OBJECTIVES: Anemia affects about 25% of the world population and is the second main cause of incapacity worldwide. Clinical guidelines recommend upfront use of iron (oral or intravenous, IV) to correct iron deficiency anemia (IDA) instead of blood transfusion (BT). Nonetheless, utilization of BT is a common practice frequently associated with clinical complications and high economic burden. Our main objective was to estimate the impact of BT in hospital length of stay (LOS) and in intra- and extra-hospital mortality of patients with IDA. **METHODS:** This was a non-interventional, retrospective, single-center study. A convenience sample composed of 196 IDA patients treated with IV iron during 2013 was analyzed. Data was retrieved from patients' clinical and pharmacy records. Between-group comparisons were performed using non-parametric methods (Mann-Whitney-Wilcoxon and Kruskal Wallis). Logistic regression models were estimated to assess the odds of intra-hospital mortality. Kaplan-Meier survival estimates and Peto test were used to assess survival after hospital discharge (extra-hospital mortality). A 5% significance level was adopted. **RESULTS:** Here we present the results of the subset of 136 hospitalized patients, mean (SD) age of 68.6 (18.7) years and 50.7% males, corresponding to 148 hospitalization episodes. In 62.2% of these episodes at least 1BT was performed. Mean LOS was significantly higher in patients with BT (29.2 versus 15.3 days; p-value<0.0001). The odds ratio of dying during hospitalization in patients with >2BT was 3.73 (95%CI: 1.01-13.74) when compared to patients with ≤2BT, adjusting for age, sex, surgery or iron IV utilization. We observed a higher extra-hospital mortality in BT patients although not statistically significant (p-value=0.118), probably due to the low number of patients with follow-up data (n=33). **CONCLUSIONS:** Blood transfusions result in higher hospital length of stay and mortality. A better patient blood-management may improve patient outcomes and provide a more efficient use of health resources.

PSY104

CONVERGENT VALIDITY OF NEW DISEASE ASSESSMENT INSTRUMENTS (DAI) IN SYSTEMIC LUPUS ERYTHEMATOSUS (SLE) IN RELATION TO SLEDAI-2K

Narayanan S[1], Collins CE[2], Busch HM[3], Gaylis N[4], Hautamaki E[1], Chatham WW[5]
[1]*Ipsos Healthcare, Washington, DC, USA,* [2]*MedStar Washington Hospital Center, Washington, DC, USA,* [3]*Science and Research Institute, Inc., Jupiter, FL, USA,* [4]*Arthritis and Rheumatic Disease Specialties, Aventura, FL, USA,* [5]*University of Alabama, Birmingham, AL, USA*

OBJECTIVES: Current validated DAIs in SLE require disease expertise and/or involve complex scoring systems, and are often impractical for use in daily clinical practice. To address this, several new simplified DAIs in SLE are in different stages of development, including SLE Activity Tracking Evaluation Tool (LAST:14 items; includes medications, Physician Global Assessment (PhGA) 0-10 scale, Patient Global Assessment (PtGA:0-10), C3, C4 & anti-dsDNA lab measures), Clinical LAST (C-LAST:11 items; similar to LAST, without lab measures), Total Lupus Activity Score (TLAS:30 items; includes 15 physician clinical assessments and 15 patient self-assessment of symptoms), and Simple Disease Assessment for People with Lupus Erythematosus (SIMPLE:17 items; includes patient self-assessment of symptoms, Lupus Impact Tracker, steroid medication, C3, C4 & proteinuria). The primary objective of this analysis is to assess the convergent validity of these various tools as well as PhGA indices in relation to SLEDAI-2K. **METHODS:** This is a prospective real-world observational cohort study of SLE patients receiving standard care in 2 private and 2 academic sites in the US. Respective portions of DAIs are completed by physicians and patients at four timepoints: baseline/3mo/6mo/12mo. DAI completions: SLEDAI-2K:201; TLAS/SIMPLE: approx. 152; LAST/C-LAST:168. Pearson correlation coefficient (r) was computed comparing mean SLEDAI-2K score to other DAI scores at baseline. **RESULTS:** 201 SLE patients were enrolled (mean age:45yrs; female:91%; Black/African-American (AA):56%; Hispanic:10%). At baseline, mean SLEDAI-2K score was 3.4 (SD:3.2); each of the DAIs had statistically significant positive correlation with the SLEDAI-2K: LAST (r=0.566/p<0.0001), PhGA:0-10 scale (r=0.563/p<0.0001), SIMPLE (r=0.560/p<0.0001), PhGA:0-3 scale (r=0.553/p<0.0001), C-LAST (r=0.450/p<0.0001) and TLAS (r=0.348/p<0.0001). Analysis stratified by AA and non-AA status revealed similarly significant correlations between SLEDAI-2K and the other DAIs. **CONCLUSIONS:** In this cross-sectional analysis, each of the studied DAIs appeared to have convergent validity with the SLEDAI-2K. Further analyses with longitudinal data will assess additional psychometric properties of the instruments.

PSY105

THE RISE OF ORPHAN DRUGS IN EUROPE VS THE UNITED STATES: COMPARING ORPHAN DRUG DESIGNATIONS BETWEEN THE EMA AND FDA

Mildred M, Lee S
Boehringer Ingelheim Ltd, Bracknell, UK

OBJECTIVES: Developing innovative treatments for rare diseases is intrinsically difficult due to small patient population sizes and the scarcity of high quality evidence. Orphan drug designation is attributed to drugs which have been developed to treat rare diseases; however, the European Medicines Agency (EMA) and US Food and Drug Administration (FDA) define rare diseases differently. The objective of this study was therefore to investigate the number and type of orphan drug designations across the EMA and FDA. **METHODS:** Data on authorised drugs awarded orphan status by the EMA and FDA is freely available and was obtained from their respective websites. Authorised orphan drugs were analysed dating back to the first EMA orphan drug designation in 2002. **RESULTS:** The EMA designated 82 drugs orphan status, whilst the FDA designated 170 drugs orphan status. An upward trend in the number of drugs designated orphan status by the EMA was observed, whilst a downward trend was present in FDA orphan designations. The distribution of orphan drugs across WHO Anatomical Therapeutic Chemical (ATC) classifications appears to vary, for instance: 18% of EMA authorised orphan drugs were in the alimentary tract and metabolism group (ATC code A), compared to just 6% of FDA orphan drugs. Conversely, orphan drugs in the blood and blood forming organs category (ATC code B) comprised 2% of EMA authorised orphan drugs compared to 12% with the FDA. **CONCLUSIONS:** The EMA has designated half the number of drugs orphan status compared to the FDA however the number of authorised drugs designated orphan status appears to be on the rise in Europe. The pattern of orphan drugs across ATC groups appeared to differ between the EMA and FDA suggesting the regulatory bodies may have different preferences with regard to research and development in the various therapeutic fields.

PSY106

ANALYSIS OF GOVERNMENT PROCUREMENTS OF MEDICINES FOR RARE DISEASES IN RUSSIA

Fedyaev D[1], Fedyaeva VK[2], Omelyanovskiy VV[2]
[1]*Financial Scientific Research Institute of the Ministry of Finance of Russia, Moscow, Russia,* [2]*The Russian Presidential Academy of National Economy and Public Administration, Moscow, Russia*

BACKGROUND: In Russia all drugs for state medical organisations and programs are purchased through public tenders in order to enhance competition, decrease price and prevent corruption. Drugs for rare diseases (DRD) are often produced by the only manufacturer thus being monopolistic. There is lack of information about the effectiveness of tender procedures for DRD in real life. **OBJECTIVES:** To assess the competition in public procurement of DRD and effectiveness of tender procedures. **METHODS:** The analysis was conducted on the basis of 1499 selected trade procedures on DRD procurement in 2013. We evaluated the proportion of procedures that have passed with the only one participant, the level of price reduction resulted from trading, the amount and volume of purchases of DRD in the subjects of the Russian Federation. Data on drug procurement procedures were obtained from the official website of state purchases www.zakupki.gov.ru. Data collection and processing were performed using the program Microsoft EXCEL and Statistica 6.1. **RESULTS:** Total amount of spending for DRD in 2013 was €192.23 million, with declared procedures on €197.93 million. Competition was absent in 592 (74.47%) procedures of monopolistic drugs procurement. 75% of tenders were performed without reducing the price. There were €5.69 million savings generated by purchases (3% of the amount of declared procedures). **CONCLUSIONS:** Tender procedures used for the procurement of monopoly drugs are ineffective because of the lack of competition; distributors are not interested in price reduction at noncompetitive procedures.

PSY107

COMPARATIVE ANALYSIS OF THE REIMBURSEMENT LANDSCAPE OF ORPHAN DRUGS ON AN INTERNATIONAL LEVEL

Nazha S
University of Montreal, Montreal, QC, Canada

OBJECTIVES: The objective of this study is to analyze and compare the recommendation made by several countries for orphan drugs such as Canada, Scotland, Australia, France and England.The study will try to address solutions and innovative ways to evaluate orphan drugs from an economic perspective and try to propose the hallmarks of an ideal policy to be implemented. **METHODS:** The Orphanet website was screened to find orphan drugs with national designations.237 orphan drugs indication were retrieved and 36 technologies were included in the study and stratified by therapeutic areas.In total, 180 recommendations were analyzed for the 5 included HTA agencies. Each recommendation was analyzed for the following parameters: Listing, clinical recommendation, economic recommendation, QALYs and rarity of disease taken in consideration. **RESULTS:** Scotland (88%) was the country that took the most in consideration the orphan designation of drugs, followed by France (74%) and Australia (45%). In addition, France and Scotland were the 2 countries that had the most positive listings (with criteria or without). The clinical benefit was demonstrated in multiple drugs throughout the different agencies. However, the economic part was very heterogeneous. Scotland issued a lot of positive economic recommendations (60%); however countries such as Canada had a very low percentage of economic being accepted (19%). QALYs did not seem to correlate with the type of listing.Countries that had the most health policies regarding orphan drugs had a better rate of orphan drugs being accepted for reimbursement. **CONCLUSIONS:** Though there are certain similarities across countries with regards to the HTA of orphan drugs, significant differences exist, which are likely to continue to cause variations in access to orphan drugs across countries. Positive listing by CADTH was one of the lowest between 5 countries and the economic recommendations were the most negative in Canada.Seperate funding for orphan drugs and specific clinical assessment should idealy be implemented throughout the world.

PSY108

SIMILARITIES AND DIFFERENCES IN ORPHAN DRUG REIMBURSEMENT IN 16 EUROPEAN COUNTRIES

Falk K, Brown A, Faria-Billinton E, Martensson M, Murray G, Nwogu G, Pritchett L, Stoor L, Wiesinger A
Abacus International, Manchester, UK

OBJECTIVES: Since the introduction of the European rare diseases policy in 2000, 82 drugs have received orphan status. As the orphan drug market is rapidly expanding, this number is expected to increase. The research objective was to identify how European countries manage the increase in available orphan drugs and whether there are similarities or differences in orphan drug reimbursement policies across Europe. **METHODS:** We assessed orphan drug policies in 16 European countries, grouped into four archetypes: health insurance markets (Austria, Belgium, France, Germany, The Netherlands, Switzerland); devolved markets (Italy, Spain); health economic markets (Ireland, Portugal, UK); Nordic country markets (Denmark, Norway, Sweden). Policies were assessed considering five key factors: key stakeholders; treatment setting; evidence requirements; reimbursement processes; source of funding. **RESULTS:** In all country archetypes, except Nordic markets, the reimbursement assessment of orphan drugs used for in- and out-patient care is the same. All archetypes, except health insurance markets, follow the same processes for orphan and non-orphan drugs. Although in Scotland and England, drugs classified as ultra-orphan go through specialised processes. In devolved markets, economic and clinical data are considered; in health insurance markets, economic data are only considered under certain circumstances in some countries. Nordic countries have fewer evidence requirements than other archetypes. In both health economic and health insurance markets, orphan drug funding varies with treatment setting.