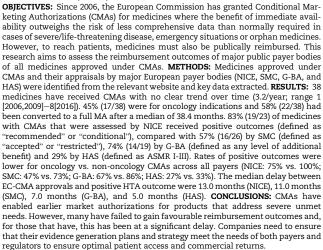
grown in Italy in recent years. Our analysis reflects the strong AIFA's commitment to allow early access to new drugs for patients in high need.

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

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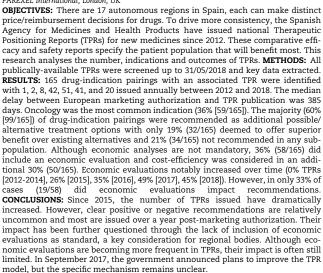


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SPANISH THERAPEUTIC POSITIONING REPORTS: 6-YEAR UPDATE — INCREASING IN FREQUENCY BUT NOT RELEVANCE?

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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



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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 ECapproved 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.

PAYER ARCHETYPING - EXPANDING HORIZONS

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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.

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

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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.

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



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