

## How can orphan drug manufacturers combine pricing and market access strategies with health economics to optimize pricing and market access?

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The number of new compounds in research and development (R&D) with an orphan drug (OD) designation has steadily increased over the past decade. In 2000, only 12 compounds in R&D had received an OD designation by the EMA. But by 2009, that number had increased to 105. Overall, during that decade, 615 OD designations had been granted; yet, fewer than 10% of these agents received an authorization over the same decade.

Success in bringing any drug to market is fraught with numerous challenges. However, regulators have tried to facilitate this process for ODs. For example, unlike other drugs, ODs are always authorized via the centralized European procedure. Moreover, due to the small number of patients with, and the severe or life-threatening nature of most orphan diseases, EMA authorization can often be filed immediately after conducting phase II clinical trials rather than waiting for phase III results. However, one hurdle that has not been streamlined for OD is many countries' requirement to demonstrate cost effectiveness for reimbursement.

Even in European countries where cost-effectiveness is not (yet) mandatory, the available cost-effectiveness evidence can help with pricing negotiations. Ironically, since many OD authorizations are based on limited data (i.e., small sample sizes for clinical trials and phase II study design), the body of evidence available to develop cost-effectiveness analyses at the time of market access is often incomplete. In addition, cost-effectiveness guidelines vary per country, resulting in the need to conduct analyses in each individual country for which market access is sought.

Besides clinical and health economic research, companies can conduct several types of strategic research that can help optimize the OD reimbursement process. For example:

- Landscape analyses
  - Identify the epidemiology and available drugs for the corresponding indication, their prices, safety and efficacy profiles, and reimbursement status.
- Value messaging
  - Interview key opinion leaders (e.g., clinicians) to test the target product profile (TPP) and to identify most important value messages and unmet medical needs.

- Payer research
  - Conduct interviews with local, regional, and national payers to
    - Assess their perceptions regarding the TPPs (including price points) and value drivers;
    - Identify requirements for OD market access and reimbursement;
    - Select comparators, data to be delivered, analyses to be conducted, etc.
  - Initiate dialogue between regulatory and payer communities (data requirements, desired outcomes, etc.), in order to align payer expectations with regulatory data.

For ODs, the timing of conducting this strategic research is critical given the relatively short authorization time-frame. The same is true for health economic assessment. Hence, to optimize drug pricing and market access, strategic and health economic research should be conducted in tandem as early as possible.

Below some examples are presented where strategic research could benefit from health economic information:

- Optimize the output of strategic research by including early health economic information in TPPs tested during payer research
- Quantify the identified value drivers in monetary terms
- Test at an early stage of development the cost-effectiveness of the drug based on numerous pricing scenarios and TPPs.

Another type of research increasingly conducted by pharmaceutical companies during drug development is trial simulation, which helps to minimize risks and guide decision-making by formalizing assumptions and quantifying uncertainties about the drug and upcoming trials. Trial simulations are especially valuable for ODs as there is often very limited data available due to the limited number of patients and comparisons mostly with placebo. This information could be used for registration but potentially also when designing subsequent trials, which might be a sine qua non condition to obtain preliminary approval and data necessary for reimbursement dossiers and pharmacoeconomic analyses. For instance, by imputing early clinical results in a health economic model, the optimal dose and best patient subgroup can also be identified to ensure the new drug's cost-effectiveness.

With its international reach and multidisciplinary staff, including experts in OD regulations, payer research, market access and health economics, and trial simulation, Pharmerit's mission—and passion—is to help manufacturers seamlessly integrate pricing/reimbursement and pharmacoeconomic evidence and strategies to ensure that patients have access to needed treatments.

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