OBJECTIVES: The National List of Health Services (NLHS) in Israel has been updated annually since 1999 but results from economic evaluations (EE) were not used to support coverage decisions. We explored the potential availability of EE results to the committee responsible for updating the NLHS at the times of coverage decisions. The availability and use of these data could have altered these decisions. METHODS: We used the Tufts Medical Center Cost-Effectiveness Analysis Registry (http://www.caregistry.org) to search for relevant cost/QALY EE for all drugs and their relevant indications added to the NLHS from 1999 through 2008. For each pair of drug and cost/QALY publication we recorded the publication date, the intervention and comparator(s) considered and the incremental cost-effectiveness ratio (ICER) to determine value for money. Based on available ICERs we qualitatively classified each coverage decision into one of three categories: 1) The coverage decision can be justified on EE grounds; 2) The coverage decision cannot be justified on EE grounds; 3) The evidence from EE is mixed and we could not determine whether the coverage decision can be justified or not. RESULTS: Relevant cost/QALY analyses were found for 181 (40%) of 451 drugs included in the updates of the NLHS during which only 71 (16%) of drugs had relevant EE prior to the coverage decision. Based on the evidence gathered from EE prior to and following the coverage decision, we suggest that decisions were correct in 56% of the cases, incorrect in 17% and ambiguous in 27% CONCLUSIONS: The use of EE to support coverage decisions could have altered coverage decisions in a sizeable proportion of drugs added to the NLHS in Israel. Avoiding the use of results from EE to support public funding of drugs may lead to a non-optimal use of scarce healthcare resources.

ORPHAN DRUGS FACE TOUGHER SCRUTINY IN SECURING FAVORABLE PRICING AND MARKET ACCESS

Grovenor A, Sara S, Jones K

OBJECTIVES: Orphan drug (OD) legislation has been highly successful in incentivising pharmaceutical companies to invest in developing medicines for previously-ignored rare diseases. Since 2005, a third of all new drug approvals in the US have been ODs, and worldwide the market is forecast to grow at a CAGR of 6%, reaching $112 billion by 2016. Although these products only account for 2-3% of total drug budgets in the US and EU, their burgeoning number is placing increased pressure on funding. This paper aims to explore how payers in the US and EU are responding to these financial demands. METHODS: We reviewed all OD approvals in the US and EU since 1st January 2000 and, for each, calculated the average cost per patient per year, both in the pre- and post-launch periods. We also reviewed published payer assessments of these products, and all government proposals regarding OD policies since 2005. RESULTS: Since 2007, there has been an obvious drop in prices secured for novel ODs. In the US alone, the average cost per patient per year for products approved in 2008 to 2010 ($43,896) is 73% lower than for those approved in 2000 to 2007 ($129,228). Furthermore, our analysis suggests payers are adopting a more discerning approach to the way they evaluate orphan drugs, especially those perceived to be exploiting the original intent of the legislation. For example, Germany’s recent healthcare reforms highlighted plans to target ODs and other high cost therapies, acknowledging the need for a more selective approach. In the US, a number of states have reduced or eliminated pricing flexibilities to make certain that prices are justified. CONCLUSIONS: In order to lay a policy foundation for international OD policy assessment, we need to understand the evidence base supporting current decisions and identify gaps in the evidence base and potential future opportunities in evaluating ODs.

WIDER CONSULTATION IN HEALTH TECHNOLOGY ASSESSMENT (HTA) DECISIONS: BETTER UNDERSTANDING OR A LOBBYING OPPORTUNITY?

Bending MW1, Hutton J2, McGrath C2

OBJECTIVES: HTA agencies worldwide have varying processes that allow consultation with stakeholders during decision-making. The objective of this study is to determine the impact of the National Institute for Health and Clinical Excellence (NICE) Single Technology Appraisal (STA) consultation stage on reimbursement decisions of pharmaceuticals. METHODS: Documentation was accessed from the NICE website for all STA’s conducted between 2006 and August 2010. Details of the first Appraisal Consultation Document (ACD) draft decision, subsequent ACDs, Final Appraisal Determination (FAD) and final guidance decision were extracted. The decisions were categorised with respect to the licensed indication (recommended, restricted, not recommended, only in research). Details of the further analysis and evidence submitted by the manufacturer as a result of these decisions were extracted. These data were analysed for the different stages of decision-making. RESULTS: The website search identified 55 NICE appraisals of which over fifty percent were for cancer medicines. Final decisions (draft first provisionally recommended 36% (13%) restricted, 36% (20%) restricted, 16% (56%) not recommended decision and 11% (51%) terminated decision. One appraisal contained only in research recommendations in addition for use in routine practice. An ACD was produced in 42 appraisals, followed by the manufacturer providing further economic analysis in 26 appraisals, a patient access scheme in 5 appraisals and new clinical evidence in 2 appraisals. Types of further economic analysis provided by the manufacturer were for other treatments/strategies, different modelling assumptions; alternative survival distributions; further sensitivity analysis; and other. CONCLUSIONS: NICE’s iterative consultation process allows consideration of evidence and wide consultation with stakeholders. This results in evidence that is more appropriate for the evaluation of pharmaceutical’s and partly explains the higher recommendation rate when compared with similar international reimbursement agencies. There is a need for further research to understand the impact of the different processes employed across countries’ decision-making.

WHAT IS THE IMPACT OF COMPARATIVE EFFECTIVENESS AND VALUE BASED PRICING ON A PRODUCT’S VALUE AND MARKET ACCESS?

Walker R, Ng-Haing J, Koruth R, Sparrowhawk K

OBJECTIVES: Comparative effectiveness (US) and value-based pricing (VPB) (UK) are anticipated to bring changes to a ‘free-pricing’ system for drugs to one where prices are influenced by governmental authorities. A product’s value will take into account additional factors, such as wider societal benefits and therapeutic innovation. It is unclear if and how the impact of comparative effectiveness and VPB on the market access for new drugs in depression. METHODS: A literature review was conducted using electronic databases (Medline, Embase, Google Scholar). The search was performed for the years 2009-2011 and key terms included comparative effectiveness USA, value based pricing UK, drug value and societal benefits. In addition, an analysis of a UK Department of Health consultation paper and the US Agency for Healthcare Research and Quality policy documents was performed in order to determine how comparative effectiveness and VPB may affect the market access and commercial viability of products in disease area of depression (SSRIs, SNRIs and atypical antidepressants). A spreadsheet was used to capture data and a comparison was undertaken to contrast the two markets and the different implications. RESULTS: The addition of comparative effectiveness and VPB are to take account of indirect cost associated with all disease areas. Depression has high indirect associated costs associated and, as such, the value of novel antidepressants will increase. This is likely to enable better access to new products. CONCLUSIONS: The move to comparative effectiveness and VPB is likely to affect the market access and commercial viability of new drugs in depression. There will be more positive drivers for investment in the disease area of depression. Moreover, decisions taken at the margin during the drug development process will be impacted as any change in market access is likely to affect the ‘Go/No go’ decision criteria. WHAT INFLUENCES PHARMACEUTICAL REIMBURSEMENT DECISIONS? A SYSTEMATIC REVIEW OF FACTORS REPORTED TO INFLUENCE DECISIONS IN OECD COUNTRIES

Bending MW1, Hutton J2, McGrath C2, Glavnić J

1University of York, York Health Economics Consortium, York, UK, 2Eli Lilly, Surrey, UK

OBJECTIVES: Many factors influence pharmaceutical reimbursement decisions. This study aims to determine the influence of factors considered in the evaluation of pharmaceuticals on the reimbursement decisions of government funded bodies in OECD countries. METHODS: A search of MEDLINE, EMBASE, EconLit, Health Management Information Consortium, NHS EED and REPEC Economic working papers until July 2010 was conducted. A hand search of the International Journal of Technology Assessment in Health Care was undertaken (1990-2010). The following study design criteria were applied: eligible: experimental; prospective, retrospective, quasi-experimental, retrospective, prospective, experimental, observational, case series and surveys or questionnaires design. The influential factors were reviewed across and within OECD countries. RESULTS: The search identified 12 quantitative studies and 23 qualitative studies. The quantitative studies considered factors that influence the decision-making process, such as cost efficacy, effectiveness and clinical utility. The qualitative studies explored stakeholder views and decisions during the evaluative process. CONCLUSIONS: The impact of factors that influence reimbursement decisions is complex and dynamic and there is a need for more research to understand the impact of the different processes employed across countries' decision-making. This is particularly true in certain disease areas. There will be more positive drivers for investment in the disease area of depression. Moreover, decisions taken at the margin during the drug development process will be impacted as any change in market access is likely to affect the ‘Go/No go’ decision criteria.

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yss, logistic analysis, survival analysis, and recursive partitioning decision analy-
sis were used to estimate the relationship between the financial impact of a new
drug indication and the probability of its reimbursement. The multivariable analy-
ces controlled for other clinical and economic variables that have been shown to be
correlated with reimbursement eligibility of regenerative medicines including the cost-
adjusted life-year gained. RESULTS: In all analyses, financial impact was a signif-
ificant predictor of the probability of reimbursement. For example, in the logistic analy-
sis, the odds ratio of reimbursement for a drug submission with a financial impact greater than A$10 million compared with A$0 or less was 0.12 (95% confi-
dence interval [CI]: 0.03-0.55), the odds ratio of reimbursement for a drug submis-
sion with a financial impact greater than A$0 up through A$10 million compared
with A$0 or less was 0.16 (95% CI: 0.04-0.60). Similar results were obtained in the
survival analysis. In the recursive partition decision analysis, the first split of the data
was done in a comparison with a positive financial impact compared with those with
a negative financial impact. CONCLUSIONS: In Australia, financial impact on the
health care system is an important determinant of whether a new drug is
recommended for reimbursement, even when cost-effectiveness estimates and oth-
er clinical and economic variables are controlled.

PHP65 HEALTH OUTCOMES AND ECONOMICS RESEARCH FOR CELLULAR THERAPIES AND
REGENERATIVE MEDICINES: LESSONS FROM A HEALTH TECHNOLOGY ASSESSMENT AND
REIMBURSEMENT ANALYSIS IN THE UNITED STATES
Faulkner EC, Spinner DS
RTI Health Solutions, Research Triangle Park, NC, USA
OBJECTIVES: Cellular therapies and regenerative medicines, are poised to have the
same paradigm-shifting influence on healthcare as monoclonal antibodies (mAbs)
and personalized medicine. While these therapies hold similarities to conventional
biopharmaceuticals, they also differ in material ways including attributes of both
device and pharmaceutical quality. The materials are based on the documentation of Taiwan’s NHI Drug Review Committee (DRC) over 15 years
period (1996–2010). We defined the criteria of pricing methods into 9 categories: International Price Comparison, Comparison with Similar Products with Equiva-
lent Therapeutic Effects, Price as a Key Driver, Specific Product, Reference Pricing,
Lowest Available International Price, Cost Analysis, Grouping and Others in-
cluded risk sharing managements. RESULTS: The total number of new drugs that
approved for reimbursement during 1996–2010 was 1103, and the number of petition cases was 587(53%). The total number of new drugs with final
pricing decisions in this study was 802. Among them, 343 items were issued with
reimbursement price without petition, and the remaining new drugs received their
reimbursement prices after petition on initial pricing decisions. The approved price
was averaged 69% of the international median prices, and was only 60% of inter-
national median prices among petition cases. The top three methods of pricing are
Price Proportion Method, Equivalent Therapeutic Effect with Similar Product
(23.5%), and International Price Comparison. Because NHI faced financial crisis in
the past 10 years, the price of reimbursement came as approximately 70% of their
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PHP66 DEMONSTRATING “DISEASE MODIFYING THERAPIES”? AN HTA PERSPECTIVE
McDonald F, Colasante W, Oshinowo B, Saraf S
PricePictive Ltd., London, UK
OBJECTIVES: The objective of this study was to understand what stakeholders in
US, EU5, Canada & Australia interpret as disease modification. In chronic progres-
sive conditions, disease modification versus symptom control is the ultimate goal
of healthcare specialists. However, there is no consensus on what “disease modi-
fication” really is. From a HTA perspective, not only is there is difficulty in valuing
disease modifying interventions, but also implied risk to payers approving to re-
imburse these drugs at launch. METHODS: Primary research was undertaken with
payers and medical specialists to understand requirements to support disease
modification claims in HTA assessments. Various attributes were assessed during
in-depth discussions and through discrete choice conjoint. Over 100 respondents were
interviewed. RESULTS: The results show that that efficacy is the most impor-
tant attribute considered in disease modification for HTA assessments. The corre-
lation of biomarkers to clinical endpoints also has utility. The market access im-
lications for such products across geographical vary considerably. However, cost-
effectiveness as a key driver in specific markets. CONCLUSIONS: Disease
modification means delaying or halting the progression of a disease. Efficacy is the
most important single factor, with the evidence of magnitude and duration of
effect both being essential. However, efficacy alone is insufficient to support a
disease modification claim. Robust long term data are also required and data show-
ing a real improvement in health status over current health status. Efficacy and
payers, acceptance of a disease modification claim means that payers take the
risk of reimbursing based on some extrapolation of data at launch. A commitment
to integrate a process of data review of outcomes over time linked with perfor-
manee will ensure any risk is mitigated.

PHP67 UNDERSTANDING THE VIETNAMESE PRICE AND REIMBURSEMENT ENVIRONMENT THROUGH A COMPARISON WITH THAT OF CHINA
Lewis S, Dummett H
Double Helix Consulting, London, UK
OBJECTIVES: Vietnam is an emerging pharmaceutical market that is both poorly
understood and undergoing change, with a target for introducing uni-
versal health coverage by 2014. The purpose of the research is to place Vietnam’s
pricing and reimbursement environment in a context that brings its dynamics into
clearer focus and to gauge the likely future direction of its evolution. METHODS: A
comparison with its neighbour, China, was conducted based on interviews in both
markets with government advisers, health economists and health policy profes-
sors and KOLs. A comparative analysis was then conducted of the market
access dynamics and drivers, as well as of policy reform plans. RESULTS: Many
similarities exist between the P&R environments of the two markets, although they
sit at different places along the P&R development continuum. In both, branded
drugs enjoy a considerable premium over generics, which could be as high as
40 times in Vietnam. However, China is looking to change the situation by remov-
ing the premium for off-patent branded drugs. Despite efforts in to develop their
reimbursement system, direct-sale to hospitals is the primary revenue channel for
pharmaceuticals, with KOL-endorsement a major market access driver. In terms of
drug price regulation, the most commonly used measure is through the enforce-
ment of price caps. CONCLUSIONS: The Vietnamese P&R system is similar to that of
China 5-10 years ago. Several fundamentals in terms the structure of the health
system formalisation of the reimbursement system and market access drivers are
the same, but China is significantly further down the line than its neighbour. How-
ever, the health reform agendas of the two markets are both heading in similar
directions.

PHP68 THE 15 YEARS EXPERIENCE OF NEW DRUG ADOPTION AND REIMBURSEMENT IN TAIWAN’S NATIONAL HEALTH INSURANCE
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OBJECTIVES: To present the empirical experience of new drug listing and reim-
bursement under Taiwan’s National Health Insurance (NHI), and to discuss the
performance of such mechanism. We also attempt to assess its impact on the public
access to pharmaceutical innovations. METHODS: The materials are based on
the documentation of Taiwan’s NHI Drug Review Committee (DRC) over 15 years
period (1996–2010). We defined the criteria of pricing methods into 9 categories: International Price Comparison, Comparison with Similar Products with Equiva-
lent Therapeutic Effects, Price as a Key Driver, Specific Product, Reference Pricing,
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Price Proportion Method, Equivalent Therapeutic Effect with Similar Product
(23.5%), and International Price Comparison. Because NHI faced financial crisis in
the past 10 years, the price of reimbursement came as approximately 70% of their
affected on the national median prices among petition cases. The approved price

PHP69 HOW MUCH FOR A QALY IN KOREA
Kim Y, Shin SL, Park S, Song H, Park J, Bae E, Abu J
1National Evidence-based Healthcare Collaborating Agency (NECAJ), Seoul, South Korea, 2Sung
University, Wonju, Kangan-do, South Korea
OBJECTIVES: To measure willingness to pay (WTP) for a QALY in Korea. METHODS: A
survey was undertaken with payers and medical specialists to understand require-
ments to support quality adjusted life-year gained (1 QALY) and an additional
improvements in Korea. Double bounded dichotomous choice (DBDC) questions
were also repeated for QALY improvements of a family member instead of self.
The questionnaire also included questions on demographics, disease status, and
was an open question were used to elicit WTPs. Each person was asked for
four scenarios chosen from 3-item EQ-5D scenarios (1 QALY) and an additional
scenario with live in perfect health for 1 year or die now (23.5%) and International
Price Comparison. Because NHI faced financial crisis in
the past 10 years, the price of reimbursement came as approximately 70% of their
affected on the national median prices among petition cases. The approved price

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