised data collection reduces burden: c**onsolidating data efforts streamlines resources and reduces the load on healthcare providers
5. **Cross-sector collaboration is key: a** sustainable, patient-centred data infrastructure depends on cooperation among academia, industry, and patient organisations

About Remap

Remap Consulting is a specialist pharmaceutical pricing, reimbursement and market access consultancy offering an integrated, evidence-based approach to optimising price and patient access for our client's products.

We work with a broad range of clients, from top 10 pharma through to small start-up organisations on a diverse range of business-critical projects, market access training and product launches.

Our mission is to help solve your pricing & market access challenges to enable improved patient access for your products.

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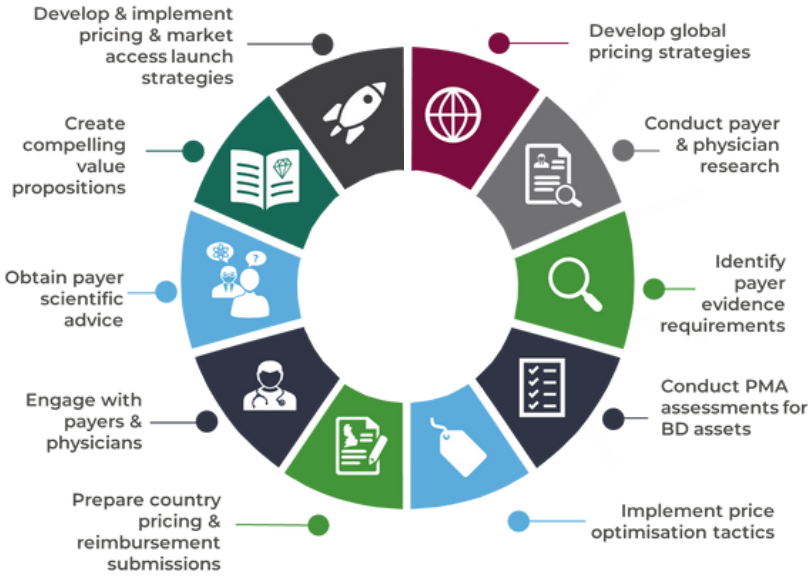

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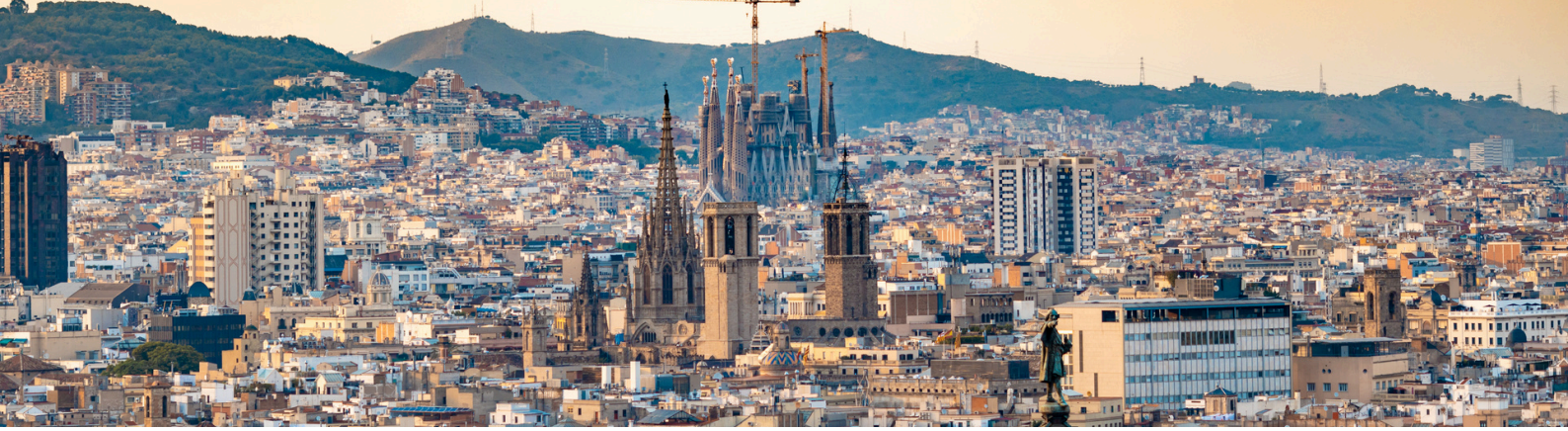

We always welcome your thoughts and opinions on the topics raised at WODC.

If you'd like to share anything from your World Orphan Drug Congress experience or hear how we can support you in getting your product to market, email our leadership team today at contact@remapconsulting.com or reach out personally by clicking their email below.

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