# Orphan drugs: rarity no guarantee of access

**Today, orphan drugs enjoy good market access in the US and EU. But, warns IMS Consulting USA Principal Rob Glik, given the large number of recently launched and pipeline orphan drugs, access may grow more difficult – especially in the EU.**

## USA

The Orphan Drug Act of 1983 provides financial incentives to pharmaceutical companies that develop treatments for rare diseases or conditions. An orphan drug is defined as a product treating a patient population of fewer than 200,000 or treating more than 200,000 but for which a company could have no reasonable expectation that it would recover the costs of R&D through sales. An ultra-orphan drug typically treats fewer than 10,000 patients.

If a product is granted orphan status, the Orphan Drug Act provides three primary incentives for its development

- federal funding of grants and contracts for clinical trials
- a tax credit of 50% of clinical testing costs
- exclusive marketing rights for seven years from the date of FDA marketing approval (thereby making step edits very difficult until this exclusivity ends).

A total of 1,129 different orphan drug designations have been granted by the Office of Orphan Products Development (OOPD) since the inception of the Orphan Drug Act and 249 orphan drugs have received marketing authorisation. In contrast, the decade prior to 1983 saw fewer than ten such products come to market.

Today in the US, payers do not have a separate reimbursement process for orphan drugs but often solicit input from key opinion leaders (KOLs) before determining appropriate prior authorisations. KOLs are usually favourable towards any new orphan therapy since it enables them to treat previously untreatable patients, and payers traditionally put no major restrictions on these drugs. For example, managed care organisations and pharmacy benefit managers typically do not limit patient population reimbursement, other than by enforcing reimbursement for the labelled indication. For products on the medical side, MCOs have historically covered all infused orphan therapies - even the most expensive ones.

As with other non-orphan drugs, however, many payers are now passing more of the cost burden to patients by increasing the co-insurance or by moving products to the pharmacy side where the patient shares more of the cost burden. Payers are also starting to manage expensive orphan drugs in the pharmacy channel, such as by placing them on fourth tiers with high co-insurance, thereby limiting access.

Given the current favourable access environment for orphan drugs, and the large number of new, 'expensive' orphan drugs recently launched (at least 15 new orphan drugs have been launched in the past 24 months, including six priced at over $100,000 per year), US payers will inevitably consider becoming more restrictive. The current payer system, however, is limiting payers' ability to contain costs. Given payers' limited knowledge of rare diseases, KOLs who are reluctant to limit access are usually consulted. Additionally, limited options for orphan diseases, as well as political sensitivity in this area, further reduces payers' ability to be more restrictive with orphan drugs.

## Europe

Although a fully functioning system for orphan drugs has been initiated in the EU via the Committee on Orphan Medicinal Products of the EMEA, each country makes its own decisions on the pricing and reimbursement of orphan drugs. If payers think the budget impact will be too high, they may restrict the reimbursement of new orphan drugs to sub-populations of patients or, more frequently, force patients to go to specialist centres:

France: Patients diagnosed with rare diseases must attend a 'Centre of Reference'. Specialists at these centres confirm diagnosis before the patient can receive a high priced orphan drug.

Italy: 'Rare Disease Centres' are the only official centres allowed to prescribe orphan drugs. These centres collect efficacy and safety data relating to products used; they also enable the tracking of any indication restrictions placed on these products.

UK: Access is often controlled by centres designated by the National Specialist Commissioning Advisory Group. Control is usually designated only for ultra-orphan drugs, such as those used to treat lysosomal storage disorders (e.g. Gaucher's disease, Fabry's disease, mucopolysaccharidosis). Only the six nationally designated centres are funded to prescribe these treatments.

Although the cost of orphan drugs is not a major concern today (they make up one percent or less of most nations' pharmaceutical budgets), the number and cost of such products is increasing. Of 62 drug approval applications submitted to the EMEA last year, 13 were for orphan drugs. In the U.S., 61 products received orphan designation in the past 12 months, including 11 new marketing approvals. In addition, six orphan drugs launched in the past 24 months cost more than $100,000 annually.

**Implications**

Given the increasing scrutiny of market access for orphan drugs, improved clinical data will be more critical moving forward. In the EU, payers are already more critical of clinical data for orphan drugs, so the quality of the data will become even more important. For example, payers will want to see real endpoints rather than surrogate endpoints.

The use of health economics data in the EU will also become more critical in the future. More EU payers will scrutinise budget impact, comparing the economic benefits of a new orphan drug to alternative therapies or to the disease burden, potentially creating a less favourable market access environment for future orphan launches.

The labelled indication is critical to access. In the US, most payers will not cover off-label use, but do not restrict the labelled indication either. Ensuring that the indication is broad enough to maximise the patient population while also restricting usage to severe patients in order to justify price, will become increasingly important.

Given the growing importance of proving the value of orphan drugs, it will be critical for companies to consider various factors in launching orphan drugs, including, but not limited to:

- Cost of alternative therapies
- Gain in life expectancy/survival data
- Patient yearly out-of-pocket costs
- Budget impact and potential payer behaviour
- Patient life-time maximum coverage in the US

Corporate and personal responsibility for patient access also needs to be considered, such as having a comprehensive patient assistance programme and working with patient assistance groups to ensure access for those with limited financial means.

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