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**RESEARCH ON METHODS STUDIES**

**PSY133**

**ASSESSMENT OF REAL-WORLD TREATMENT PATTERNS AND OUTCOMES IN RELAPSED OR REFRACTORY MULTIPLE MYELOMA: EVIDENCE FROM A BRIEF MULTI-COUNTRY SURVEY OF EUROPEAN PHYSICIANS**

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**OBJECTIVES:** Data from real-world European settings describing treatment patterns and outcomes in relapsed/refractory multiple myeloma (RRMM) are limited. This study aimed to describe the knowledge gap using a brief physician survey. Sixty-one physicians treating RRMM were surveyed (November 2014) in France (n=21), Germany (n=20), and the United Kingdom (UK) (n=20). The survey collected physicians' opinions on typical treatment patterns and outcomes of RRMM patients. Descriptive analyses were performed. Analyses were descriptive.

**RESULTS:** Sixty percent of physicians in France and the UK were haematologists, versus 10% in Germany (where 80% were once-once high-risk patients). The proportion of patients on an ISS stage and cytogenetics was 18%-24% across countries. Bortezomib/thalidomide/dexamethasone was the most common induction regimen (42%) for stem cell transplant (SCT)-eligible patients in France, but was unused in Germany and less commonly prescribed in the UK (17%); bortezomib/cyclophosphamide/dexamethasone was the reported induction therapy for 28% of SCT-eligible patients in all countries. Regardless of SCT status, lenalidomide/dexamethasone was the predominant second-line treatment (37%-48%) reported in France and Germany, in the UK second-/later-line regimens were more varied, with both lenalidomide- and bortezomib-based regimens being reported as common. Second-/later-line therapy duration was generally short, particularly in the UK where 75% of physicians reported a <6-month duration. Disease progression was the top reason for second-/later-line discontinuation, other common reasons included toxicity and completion of planned therapy course. For high-risk patients, >75% of physicians reported median survival duration >6 years from first relapse. **CONCLUSIONS:** The proportion of RRMM patients with high-risk disease in real-world settings (18%-24%) may exceed that reported in clinical trials (10%-15%). Second-/later-line therapy duration is typically short, highlighting the need for further prospective studies for palliative care, and for patients remaining on treatment for >2 years. Patient-level studies are needed to better characterize the unmet needs in RRMM signaled by our findings.

**PSY134**

**CHARACTERISTICS ASSOCIATED WITH ANNUAL BLEEDING FREQUENCY AMONG HEMOPHILIA PATIENTS IN THE UNITED STATES**

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**OBJECTIVES:** Bleeding episodes pose significant economic and quality-of-life (QoL) burdens on persons with hemophilia. This study aims to identify socio-demographic and clinical characteristics associated with annual bleeding frequency (ABF). **METHODS:** Between 2005-2007 and 2009-2012, the Hemophilia Utilization Group Studies Vsa and Vb, respectively, recruited hemophilia A or B patients from 10 geographically diverse Hemophilia Treatment Centers in the United States. Adult patients or parents of children completed a baseline survey that collected information regarding socio-demographics, clinical characteristics, and treatment patterns. Data was unblinded before analysis for 92% of included patients, followed by blinded review of 90% of patients. **RESULTS:** In total, 7,219 patients were included in the analysis. Mean age was 15.9 years (range 0.1-73.9), and 92% of patients were male. Of 477 recruited patients, 51% had severe hemophilia, and 63.1% had severe hemophilia. Variables significantly associated with ABF included age (p<0.01), history of severe bleeding (p<0.01), 3 or more annualized hemoglobin and platelet transfusion events (p<0.01), joint and surgical hemorrhage (p<0.01), non-surgical hemorrhage (p<0.01), joint-related event (p<0.01), joint disease (p<0.01), and hemophilia A (p<0.01). **CONCLUSIONS:** A number of variables are associated with ABF, including age, history of severe bleeding, hemoglobin and platelet transfusion events, surgical and non-surgical hemorrhage, joint-related event, joint disease, and hemophilia A. These findings reinforce the importance of optimizing treatment for individual differences. Future studies should investigate how variations in bleeding outcomes and treatment are associated with healthcare costs.

**RESEARCH REPOSITORY PRESENTATIONS – SESSION V**

**PM1**

**CART ANALYSIS AS A TOOL TO DETERMINE OPTIMAL TREATMENT INTENSIFICATION TIME IN DIABETES**

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**OBJECTIVES:** A key objective of treating type 2 diabetes mellitus (T2DM) is maintaining glycemic control, (glycated haemoglobin-HbA1c) with targets set between 4.5%-6.5% to intensify treatment in uncontrolled patients and a study was designed to assess the long term clinical effects of this inertia. The study involved Classification and Regression Tree (CART) analyses to evaluate the time needed for patients to gain glycemic control. **METHODS:** Incident T2DM patients were identified in the UK CPRD database between 1 January 2000-31 August 2014 and followed for 5 years. Patients initiated on metformin monotherapy or pioglitazone were included. A SyMPTOMS analysis was performed, which used Hba1c≤7% after 90 days. CART was applied, with Hba1c level as the dependent variable and three explanatory factors: time to intensity control, Year and Year by TTTc interaction. For each iteration TTTc was assigned per subject (i.e. those patients with TTI before or after TTI cut-point). The CART analysis identified the optimal timing for treatment intensification post loss of glycaemic control as ≤544 days post the first Hba1c≤7% (rapid TTI). 50.7% of patients with rapid TTI achieved Hba1c target<7%, compared to only 14.0% in delayed TTI, despite rapid patients starting with higher average Hba1c levels (8.63%) than delayed patients (7.85%). The effects of rapid intensification caused immediate and maintained reduction in glucose levels, not observed in the delayed group. **CONCLUSIONS:** CART analysis identified the optimal timing for treatment intensification post loss of glycaemic control as ≤544 days. This analytical method could be used in future database studies to aid in group definition by treatment exposure and provide valuable clinical information for physicians.

**PM2**

**HOW TO CONDUCT ECONOMIC EVALUATIONS OF NEW TREATMENTS FOR ADVANCED CANCER WHEN OVERALL SURVIVAL DATA ARE NOT AVAILABLE? RESULTS FROM A SYSTEMATIC REVIEW**

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**OBJECTIVES:** Use of surrogate endpoints like progression-free survival (FFS) and time to progression (TTP) instead of overall survival (OS) in clinical trials for advanced cancer remains challenging from a health economic standpoint. This study assessed the use of surrogate endpoints in economic evaluations of new anticancer drugs and methodological approaches adopted when reliable OS data are unavailable. **METHODS:** A systematic literature review was conducted to identify economic evaluations of treatments for advanced cancer published between January 2004 and October 2013. Cost-effectiveness and cost-utility analyses expressed in terms of cost per life-year gained and cost per quality-adjusted life-year using a surrogate endpoint as an outcome measure were eligible. Characteristics of selected studies were extracted and comprised: population, treatment of interest, comparator, line-of-treatment, study perspective, and time horizon. Use of surrogate endpoints and methods adopted when OS data were lacking were analyzed. Two reviewers independently selected studies and extracted data. **RESULTS:** In total, 7,219 studies were identified and 100 fulfilled the eligibility criteria. Most included studies assessed the cost-effectiveness of a biological therapy (65%) in the first-line setting (56%) and in the context of advanced non-small cell lung cancer (24%) or advanced breast cancer (22%). Surrogate endpoints mostly used were FFS and TTP, accounting for 92% of included studies. OS data were unavailable for analysis in nearly 25% of economic evaluations. In the absence of OS data, studies most commonly assessed the cost-effectiveness of new agents as the time to progression, as the time to first failure, or as quality-adjusted survival time. Although several approaches are used, there is no consensus method to estimate the cost-effectiveness of new anticancer drugs in the absence of reliable OS data.

**PM3**

**SELECTION OF STATISTICAL APPROACH IN UNDERSTANDING THE ROLE OF CONTRAST MEDIA IN INPATIENT INTERVENTIONAL CARDIOVASCULAR PROCEDURES**

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