six assessments that resulted in conditional remuneration were targeted therapies. Typically, with these targeted therapies, PFS or OS ranged from -3 months to 9 months with the cost-per-QALY > €50,000. Five of the six manufacturers participated in a patient-access scheme which consisted of fixed-price discounts such as Cetuximab (CRC) and Erlotinib (NSCLC) or performance schemes like sumitab (GIST), Bortezomib (myeloma), and Lenalidomide (myeloma). CONCLUSIONS: Based on the retrospective analysis, it is clear that the biggest challenge for targeted-cancer therapies is affordability with only one of the targeted therapies receiving unconditional remuneration. However, nearly all the other targeted therapies evaluated that offered >3 months OS or PFS were recommended by NICE with a proviso to bring down the cost of treatment. Therefore, when companies develop their market access strategy, they should include a patient-access scheme in order to enter the UK market.

PATIENT ACCESS SCHEMES IN UK ARE DRIVEN BY HEALTH TECHNOLOGY ASSESSMENT

Aurore A, Xavier Gallois, Muriel Buttenhagen, and Mathieu Combe
University of Lyon, Lyon, France; 'Creative Ceuticals, Paris, Ile de France, France

OBJECTIVES: Achieving market access for new products has become complex for pharmaceutical companies. Faced with growing expenditure, healthcare authorities accept or propose various schemes (risk-sharing/payment for performance) commensurate with the technology. Therefore, we performed an in-depth analysis of design of PASs in the UK to revisit their typology and rationale. METHODS: We reviewed official and grey literature on the websites of the HTA Agency—NICE, the Department of Health, and the industry, and the internet. We searched for documents containing all synonyms of PAS and different scheme types. We selected PASs launched after 2006. RESULTS: We identified 13 PASs, all of which were designed/implemented in consultation with NICE. Drug’s comparative effectiveness was central to the rationale behind the design of PASs. If effectiveness was acknowledged in the HTA, PAS was based on cost-containment (rituximab, erlotinib). If it was not recognized, this was for one of the two reasons: (1) the uncertainty about the long-term effect of the drug, or (2) the value of ICER was questioned in the HTA. In case of (1), the PAS consisted in free provision of the drug by manufacturer after a predefined period (lenalidomide, ranibizumab). In the case of (2), the PAS aimed at lowering the ICER either through cost containment (sumitab, cetuximab, pemetrexed), through extending linkage to payment outcomes (bortezomib, oralzimab), or by a mix of the two (cetrotuzimab, utoxinkinam). CONCLUSIONS: Formalized Health Technology Assessment is both a prerequisite and reason for implementing Patient Access Schemes in the UK. If the comparative effectiveness of a drug is acknowledged, the agreement is based on cost containment. On the other hand, if it is questioned, the PAS may have a form of a risk-sharing scheme and may be linked to the payment outcomes (performance-based scheme).

NICE’S COST-EFFECTIVENESS THRESHOLD REVISITED: NEW EVIDENCE ON THE INFLUENCE OF COST-EFFECTIVENESS AND OTHER FACTORS ON NICE DECISIONS

Devlin N1, Dakin H2, Rice N2, Parkin D3, O’Neill P1
Paisley, UK; 1i3 Innovus, Stockholm, Sweden; 2Pfi zer Oncology, Sollentuna, Stockholm, Sweden; 3Pfi zer, Inc, New York, NY, USA; 4Pfi zer Oncology, La Jolla, CA, USA; 5Stockholm School of Economics, Stockholm, Sweden; 6Harvard School of Public Health, Boston, MA, USA; 7University of York, York, UK

BACKGROUND: The purpose of the study is to compare alternative methods for analyzing overall survival data in the presence of cross-over, thus illustrating differences between methods, and providing guidance on choice of methodology. METHODS: Two promising methods for dealing with cross-over are inverse probability of censoring weighting and the rank-preserving structural failure time model. The methods are compared with naive censoring of data at cross-over and intention-to-treat analysis ignoring cross-over using two recent examples of trials in oncology: the receptor tyrosine kinase inhibitor sunitinib in renal cell carcinoma (RCC) and in gastrointestinal stromal tumor (GIST). RESULTS: The analyses showed that for a trial with a low proportion of cross-over from placebo to active treatment (RCC), the choice of statistical method did not affect the results to a great extent; the range of relative mortality risk for active treatment versus control was narrow. With a high proportion of cross-over (GIST), the range of relative mortality risks was broader. CONCLUSIONS: Naive censoring at cross-over can lead to bias and should be avoided. If cross-over occurs frequently, the inverse probability of censoring weighting method or the rank-preserving structural failure time model are recommended depending on the characteristics of cross over in the trial, trial size, and available data.

DO PATIENT ACCESS SCHEMES RESULT IN AN ACCEPTABLE ADMINISTRATIVE BURDEN?

Hawes S, Costello S, Kurtz J, Hamer N, Brooks-Royen C
Costello Medical Consulting Ltd, Cambridge, UK

OBJECTIVES: In the UK, Patient Access Schemes (PAS) have become more common in submissions to the National Institute for Health and Clinical Excellence (NICE). The increase in PAS is a result of the essential role such schemes play in enhancing the acceptability of high-cost treatments to payers. In published appraisals, minimal emphasis has been placed upon the administrative burden of PAS, which is typically described as “acceptable.” The aim of this study was to assess the impact of administering PAS in the UK, using both primary research and existing literature to identify key administrative challenges. METHODS: A literature search of PubMed and Google Scholar. Freedom of information requests were sent to NICE for data on PAS administration. A pilot questionnaire was distributed to all 19 contacts listed on the directory of NHS Chief Pharmacists in Wales, to assess the real-life burden of PAS administration. RESULTS: Limited literature is available on the administration of PAS. However, the literature search uncovered evidence that the administrative impact of PAS is being recognized. The creation of the Patient Access Scheme Liaison Unit (PASLU) in October 2009 and the publication of the Pharmaceutical Price Regulation Scheme (PPRS) are two such developments, both of which are steps to continue effectively.