Orphan Drugs, only the Eculizumab for the treatment of PNH has no approved registration by the National Health Surveillance Agency (ANVISA). CONCLUSIONS: Considering its high cost, high judicial demand and limited availability of scientific evidence, orphan drugs represent a challenge for researchers and decision makers. Clinical benefit, disease severity, availability of therapeutic alternatives, ethical, political and economic aspects should be considered. Public argumentation and disciplinary reflection on the development of HTA models and policies regarding rare diseases and innovative treatments in the SUS, as well as fostering the primary researches in this field.

PSY106 TO WHAT EXTENT DO DISEASE AND TREATMENT CHARACTERISTICS INFLUENCE HTA-BASED RECOMMENDATIONS FOR A SAMPLE OF ORPHAN DRUGS IN THREE COUNTRIES, AND COULD THESE INDICATE WHETHER ORPHAN DRUGS HAVE A “SPECIAL STATUS”? Nicolò F. London School of Economics and Political Science, London, UK Routine HTA methods may not adequately capture all the important considerations of a treatment’s value and the impact of the condition on the patient given that the evidence is often incomplete. This study aims to explore the broader considerations of scientific and social value judgments on reimbursement decisions for a sample of orphan drugs OBJECTIVES: To identify and compare the extent to which these broader considerations were captured between countries. METHODS: We compared HTA documents published in Italy, France and Brasil for 10 drugs. We compared HTA reports in a previously developed data extraction tool and assessed HTA documents for the presence of broader considerations and for their inclusion in the decision. RESULTS: Differences between HTAs were seen for the extent to which broader considerations were captured including: evidence and approval processes; and quantitative identification of agency-specific risk preferences and agreement levels across countries. CONCLUSIONS: Considerable variation was seen in the application of these broader considerations. Identifying these is a way forward to highlight areas where more research is needed, or consistency and transparency are needed. Some of these other considerations may also favour orphan drugs, furthering the debate around whether orphan drugs deserve special status.

PSY105 WHY ARE THERE DIFFERENCES IN HTA RECOMMENDATIONS ACROSS COUNTRIES? A SYSTEMATIC COMPARISON OF HTA DECISION PROCESSES FOR A SAMPLE OF ORPHAN DRUGS IN FOUR COUNTRIES Nicolò F. London School of Economics and Political Science, London, UK HTA reimbursement recommendations often result in different outcomes across countries despite the same evidence being assessed for a same technology. There is a need to understand the reasons for these differences. OBJECTIVES: To systematically compare HTA processes for a sample of orphan drugs across four countries (England, Scotland, Sweden, France) to identify the use and interpretation of evidence, and agreement levels across countries. METHODS: Ten orphan drug indication pairs were selected and systematically compared using a previously validated framework. An exploratory sequential mixed methods design divided the research into two stages: (1) a detailed in-depth analysis of the decision-making processes; and (2) quantitative identification of agency-specific risk preferences and agreement levels across countries. RESULTS: Differences at each step of the decision-making process were identified. The same pivotal trials were appraised but with varying levels of detail in reporting the clinical outcomes, explaining some of these differences. Poor to moderate agreement in the interpretation of the evidence was measured using Cohen’s kappa scores. This reflected situations where the countries interpreted the same evidence differently and situations where differences in the handling of the same uncertainties were seen, including differences in the extent to which stakeholder input influenced a decision. CONCLUSIONS: This research systematically compared HTA processes in different countries, facilitating the understanding of these complex processes including how different HTA bodies conduct value assessments. It enabled to raise awareness around the reasons for differences across countries, and highlight areas for potential methodological improvements in HTA. Further application of this framework to other disease areas and countries is a way forward to improving the drivers of coverage decisions while better understanding the settings and limitations of HTA.

PSY104 HEALTH TECHNOLOGY ASSESSMENT, PRICE AND REIMBURSEMENT REVIEW FOR ORPHAN DRUGS IN FRANCE Tavella F.1, Kornfeld A.1, Kornfeld A.1 1Cristo Réutier Paris, France, 2University of Chile, Santiago, Chile, 3University of Melbourne, Manchester, France OBJECTIVES: In France Orphan Drugs (OD) undergo the same Health Technology Assessment (HTA) procedure as other drugs. The evaluation is performed by the Transparency Committee (TC). Two scores are assigned and further used for pricing & reimbursement decision: drug’s medical benefit (SMR) and improvement in medical benefit (ASM). OD can be eligible to an accelerated procedure established by innovative products. The study aim is to analyse HTA decisions, prices and reimbursements for innovative treatments. METHODS: All OD assessed in Italy since 2000 were identified. Prices, reimbursements rates and decision details were extracted for each drug using Farmadati database. RESULTS: Among 74 OD approved in Europe 66 molecules are officially available in Italy. It took 1-17 months from granting market authorization to final decision on pricing and reimbursement and publication in ‘Gazetta ofﬁcielle’. The mean time was 17 months (SD 11). The number considered and grouped into 15 clusters based on the information provided. The most common related to the nature of the disease, and considerations based on rarity or unmet need. 52% were one of the main reasons for the reimbursement decision. The most common score was 5. Further, there is also a potential tendency in accepting a broad and uncertain ICERs. Categorising these as social or scientific value judgments was done to identify areas where further elicitation of societal preferences, and where more consistency and transparency their use are needed, respectively. Each of these was then compared to the actual situation to determine the extent to which OD are reimbursed in Italy. The process is more transparent than France and better describes the actual situation, with the exception of one. CONCLUSIONS: There is an awareness of the need to standardise OD reimbursement in Italy but with varying levels of detail in reporting the clinical outcomes.

PSY103 HEALTH TECHNOLOGY ASSESSMENT, PRICE AND REIMBURSEMENT REVIEW FOR ORPHAN DRUGS IN ITALY Tavella F.1, Kornfeld A.1, Kornfeld A.1 1Cristo Réutier Paris, France, 2University of Chile, Santiago, Chile, 3University of Melbourne, Manchester, France OBJECTIVES: In Italy HTA (Health Technology Assessment) is conducted by the Scientific Technical Commission of Italian Medicines Agency (AIFA) with further negotiation between the manufacturers and the AIFA’s Pricing & Reimbursement Committee on price and reimbursement. After the decision is taken it is published in the Gazette ofﬁcielle or newspaper. AIFA’s Pricing & Reimbursement Committee negotiates the price, and if an agreement is not reached, prices are slightly higher in Italy but France displays a multiple confidential rebates making it impossible to compare net prices. In Italy the actual accessibility depends a lot on regional level unlike France. METHODS: All OD assessed in Italy since 2000 were identiﬁed. Prices, reimbursement rates and decision details were extracted for each drug using Farmadati database. RESULTS: Among 74 OD approved in Europe 66 molecules are officially available in Italy. It took 1-17 months from granting market authorization to final decision on pricing and reimbursement and publication in ‘Gazetta ofﬁcielle’. The mean time was 17 months (SD 11). The number considered and grouped into 15 clusters based on the information provided. The most common related to the nature of the disease, and considerations based on rarity or unmet need. 52% were one of the main reasons for the reimbursement decision. The most common score was 5. Further, there is also a potential tendency in accepting a broad and uncertain ICERs. Categorising these as social or scientific value judgments was done to identify areas where further elicitation of societal preferences, and where more consistency and transparency their use are needed, respectively. Each of these was then compared to the actual situation to determine the extent to which OD are reimbursed in Italy. The process is more transparent than France and better describes the actual situation, with the exception of one. CONCLUSIONS: There is an awareness of the need to standardise OD reimbursement in Italy but with varying levels of detail in reporting the clinical outcomes.

PSY102 HEALTH TECHNOLOGY ASSESSMENT, PRICE AND REIMBURSEMENT REVIEW FOR ORPHAN DRUGS IN FRANCE Tavella F.1, Kornfeld A.1, Kornfeld A.1 1Cristo Réutier Paris, France, 2University of Chile, Santiago, Chile, 3University of Melbourne, Manchester, France OBJECTIVES: In France Orphan Drugs (OD) undergo the same Health Technology Assessment (HTA) procedure as other drugs. The evaluation is performed by the Transparency Committee (TC). Two scores are assigned and further used for pricing & reimbursement decision: drug’s medical benefit (SMR) and improvement in medical benefit (ASM). OD can be eligible to an accelerated procedure established by innovative products. The study aim is to analyse HTA decisions, prices and reimbursements for innovative treatments. METHODS: All OD assessed in Italy since 2000 were identified. Prices, reimbursement rates and decision details were extracted for each drug using Farmadati database. RESULTS: Among 74 OD approved in Europe 66 molecules are officially available in Italy. It took 1-17 months from granting market authorization to final decision on pricing and reimbursement and publication in ‘Gazetta ofﬁcielle’. The mean time was 17 months (SD 11). The number considered and grouped into 15 clusters based on the information provided. The most common related to the nature of the disease, and considerations based on rarity or unmet need. 52% were one of the main reasons for the reimbursement decision. The most common score was 5. Further, there is also a potential tendency in accepting a broad and uncertain ICERs. Categorising these as social or scientific value judgments was done to identify areas where further elicitation of societal preferences, and where more consistency and transparency their use are needed, respectively. Each of these was then compared to the actual situation to determine the extent to which OD are reimbursed in Italy. The process is more transparent than France and better describes the actual situation, with the exception of one. CONCLUSIONS: There is an awareness of the need to standardise OD reimbursement in Italy but with varying levels of detail in reporting the clinical outcomes.

PSY101 TOP 20 ORPHAN DRUGS AVAILABILITY, PRICING AND REIMBURSEMENT IN SELECTED COUNTRIES: A SYSTEMATIC REVIEW Rempazat C.1, Rodrigues J.2, Slezakova Z.1 1Brazilian Ministry of Health, Brasília, Brazil, 2Brazilian Ministry of Health, Brasília-DF, Brazil OBJECTIVES: Publicly available HTA reports were reviewed using thematic analysis to systematically identify and compare broader considerations across countries using an existing analytical framework. METHODS: Among 74 OD approved in Europe 66 molecules are officially available in Italy. It took 1-17 months from granting market authorization to final decision on pricing and reimbursement and publication in ‘Gazetta ofﬁcielle’. The mean time was 17 months (SD 11). The number considered and grouped into 15 clusters based on the information provided. The most common related to the nature of the disease, and considerations based on rarity or unmet need. 52% were one of the main reasons for the reimbursement decision. The most common score was 5. Further, there is also a potential tendency in accepting a broad and uncertain ICERs. Categorising these as social or scientific value judgments was done to identify areas where further elicitation of societal preferences, and where more consistency and transparency their use are needed, respectively. Each of these was then compared to the actual situation to determine the extent to which OD are reimbursed in Italy. The process is more transparent than France and better describes the actual situation, with the exception of one. CONCLUSIONS: There is an awareness of the need to standardise OD reimbursement in Italy but with varying levels of detail in reporting the clinical outcomes.

PSY100 THE IMPACT OF THE EVALUATION OF THE EVIDENCE ON THE PAYMENT/REIMBURSEMENT DECISIONS FOR ORPHAN DRUGS: A SYSTEMATIC REVIEW A540

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