Accelerating Market Access in Rare Diseases

Leveraging Patient-Centric RWE for Orphan Drugs and Advanced Therapy Medicinal Products

David Schwicker

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Leveraging Patient-Centric RWE for Orphan Drugs and Advanced Therapy Medicinal Products

Learn

- Why market access challenges for OMPs and ATMPs, including autologous and allogeneic gene therapies, tissue engineered products and somatic cell therapies, are different from other drugs.
- How to collaborate with patients and caregivers in rare conditions to generate patient-relevant outcomes.
- Leveraging applications of RWE throughout the OMP and ATMP value chain.
- To mitigate payer uncertainty by addressing the challenge of early market access with accelerated approvals and fewer clinical data.
- How to develop a next-generation market access strategy with a full-spectrum lifecycle evidence generation plan that maximises product value from launch to loss of exclusivity.
- To differentiate and succeed in the increasingly competitive rare disease space with a “fast to patient” approach.

The Expert

David Schwicker

- Founder of ORPHA Strategy Consulting, David is a sought-after expert for accelerating time to launch and patient access for OMPs and ATMPs.
- David's experience includes rare oncology and haematology diseases (ALL, AML, MM, CD30+ HL/NHL, CTCL, ALK+/EGFR+ NSCLC, breast cancer), metabolic and endocrine diseases (MPS, Graves' disease, acromegaly), neurology diseases (CIDP, RTT, MG, narcolepsy, Friedrich's ataxia), inherited retinal dystrophies (retinitis pigmentosa, LCA), USHER syndrome, hepatic conditions (PSC), and rare immunological diseases (AMP, GvHD).
- Strategic Advisor to Ziphius Therapeutics, a startup biopharmaceutical company, with a focus on the planning of clinical and value evidence generation.
- Author and co-author of numerous peer-reviewed publications, David is a regular speaker at international meetings (ISPOR, RAPS EU Congress, Orphan Drugs Development and Commercialisation).

Dates & Locations

19 November 2019, Brussels
2 April 2020, Brussels
8 October 2020, London

Visit www.celforpharma.com for registration fees and updates.

Additional Benefits

- You will receive an up-to-date collection of demonstrator cases and literature focused on rare patient engagement, PROs, patient-centric RWE applications in rare conditions, value demonstration and market access.
- You will be able to share your unique OMP- or ATMP-related challenges and to discuss practical solutions with the expert and delegates.

Why You Should Attend

The fundamental challenge for the successful market access and commercialisation of OMPs and ATMPs is that the evidence generated for regulatory approval cannot satisfy the requirements of HTA bodies, pricing & reimbursement, and contracting negotiations with payers. Approval does not guarantee a premium price nor commercial success. In this course you will learn to address the challenge of early market access based on fewer and less mature data, with the creation of an Early Value Proposition and a Patient-Centric Evidence Generation Plan that both comprehensively support value demonstration, product positioning, and that both meet the requirements of all healthcare stakeholders (regulators, patients & caregivers, physicians & KOLs, HTA bodies and payers).
10:00 Welcome and Audience Expectations

10:15 Opportunities and Challenges in OMPs and ATMPs
- Defining rare and ultra-rare conditions
  - Industry outlook to 2024: Enormous potential, but commercial challenges too
- Why the OMP & ATMP regulatory approval package is often insufficient for market access
- Orphan legislation and incentives for developers of OMPs and ATMPs in the US and EU
- Key to OMPs and ATMPs: Fast track, breakthrough therapy, PRIME and accelerated approvals
- Price premiums – is rarity valued by payers & the public?
- De-risking and increasing ROI: The economics of rare disease R&D
- Pricing and reimbursement challenges for OMPs and ATMPs – The reasons QALYs often don't work in rare conditions, and why payers dislike uncertainty
- Million-dollar cures and the affordability debate: Hyper-personalized, hyper-accurate, hyper-expensive – who will have access?

11:00 Empowerment & Involvement of Rare Disease Patients
- Transforming orphan drug development and evidence generation through patient-led research
- Patient engagement objectives and ROI: Identifying populations, faster and smaller clinical trials, incorporating patient-relevant endpoints, accelerated patient & market access
- Collaborating with rare disease patients, caregivers and patient advocacy organisations: Principles and guidelines
- How patients & caregivers influence regulatory and HTA decision-making
- Patient Reported Outcomes (PROs) in labelling: An underutilised high-risk, high-reward differentiation opportunity
- PRO strategy: Selection, research, validation and regulatory/HTA qualification of tools

11:45 Coffee Break

12:00 Leveraging RWE Throughout the Value Chain
- Key RWE objectives and applications throughout the OMP and ATMP lifecycle
- How RWE can accelerate regulatory and HTA decision-making (FDA, EMA, HTA bodies)
- Using the full evidence spectrum: The RCT to RWE continuum
- Biomarker & surrogate endpoint-based strategies – Increased speed, decreased risk
- Non-traditional and hybrid Real World Data (RWD) – Advances driven by digital health solutions
- Regulatory & HTA-grade RWD quality criteria (FDA and Europe)
- Strategic partnerships with commercial and academic RWD curators, RWE/RWD initiatives and sources, The European Rare Disease Reference Networks (ERN) and Orpha.net

13:00 Lunch

14:00 Learning from High-Impact Demonstrator Cases
- The current “state of play” of RWE application by biopharma, regulators, HTA bodies and payers
- Discussion of practical solutions for challenges based on real world case examples, such as:
  - Understanding the rare condition: Natural history, epidemiology, patient identification, subpopulations, burden of disease, unmet medical needs
  - Biomarker and Genotype-Phenotype identification, validation of novel endpoints
  - Primary regulatory approval based on nonrandomised studies with historical controls
  - RWE-based label extensions (lifecycle management and iterative development)
  - Post-authorisation studies: Growing conditional marketing authorisation
  - Leveraging RWD & RWE from expanded access and compassionate use
  - Pragmatic clinical trials
  - Value demonstration and market access in rare and ultra-rare conditions

15:00 Early Value Proposition and Evidence Generation Planning
- Creating an evolving Early Value Proposition (EVP) as a complement to the labelling concepts and the Target Product Profile
- Integrating the EVP into the asset strategy, strategic imperatives and critical success factors
- Core value drivers: Early value demonstration with limited clinical data based on accelerated regulatory approvals – Addressing few data and uncertainty
- Developing a full-spectrum lifecycle evidence generation strategy plan to employ the complete toolbox from RCTs over PROs to patient-centric RWE
- Early dialogues: Seeking scientific advice in parallel EMA/HTA and single HTA consultations

16:00 Coffee Break

16:15 Next-Generation Market Access Strategies for OMPs and ATMPs
- Differentiated value communication: Involving stakeholders and improving education to prepare the community and accelerate patient access
- Value assessment trends – holistic approaches: Mechanism of Coordinated Access (MoCA), ORPH-VAL, the new AIFA Innovation Algorithm
- Why compassionate use (US: expanded access) is ideally suited for generating an early market presence & revenue and enabling rapid commercial uptake
- Managed Entry Agreements (MEAs): Risk sharing & pay-for-performance contracts, value- and indication-based pricing, the “Netflix” model – Current challenges and limitations
- Orphan-specific launch, commercialisation and product differentiation strategies for increasingly crowded rare disease markets

17:00 Key Takeaways & Final Q&A

17:30 Close

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