grown in Italy in recent years. Our analysis reflects the strong AIFA's commitment to allow early access to new drugs for patients in high need.

PHP57

EUROPEAN CONDITIONAL MARKETING AUTHORIZATION – DOES EARLY MARKETING AUTHORIZATION TRANSLATE INTO EARLY REIMBURSEMENT AND PATIENT ACCESS?

Macaulay R, Rothwell S, Sutcliffe D
PAREXEL International, London, UK

OBJECTIVES: Since 2006, the European Commission has granted Conditional Marketing Authorizations (CMAs) for medicines where the benefit of immediate availability outweighs the risk of less comprehensive data than normally required in cases of severe/life-threatening disease, emergency situations or orphan medicines. However, to reach patients, medicines must also be publically reimbursed. This research aims to assess the reimbursement outcomes of major public payer bodies of all medicines approved under CMAs. **METHODS:** Medicines approved under CMAs and their appraisals by major European payer bodies (NICE, SMC, G-BA, and HAS) were identified from the relevant website and key data extracted. **RESULTS:** 38 medicines have received CMAs with no clear trend over time (3.2/year; range 1 [2006,2009]–8[2016]). 45% (17/38) were for oncology indications and 58% (22/38) had been converted to a full MA after a median of 38.4 months. 83% (19/23) of medicines with CMAs that were assessed by NICE received positive outcomes (defined as "recommended" or "conditional"), compared with 57% (16/26) by SMC (defined as "accepted" or "restricted"), 74% (14/19) by G-BA (defined as any level of additional benefit) and 29% by HAS (defined as ASMR I-III). Rates of positive outcomes were lower for oncology vs. non-oncology CMAs across all payers (NICE: 75% vs. 100%; SMC: 47% vs. 73%; G-BA: 67% vs. 86%; HAS: 27% vs. 33%). The median delay between EC-CMA approvals and positive HTA outcome were 13.0 months (NICE), 11.0 months (SMC), 7.0 months (G-BA), and 5.0 months (HAS). **CONCLUSIONS:** CMAs have enabled earlier market authorizations for products that address severe unmet needs. However, many have failed to gain favourable reimbursement outcomes and, for those that have, this has been at a significant delay. Companies need to ensure that their evidence generation plans and strategy meet the needs of both payers and regulators to ensure optimal patient access and commercial returns.

PHP58

SPANISH THERAPEUTIC POSITIONING REPORTS: 6-YEAR UPDATE – INCREASING IN FREQUENCY BUT NOT RELEVANCE?

Macaulay R, Fernandez Dacosta R
PAREXEL International, London, UK

OBJECTIVES: There are 17 autonomous regions in Spain, each can make distinct price/reimbursement decisions for drugs. To drive more consistency, the Spanish Agency for Medicines and Health Products have issued national Therapeutic Positioning Reports (TPRs) for new medicines since 2012. These comparative efficacy and safety reports specify the patient population that will benefit most. This research analyses the number, indications and outcomes of TPRs. **METHODS:** All publically-available TPRs were screened up to 31/05/2018 and key data extracted. **RESULTS:** 165 drug-indication pairings with an associated TPR were identified with 1, 2, 8, 42, 51, 41, and 20 issued annually between 2012 and 2018. The median delay between European marketing authorization and TPR publication was 385 days. Oncology was the most common indication (36% [59/165]). The majority (60% [99/165]) of drug-indication pairings were recommended as additional possible/alternative treatment options with only 19% (32/165) deemed to offer superior benefit over existing alternatives and 21% (34/165) not recommended in any subpopulation. Although economic analyses are not mandatory, 36% (58/165) did include an economic evaluation and cost-efficiency was considered in an additional 30% (50/165). Economic evaluations notably increased over time (0% TPRs [2012-2014], 26% [2015], 35% [2016], 49% [2017], 45% [2018]). However, in only 33% of cases (19/58) did economic evaluations impact recommendations. **CONCLUSIONS:** Since 2015, the number of TPRs issued have dramatically increased. However, clear positive or negative recommendations are relatively uncommon and most are issued over a year post-marketing authorization. Their impact has been further questioned through the lack of inclusion of economic evaluations as standard, a key consideration for regional bodies. Although economic evaluations are becoming more frequent in TPRs, their impact is often still limited. In September 2017, the government announced plans to improve the TPR model, but the specific mechanism remains unclear.

PHP59

MOVING FROM THE REGULATOR TO THE PAYER AND PRESCRIBER - HOW PHARMA CAN WORK BETTER TO EFFECTIVELY MANAGE EMERGING CHALLENGES TO THE COMMERCIAL SUCCESS OF INNOVATIVE NEW THERAPIES

Macaulay R[1], Gokool N[2], Tang M[1], Samuels ER[1], Langford C[2]
[1]*PAREXEL International, London, UK,* [2]*PAREXEL International, Brighton, UK*

OBJECTIVES: Regulatory approval is traditionally the main hurdle for developers bringing new therapies to patients. However, today, physician and payer acceptance is becoming more critical as expedited regulatory pathways provide marketing authorization with more preliminary data to therapies for severe diseases, whilst payers, facing budgetary pressures, demand more evidence to justify prices. Innovative new therapy classes will further amplify this trend: for example, CAR-T cell and gene therapies with potential curative benefits across multiple severe and rare diseases will be likely candidates for expedited regulatory approval but their high prices will incur strong payer scrutiny. Glybera®, the first EC-approved gene therapy, is no longer marketed for commercial reasons, emphasizing these reimbursement and prescribing challenges. Glybera® is an Advanced Therapy Medicinal Product (ATMP), encompassing gene, cell, and tissue-engineered therapies. This research assesses the commercial success of ATMPs. **METHODS:** European Commission (EC)-approved ATMPs were identified alongside their reimbursement status in UK, Italy, and Germany from relevant regulatory/HTA websites plus a targeted review of company press releases (to 12/21/2017). **RESULTS:** Nine ATMPs have received EC-approvals. The first four (ChondroCelect®, Glybera®, MACI®, Provenge®) are no longer marketed for commercial reasons after a mean 3.5 years from approval. Glybera®, the first EC-approved gene therapy (2012) priced at >€1 million/patient, was withdrawn in 2017 having reported one sale to 2016. Five other ATMPs have been EC-approved since 2015 but only Strimvelis® (second EC-approved gene therapy) received >1 positive HTA appraisals. However, Strimvelis® reported only one sale in its first year-on-market and GSK divested its rare disease portfolio in 2018. **CONCLUSIONS:** The reimbursement and prescribing hurdles are becoming increasingly key to commercial success, particularly among emerging transformational therapy classes. Early development and co-creation of payer messaging and scientific communication points can ensure alignment, facilitate engagement, and identify and prospectively mitigate key evidence gaps to reduce uncertainty and optimize reimbursement.

PHP60

PAYER ARCHETYPING – EXPANDING HORIZONS

Campbell J, Roibu C, Ivanova H, Macaulay R
PAREXEL International, London, UK

OBJECTIVES: The traditional pharmaceutical market model is being challenged by the emergence of middle-income countries with rapidly growing economies, healthcare coverage, and willingness-to-pay for medicines. Understanding how these emerging markets determine appropriate pricing and reimbursement for public healthcare coverage of new therapies will thus become increasingly important considerations for global product launches. This research aims to analyse pricing and reimbursement processes across a broad range of markets and archetype these into groups based on commonalities in key decision-drivers. **METHODS:** 30 countries were selected based on their total healthcare expenditure (using World Health Organisation data). Publically-available information on the reimbursement processes and decision-making criteria were identified from the relevant sources and supplemented with internal expertise, which were grouped into archetypes based on commonalities. **RESULTS:** Four payer archetypes for public healthcare technology reimbursement were identified and countries were classified into: cost-effectiveness driven, budget impact driven, competitive tendering driven, and clinical-benefit driven. Of the markets assessed, 27% (9/30) were cost-effectiveness driven (Australia, Canada, Colombia, Ireland, Netherlands, England, Republic of Korea, and Sweden), 50% (15/30 were budget impact driven (Argentina, Austria, Brazil, Chile, China, Finland, Indonesia, Iran, Israel, Italy, Mexico, Poland, Russian Federation, South Africa and Spain), 7% (2/30) were competitive tendering (the USA and India), and 17% (5/30) were clinical-benefit driven (Belgium, France, Germany, Japan, Switzerland). **CONCLUSIONS:** Optimal pricing and reimbursement in emerging markets is becoming increasingly important in determining the global commercial success of new medicines. This research identifies common criteria from their diverse pricing and reimbursement processes and groups these into discrete archetypes. Utilizing these groupings can better enable companies to appropriately and efficiently develop strategic decision-making, evidence generation, and communication plans that can be inclusive of the needs of emerging markets.

PHP61

THE NEW AIFA INNOVATION FRAMEWORK TO RECOGNIZE INNOVATIVE DRUGS: A PRELIMINARY ANALYSIS OF KEY DRIVERS OF EVALUATION

Meremetidis A, Ferrario M, Giuliani G
Roche S.p.A., Monza, Italy

OBJECTIVES: The purpose of the following analysis is to provide a deeper understanding of the key drivers that determine if a molecule can be recognised with the innovation status in Italy. In March 2017, AIFA (Italian Medicine Agency) defined three criteria (unmet need, therapeutic added value and clinical data robustness assessed through the GRADE methodology) to assess the innovation status of new treatments. The aim is to accelerate access to innovative drugs, also granting access to 1Bio€ funds allocated by the Italian Government. **METHODS:** The possible outcomes of the evaluation for the three criteria have been transformed into a numerical scale from 1 to 5. This way, it has been possible to compute the mean of the results obtained for each criterion, by distinguishing between drugs that got the innovation status or the conditional innovation status and those defined as non-innovative. The analysis is based on the reports published on the AIFA website (update: May 10). **RESULTS:** Outcomes of the research are: The outcome of unmet need evaluation is medium-high for both innovative and non-innovative drugs (innovation: 4, conditional: 3, non-innovation: 3.86). The therapeutic added value results to be fundamentally different for the three types of drugs (innovation: 4, conditional: 3, non-innovation: 2.33). Non-innovative drugs got, on average, significantly lower evaluations. In regard of the last criterion, it is worth distinguishing between molecules for rare diseases and other drugs. While in the first case the evidences' quality proves to be less relevant (innovation: 2.92, conditional: no data, non-innovation: 2.5), for the others it is observed a more peculiar pattern (innovation: 3.75, conditional: 3.75 non-innovation: 3). **CONCLUSIONS:** A significant unmet need is fundamental for the status recognition. However, it seems that the key criterion is the therapeutic added value, based on validated endpoints and proper comparators. Additional experience is needed to validate those primary findings.

PHP62

HOME BREWED AUTOLOGOUS THERAPIES: CAN BIOPHARMA WIN THE WAR WITH DOMESTIC AUTOLOGOUS CELL & GENE THERAPY MANUFACTURERS?

Khurana S, Kumar A, Sotou DS
Huron Consulting Group, London, UK

OBJECTIVES: Governments in many countries (e.g. UK and Canada) are investing in developing cell and gene therapy (CGT) R&D, manufacturing, and