questions about priority and importance of certain outcome parameters like the thrombolysis rates vs. the independence of patients after 3 months. Finally, the combination of MCDA and simulation modeling contributes to a transparent analytic process and results in a more complex understanding of the technology.

**PHP218**

**MECHANISM OF COORDINATED ACCESS TO ORPHAN DRUGS**

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Although the EU Council stated that *"All health systems in the EU aim to make provision, which is patient-centered and responsive to individual need"*, unacceptable differences in access to orphan medicinal products (OMP) in the Member States of the European Union are identified. In the context of the 2010 Belgian EU presidency initiative on 'Innovation and Solidarity' and within the framework of the process on corporate responsibility in the field of pharmaceuticals, EU Commissioner Tajani therefore launched the project Mechanism of Coordinated Access to OMP.**OBJECTIVES:** Designing a operational mechanism of coordinated access to OMP for patients, stakeholders and Member States to provide, irrespective of the local conditions, access for patients with unmet medical needs and for whom these solutions would otherwise be out of reach – in an affordable and sustainable way ("real life access"). **METHODS:** The project is managed by Belgium (NIHDI), supported by the European Commission and Eminet. Thirteen Member States participated, with the stakeholders (AIM, EPF, ESIP, Eurordis, CPME, EFPIA, EGA, EuropaBio, GIRP). Three Workpackages cover the different aspects of granting effective access to medicines: Identifying and assessing a relevant orphan drug (assessment/evaluation) - Selection of target population and mechanisms of funding (structural access) - Treatment (individual access). Feasibility at present and opportunities for near future development of desirable activities were studied, and no-go solutions were documented and rejected in order to develop implementable scenarios for pilot projects and policy recommendations. Discussion: Although coordinated access at an European level will be organized on a voluntary basis, some sort of commitment from the participating partners is required. Moreover, it is crucial that the subsidiarity principle is not jeopardized or compromised. Duplication of efforts will be avoided and previously made investments – in terms of financial and human resources, expertise and experience - (ex. by EUnet HTA, EMA COMP, EUCERD, CAVOD,…) will be valorized.

**PHP219**

**THE FUNDING OF ORPHAN MEDICINES IN EUROPE: PAYERS ACHILLES' HEEL?**

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**OBJECTIVES:** To look at the affordability of orphan medications across Europe and whether payer attitudes to high-price medications are changing in the face of rising health care expenditure and tighter budgets. **METHODS:** A detailed review of 7 EU markets (France, Germany, Italy, The Netherlands, Poland, Spain, UK) looking at payer attitudes and funding decisions for key orphan drugs and the political, economic and societal impact of these. A key focus of the research was insight into payer attitudes towards the evidence base for the purpose of pricing negotiations and how anecdotal evidence, such as Patient Reported Outcomes (PROs) and patient case studies, have an impact on decision-making. Detailed research was also undertaken to ascertain the pricing levels achieved for a number of orphan drugs across Europe looking at payer thresholds and the implications of these for the purpose of reimbursement. **RESULTS:** The research demonstrates that there is considerable variation in pricing levels across the European markets and difference in payer attitudes towards the way orphan drugs are funded. Overcoming evidence challenges in orphan diseases remains a headache for payers and scepticism remains around dosing, innovation and whether approaches such as "coverage with evidence development" are adequate and/or sustainable in the long-term following initial approval. **CONCLUSIONS:** The environment for orphan medicines in Europe is changing; and as the financial performance of European countries begins to diverge, so do attitudes towards the funding of orphan medicines. Orphan medicine prices are rarely justified on the basis of traditional cost-effectiveness thresholds and most markets still differentiate them from other pharmaceuticals. However, payers are afraid of uncertainty and, given the increasing number of orphan drugs and the often tentative evidence base at launch, may be forced by overwhelming financial necessity to make tougher decisions on funding.

**PHP220**

**BUDGET IMPACT AND THRESHOLDS: HOW ARE REAL DECISIONS MADE? - EXPECTED UTILITY OR PROSPECT THEORY?**

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Decision making rules in health technology assessments are often based on a fixed willingness to pay threshold for the incremental cost effectiveness. This may be thought of as consistent with expected utility – with utility here defined in terms of Incremental Net Benefit (INB) – a combination of QALY gain and the threshold value. Alternative methods such as multi criteria decision analysis allow incorporation of other dimensions into the decision space. These seek to explore whether utility may be driven by factors other than simple QALY gain. Prospect theory suggests that decision makers are concerned in practice with the 'size' of a given decision. Also, they handle investment and disinvestment differently. In the Irish State, every new drug is examined. In order to look at actual decisions, lifetime QALY gains were extracted from completed economic evaluations submitted to the Irish health care payer. Total spend on these was calculated using a combination of the payer reimbursement database and predicted budget impact. Real choices indicate that where the budget impact is relatively small the drug is more likely to be reimbursed even with a comparatively small QALY gain. Technologies in areas of cancer and orphan diseases often lie outside of the threshold where a technology would be accepted. Decision makers are faced with choices with varying degrees of risk. Choices associated with a low budget impact are deemed to be less risky. This is pragmatic; it reflects a preference on behalf of the decision maker. Reality does not always match the ideal of a fixed threshold.

**PHP221**

**DOES HTA PROCESS HELP TO ACHIEVE THE HEALTH OBJECTIVES OF THE MILLENNIUM? A SOUTH AMERICAN ANALYSIS**

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**OBJECTIVES:** South America (SA) is a continent with 400 million people and occupies 12% of the world's territory. It is composed by 12 countries and 6.75% of its population is below the poverty line, as defined by UN. The proper distribution of financial health resources, through an HTA process in public systems is potentially essential to improve the quality of health care expenditure. The objective of this study is to understand the incorporation process of new health technologies and compare the general health status in each country, regarding the Objectives of the Millennium (OM). **METHODS:** A public data collection was performed in official sources linked to UN, to governments of SA and the Unión de las Naciones Suramericana (UNASUR). **RESULTS:** The public health financing in SA countries was between 2.43% and 6.20% of the GDP. An HTA process in an institutionalized and specialized form is in place in only 3 countries (Argentina, Brazil and Peru). Bolivia, Chile, Colombia, Paraguay and Venezuela do not have a specialized HTA process and the other four countries have no HTA process at all. Regarding the OM the decrease in child mortality, increase in vaccination, increase in malaria and tuberculosis treatments are among the closest to be achieved in all countries. There is a linear positive correlation of OM with the Human Development Index and with the percentage of GDP invested in public health but not with having a HTA process in place. **CONCLUSION:** At this moment, there is no evidence that an HTA process in place helps SA countries to achieve the OM.

**PHP222**

**THE NEW ITALIAN HEALTH CARE REFORM: INTRODUCING NEW MEASURES TO SPEED UP MARKET ACCESS**

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**OBJECTIVE:** The Balduzzi law (189/2012) introduced several changes aimed at promoting the country's development through a higher level of health protection and at bridging the gap left by the rationing of health care resource from the Spending Review (135/2012). Reducing the time to drug market access is one of the main purposes. The aim of the research is a critical analysis of this law to understand its actual and future impact on the health care scenario. **METHODS:** An evaluation of the laws issued in the last three years that aimed at regulating the drug market was carried out. To build a future scenario analysis, we focused our attention on the Balduzzi law and two of its articles (11 and 12) and on the new drugs approved by AIFA and commercialized under the new regulation. **RESULTS:** The changes that will have a major impact on the drug market are: the allocation of the medicines approved under centralized procedure in the non-negotiated C Class within 60 days from the publication in the Official Gazzette of the European Union and the direct placement of generics and biosimilars in the reimbursement class of the originator without any price negotiation. As of now, a total number of 49 drugs have been included in the non-negotiated C Class, within this new group there are 15 first drug authorizations. **CONCLUSION:** The new reform can be potentially an interesting innovation to speed up market access, though the impact of including new drugs in the C class (at patient charge) before the price negotiation is still under debate. The increased competitiveness coming from having a faster introduction in the market of generics/biosimilars could lead to important savings for the National Healthcare System over the next years.

**PHP223**

**INVESTING IN EUROPEAN HEALTH R&D – A PATHWAY TO SUSTAINED INNOVATION AND STRONGER ECONOMIES**

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A large number of factors point to an unavoidable rise in health care expenditure to 13%-18% of Europe's GDP by 2030, even with policy interventions or budget caps that aim to counterbalance these pressures. This growth in health care costs need not be undesirable especially so when higher spending on health care leads to improved health care quality and life expectancy. Therefore, the challenge is not "how do we reverse the growth of health care costs?" but "how can we best deploy the increasing resources spent on health care to create optimal benefits for the European population?" Health R&D is the key to being able to respond to this dilemma. Increased investment in R&D leads to improved health outcomes, long term efficiency gains, better productivity and high economic yields. However, the outlook for Europe is not as positive as it could be. Recently, there has been a stagnation or even decline in European private and public investment in R&D, which is in sharp contrast with the much higher investments in the US. Private biopharmaceutical investments in health R&D, which are double the size of public health R&D, in 2011 actually decreased in absolute terms. Public R&D investments declined or stagnated in most European countries and will be further under pressure in the near future due to public budget deficits. Janssen commissioned the Deloitte European Center on Health Economics and Outcomes Research to set out the arguments in support of increased investment in health R&D in Europe. The paper demonstrates that, even in times of austerity, policymakers need to prioritise approaches that will enhance public R&D investments and adopt strategies that produce incentives for private enterprises so that the current decline in private sector investment is halted.

**PHP224**

**HOW DO ONCOLOGY DRUG PRICES VARY ACROSS EU5, THE UNITED STATES, AND BRIC MARKETS?**

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