Susanne Michel, decision making in early market access: Plan, learn, and provide a clear pathway

Manufacturers have to make decisions early in the development cycle to submit a molecule for an early access pathway. These decision-making processes are challenged to incorporate feasibility of 'early access' execution, accounting for implementation hazards and aligning all internal stakeholders. Regulatory and HTA timelines, and moving the molecule to its next development stage, allow only for a short window of time to make well-planned and informed decisions.

Many of the early access pathways have set out clear criteria under which molecules qualify for a scheme, including demonstrating medical unmet need, orphan designation, or no other treatment options available or sufficient to meet treatment goals.

Multiple functions across the organisation are working to deliver value, through meaningful innovation for patients and healthcare systems around the world. In early market access decision-making, these functions (e.g. R&D, clinical, regulatory, marketing, and business development) must work simultaneously. They need to be aligned on the value, challenges, and solutions. Each brings a unique viewpoint of the asset and its potential opportunities and challenges, strengths and weaknesses, and degree of differentiation. Simultaneously, the external environment into which the asset will be launched is changing; health reforms are being initiated and implemented in far less time than it takes to develop a pharmaceutical asset. As such, the environment into which a new asset is launched can be far different than the environment under which it was initially developed. In fact, dramatic changes in how innovation and value are defined, substantiated, and rewarded have taken place in key markets. This requires a risk assessment against HTA requirements.

Because innovation and value are measured along multiple dimensions, it is critical that companies institute a process of cross-functional collaboration to transparently identify key product assumptions; discuss opportunities to convey value and differentiation; and align on an early value proposition that can be substantiated to meet external requirements.

Recommendation: Establish and undertake a structured, collaborative, and transparent process to openly discuss early market and product assumptions, clinical evidence and value drivers, and a clinical and commercial strategy, to develop a compelling early value proposition aligned to internal objectives and external requirements. This mindset focuses on the critical path leading to a best possible decision outcome – establishing a decision framework against which a molecule in early stage can be assessed and into which each team can feed its assumptions, knowledge, and perception.

Critical Parameters of an Early Assessment Decision

LEVEL 1: Indication and therapeutic relevance

→ Anticipated level of innovation – improvement to Standard of Care (SoC)
→ Prevalence – orphan, ultra-orphan
→ Anticipated care setting
→ Nature of therapeutic area
- Indication has high HTA challenges
- Public health relevance/disease awareness
- Public health priority
- New to the manufacturer

LEVEL 2: Data availability (Variable over time)

→ Degree of evidence uncertainty
- Patient population
- Trial length, comparator, endpoints
- HE and models
- Humanistic instruments
- Value of PROs and RWE

LEVEL 3: Competition/disease area landscape (Variable over time)

→ Treatment paradigm changing
→ Crowded therapeutic areas with varying guidelines across geographies
→ Disease management not standardised
→ New indication – no existing "licensed" therapy

This approach places a critical focus on allowing a cross-functional team to assess the opportunities, identify the risks, and build awareness of the efforts needed in submitting a molecule for early access, addressing critical decision domains. **Step 1: Identify desired results - Step 2: Determine acceptable and feasible evidence - Step 3: Plan a learning experience and provide clear instructions for decision making.** The approach is structured to cultivate a joined culture across disciplines for incorporating new information and adapting plans as necessary to optimise product potential and return on investment and develop a focused P&R strategy. P

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Feasibility of execution and implementation hazards (across all levels)

Efforts of preparation/early access process
- Time needed to prepare
- Level of information sharing/ manufacturers' participation
- Consultation (early scientific advice)
- Costs (manpower time, fees, etc)
- Track record of products achieving positive value assessment/price

Level of collaboration or guidance from EMA/HTA (HE, clinical, RW evidence)

Output and interaction
- Reports from assessments
- Consensus across partners
- Participation of other stakeholders