Early access pathways

ACHIEVING EARLY ACCESS IN ONCOLOGY WITH NON-COMPARATIVE DATA

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Introduction

While oncology drugs can gain regulatory approval based on immature or early clinical data, the decision-making for payers is not as straightforward, with more mature data sets to inform their decisions. Early access pathways enable patients to receive novel drugs that serve a high unmet need while HTA and reimbursement authorities assessments are being performed.

Market Access Transformation recently met with HTA and payer experts from France, Italy and the UK to discuss early access in oncology based on non-comparative evidence and the associated challenges and opportunities for drug developers.

In the final chapter of this 3-part series, we look at early access pathways in France, Italy and the UK along with the benefits that these can provide.

THERE ARE MECHANISMS IN PLACE TO ENSURE PATIENTS GET EARLY ACCESS TO PROMISING DRUGS

Most countries have specific pathways in place to ensure that certain patients can gain early access to novel drugs, particularly where there is a high unmet need. In Italy for example, there are CUP (CNN) that allow commercialization prior to receiving a classification from AIFA along with other special pathways that enable early access to orphan drugs. One such pathway is the so-called "648 list" which is used in exceptional circumstances to permit the supply and reimbursement of products that do not have a marketing authorization – where they have obtained a marketing authorization in other countries but not in Italy, or is undergoing clinical research but it is not yet authorized or where the product's use is for an indication other than that authorized in Italy. A former Italian national and regional payer, explains "648 is a program where the price is decided by the manufacturer…the drivers uptake are unmet needs, lack of alternatives, a relatively small population, small budget impact."

Similar programmes exist in France, where patients can be given early access to new drugs through Temporary Use Authorizations (ATU), which allow access to new promising drugs that are not yet approved in France and which address a high unmet need It is seen as very beneficial for patients, as our French HTA expert, (and ex member of the Transparency Commission in France) explains, "The benefit is obviously to give patients access to treatment options. [It's been] expanded to include use of a product as a last resort assuming there is sufficient evidence to prescribe before market approval. And after [approval] there's a continuous push to use the scheme so that patients can still benefit from access."

There are some drawbacks, as the French HTA expert continues "The company has to pay a "clawback" on the net price when they finalize the price negotiation. But [it is a risk as] the committee may determine the net price to be $0 and you cannot achieve market approval – so what would the clawback in that case?" A new out-comes-based approach is currently being used in France, where some patients are able to get early access to a new drug, with data generated post-MA to inform HTA and reimbursement discussions, "We do have a new innovative scheme, which is for example for gene therapies, where you can ask for reimbursement, collect data and later on get HTA approval and then final price negotiation."

There are also a range of different early access schemes utilized in the UK, such as compassionate use programs, early access to medicines schemes, access via the Cancer Drugs Fund, used alongside rapid NICE assessment. Clinical lead for the Cancer Drugs Fund in England and former member and chair of the NICE technology appraisal committee comments, "In England, prior to marketing authorization, there are various types of compassionate access scheme, through which the drug or the technology costs nothing. If NICE is appraising every cancer drug indication and starts its appraisal process before CHMP opinion and before the drug gets licensed, the hope is that as soon as the drug is licensed, NICE can issue its first view as to whether a drug is clinically and cost effective. Should access be granted in England it will normally come, if it comes at all, very early after the EMA has issued a license. That's highly prized for patients and for pharmaceutical companies."

EARLY ACCESS PROGRAMS PROVIDE DATA TO SUPPORT THE CLINICAL EVIDENCE

In England, the Cancer Drugs Fund is a critical mechanism to ensure early access to novel oncology drugs. It allows for drugs that do not have sufficient evidence to be positively appraised by NICE and used in a limited capacity for a period of time, meaning that patients obtain early access and real world data can be collected to bolster the evidence package. As the UK HTA expert explains "In England we have a Systemic Anti-Cancer Therapy (SACT) data set. All hospitals have to submit outcome data for their chemotherapy patients…we can collect treatment duration, and survival. At the time that the drug goes back to NICE for reappraisal, it supplies the appraisal process with 100% accurate data on that treatment duration and survival. We may be talking about relatively small drugs. But even for the CAR-Ts, there will be hundreds of patients in the real world evidence base when they come back to NICE for reappraisal. Technology appraisal committees are, of course, very interested in looking at both the clinical trial data and the real-world evidence and examining any differences.", which sometimes can work both ways." Indeed, there are examples where the drug has been able to move from the CDF to regular commissioning thanks to the real-world data gathered through early access usage. As the UK expert highlights, "Brentuximab in relapsed Hodgkin's disease was a drug that was referred into the Cancer Drugs Fund, largely because of uncertainties regarding the stem cell transplant rate. The real-world evidence from England showed that the rate was significantly higher than that reported in historic comparative populations…and therefore allowed Brentuximab to swiftly move into routine commissioning."

In Italy, all use of oncology drugs must be recorded in registries, meaning that real world data is readily available for authorities and manufacturers alike. These data are increasingly being considered by decision makers, as the Italian HTA expert comments "Where the product has been already approved for other indications, real world evidence is used for safety. Theoretically it is possible to include real world evidence in the dossier. It depends on the robustness of the real-world evidence."

In contrast, the French ATU system is not designed to collect real world data and it is down to the manufacturer to ensure that this evidence is collected and analysed appropriately if it is to be used for decision-making, as explained by our French HTA expert "ATU is not designed to capture hard evidence in France, it is purely a regulatory channel to address unmet need while waiting for full approval. It could be a good tool for the company to highlight unmet need because if you have a large proportion of patients in the ATU compared to the potential population… then that's a good signal."

EARLY ACCESS INITIATIVES WILL CONTINUE TO BE USED FOR PROMISING NOVEL DRUGS

A strong foundation has been laid to ensure that patients can gain early access to promising oncology drugs that show benefit even in the absence of robust, complete clinical data. It is expected that this will continue to be built on, as our UK HTA expert comments "I think the Cancer Drug Fund has shown us how valuable temporary access with a temporary price for one, two, three years can be and then helping to make a final decision as to whether a drug should be routinely commissioned. This is only going to increase because genomics is going to split diseases into many different types. In the next year, I think we will see this type of access being rolled out to non-cancer drugs too. Proof of success is shown in fact that over the past the last three years it's approved 36 different indications into the CDF. Furthermore, that no manufacturer has, having been offered access to the Cancer Drug Fund has in effect turned it down, subject to the final pricing negotiations." This view is echoed by the Italian HTA expert, who expects the situation in Italy to remain positive, although the demands on evidence may increase. "I don't see significant differences in the future… an evolution would be in the amount of evidence you should provide vs. today." However, while it is expected that reimbursement authorities will continue to grant early access in specific situations, pharmaceutical companies must ensure a robust post-authorization data collection programme to ensure real world evidence can support and expand access for these promising drugs.

About Market Access Transformation (MAT)

Founded by industry veterans, MAT specializes in developing cutting edge technologies that enable the healthcare community to gather and exchange insight that assess the real-world potential of their products. MAT offers an online, information exchange platform, Rapid Payer Response™ (RPR), that is revolutionizing the way to global payer insights.

About Rapid Payer Response™ (RPR)

Rapid Payer Response™ (RPR) is an agile, on-demand platform that allows you to conduct faster, higher quality, more insightful research via direct payer engagement. Through our global payer network, it is possible to gather more accurate, robust feedback by probing further and asking deeper questions at any time, ensuring you always have the exact answers you need in a fraction of the time and cost of a traditional payer research project.

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