ARE EUROPEAN INCENTIVES SPEEDING ACCESS IN RARE CANCER TREATMENTS?

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To help orphan drugs reach patients, the EMA provides drug developers with R&D, regulatory and financial help. Himakshi Sharma examines the impact of this approach.

In Europe, heterogeneity in health technology assessment (HTA) across European Union (EU) member states means there is variability in the availability of, and access to, drugs. Improving access to orphan drugs – particularly those for rare cancers – is a crucial decision and one which HTA attempts to simplify.[1]

Orphan medicinal products

The EU regulation on Orphan Medicinal Products (OMP) confers orphan designation to products for conditions that affect not more than five in 10,000 people in the EU and which are unlikely to be marketed without incentives.[2] The European Medicines Agency (EMA) received 2,302 such applications between the introduction of the regulation in 2000 and September 2015, of which its Committee for Orphan Medicinal Products (COMP) approved 1,544. About a third of these were for some form of rare cancer.[3]

The EMA provides incentives to sponsors seeking marketing authorization (MA) for products designated as orphan drugs, as depicted in Figure 1.[4]

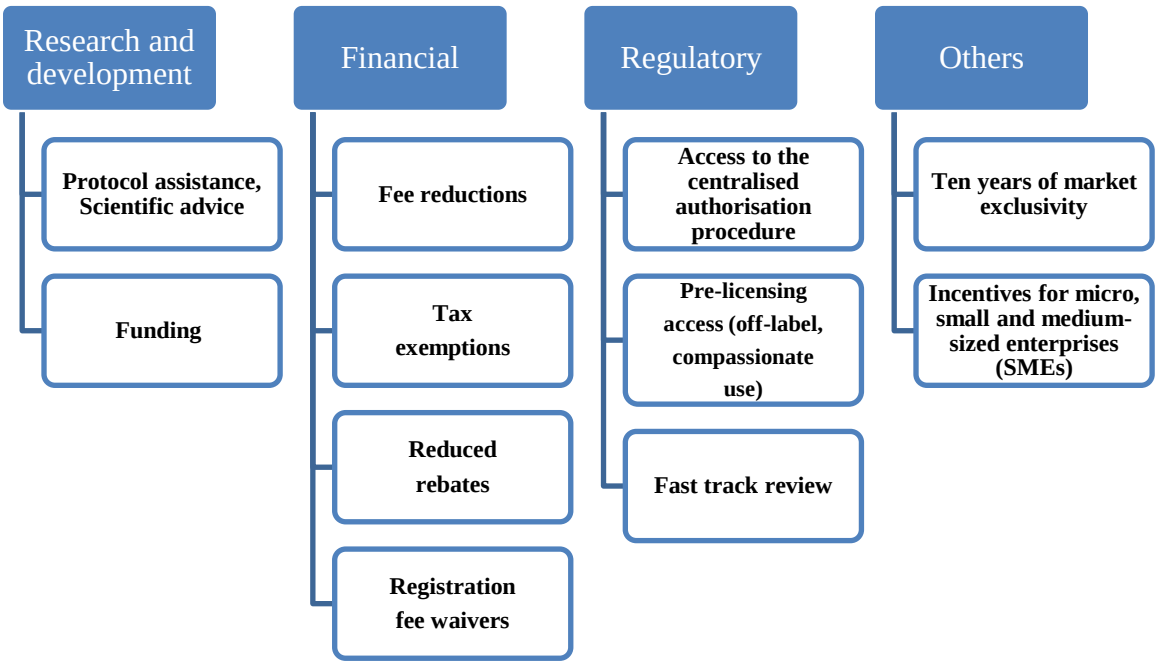

Figure 1: Types of incentive provided by the EMA[4]

Recent success stories

There are many examples where incentives have helped improve access to drugs for rare cancers in Europe.

A recent one is the addition of 'follicular lymphoma' to Gazyvaro's (obinutuzumab) approved indications. It was first granted MA in 2014 for previously untreated chronic lymphocytic leukemia (in combination with chlorambucil). Now the product will be available in combination with bendamustine for patients with follicular lymphoma who were previously treated with chemotherapy. Gazyvaro was designated an orphan medicine in 2015. The sponsors received scientific advice from the Committee for Medicinal Products for Human Use (CHMP) on the design of the phase 3 trial supporting the efficacy and safety of the product, which facilitated MA.[5]

Many drugs have been approved recently for their 'orphan designation' to address unmet cancer treatment needs, including Debiopharm International SA's IAP inhibitor Debio 1143 (for ovarian cancer), SELLAS Life Sciences Group's WT1 cancer vaccine (galinpepimut-S) (for acute myeloid leukemia and malignant pleural mesothelioma). This makes them eligible for financial incentives such as fee waivers and tax rebates, as well as other incentives such as 10-year period of marketing exclusivity in the EU after product approval.[6,7]

Other improvements in access for cancer drugs are the centralized authorization procedures, fast-track reviews and pre-licensing access programs, such as 'compassionate use'. Nivolumab was approved on compassionate grounds in Ireland recently, benefiting 200 patients with advanced lung cancer for 30 days, free of charge to the patients.[8]

Similar programs are being set up in other countries in the EU (e.g. Austria, Estonia, Greece, Hungary, Italy, Lithuania, Portugal, and Spain).[9] They have adopted various policies for compassionate use, including named patient programs for individual patients and complete reimbursement of the medicinal product under compassionate use (such as in Greece).[3]

Are incentives enough?

Incentives have radically improved MA for orphan drugs for rare cancers, but are they enough?

One major concern after receiving MA is pricing and reimbursement as cost-effectiveness and the decisions on coverage and reimbursements are addressed at national level.[10]

A recent controversy concerning this was the rejection of Imbruvica (ibrutinib) by the National Institute for Health and Care Excellence (NICE) in England. The drug had acquired the CHMP's favorable opinion on orphan designation in 2012 for the treatment of chronic lymphocytic leukemia. This was contradictory to the decision by other EU countries, such as Greece, which accepted the drug under the national reimbursement scheme.[11,12]

Jakavi (ruxolitinib) received orphan drug designation in 2008 and 2009. However, it was not covered under reimbursement policies in East European countries such as Poland and the Czech Republic until 2015.[13,14]

Conclusion

So, incentives can help increase access to orphan drugs for rare cancers across the EU. However, improvements to MA are hindered by the unpredictability of pricing and reimbursement decisions at country level. The various stakeholders need to take a more proactive approach to close the gap between regulatory approval and reimbursement decisions. They can benefit from adopting adaptive licensing, risk-sharing agreements, conditional coverage and patient registries that can provide data on local resource utilization, safety, effectiveness, and costs.

Thus the authorities are better equipped to forecast and manage already stressed budgets. Physicians can use the data to continuously monitor disease progression, compare treatment in the real-world setting and make informed decisions on sub-populations and concomitant medications. Patients are assured access to novel and otherwise unaffordable medications through a defined process, and pharma companies have a continuous flow of data that could support submissions for complete reimbursement in the future.

http://pharmaphorum.com/views-and-analysis/european-incentives-speeding-access-rare-cancer-treatments/

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