baseline to months 1 and 3. **RESULTS:** All patients treated with AVXS-101 survived event-free to 24 months. A rapid increase in mean CHOP-INTEND scores of 9.8 (n=12; SD=3.91) points as early as 1 month and 15.4 (n=12, SD=6.36) points at 3 months post-dose were observed. **CONCLUSIONS:** AVXS-101 appears to induce a rapid and significant improvement in motor function as measured by CHOP-INTEND score relative to nusinersen, consistent with its pharmacological mechanism of action designed to promptly restore SMN expression in motor neurons with a single dose administration. Advances in the understanding of SMA, currently available and investigational pharmacologic treatments, and the gene replacement therapy, AVXS-101, underscore the importance of early diagnosis and treatments with a near-immediate onset of action to maximize clinical improvements.

PSY19

USING A BAYESIAN NETWORK META-ANALYSIS (NMA) TO COMPARE FERRIC MALTOL TO TREATMENTS FOR IRON DEFICIENCY AND IRON DEFICIENCY ANAEMIA EXCLUDING CHF AND CKD PATIENTS

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OBJECTIVES: Many IDA treatments have not been directly compared in head-to-head clinical trials. Oral ferric maltol (Feraccru) is currently indicated in the UK in adults for the treatment of iron deficiency. The objective of this study was to compare ferric maltol to other available treatments for ID and IDA including mixed populations and those in patients with IBD, HUB, pregnancy, post partum and cancer, but excluding CHF and CKD, identified through a systematic literature review (SLR) and using a Bayesian NMA. **METHODS:** A comprehensive SLR was performed to identify all RCTs of IDA treatments. 7 comparators to ferric maltol were identified and included: infused ferric carboxymaltose, iron isomaltoside, iron sucrose, iron dextran and ferumoxytol; oral iron and placebo. A Bayesian ("mixed evidence") NMA was performed using a random effects model in WINBUGS comparing Hgb change in patients treated with ferric maltol and other available treatments for IDA excluding CHF and CKD. Both the 12-week measurement (randomised phase) and 64-week measurement (extension phase) from the Phase III RCT were assessed for ferric maltol. **RESULTS:** 36 trials reporting Hgb change were identified. After adjusting for baseline Hgb using a meta-regression of study outcomes, ferric maltol showed a significant favourability over all comparators when using the 12-week and 64-week measurement with mean Hgb changes from placebo: ferric maltol (2.75 and 3.59 g/dL), oral iron (1.04 g/dL), ferric carboxymaltose (1.93 g/dL), iron isomaltoside (1.27 g/dL), iron dextran (1.61 g/dL), iron sucrose (1.68 g/dL), and ferumoxytol (2.12 g/dL). **CONCLUSIONS:** To our knowledge, this is the first study comparing IDA treatments excluding CHF and CKD patients using a mixed evidence approach. Based on the NMA, ferric maltol showed favourability over the other identified comparators. Results were limited by the lack of informative priors.

PSY20

REAL WORLD TREATMENT PERSISTANCE OF GOLIMUMAB IN THE MANAGEMENT OF IMMUNE-MEDIATED RHEUMATIC DISEASES (IMRDS) IN EUROPE: A SYSTEMATIC LITERATURE REVIEW (SLR)

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OBJECTIVES: To gather evidence of the real-world persistence on subcutaneous golimumab in the routine care of patients diagnosed with IMRDs in Europe. **METHODS:** A SLR was conducted in Medline and Embase via Ovid (1/1/2009-30/4/2018), EULAR and ACR/ARHP 2017 Conference proceedings, with double review and extraction. Articles were excluded if not in English, not in Europe, IMRD different from rheumatoid arthritis (RA), Ankylosing Spondylitis (AS), or Psoriatic arthritis (PsA); or based on clinical-trial data. **RESULTS:** Following deduplication, 463 titles/abstracts were screened and 422 excluded due to population (N=29), exposure (N=175), outcomes (N=95), study design (N=112), not European (N=10). Following full-text review, 31 records were included for extraction of which 3 were duplicate studies.. Data extracted from 18 unique studies in Finland, France, Italy, Greece, Portugal and Sweden (10 in RA, 5 in AS, 7 in PsA and 5 in mixed population) reveal that treatment persistence at 6, 12, 24 and 36 months varied between 69-97%, 53-84%, 37-79% and 29-76% respectively. Only 2 studies reported mean survival time in months (95% CI): Iannone for RA 19.3 (17.7-21.0) and for PsA: 19.0 (17.8-20.2), and Dalen for mixed IMRD 18.1 (15.2-24.1). All studies accounting for differences in line of therapy found that persistence was longer among naïve than experienced patients. Definition of non-persistence differed: Iannone allowed for 31-days gaps, Dalen and Svedbom for 60 and Mourao and Fautrel for 90. Comparative analysis was done in 12 studies and showed golimumab was superior to adalimumab (N=10), to certolizumab pegol (N=5), to etanercept (N=5), and to infliximab (N=1). **CONCLUSIONS:** In EU real-world settings, golimumab has comparatively higher persistence compared to other anti-TNFs. The current study revealed a certain degree of heterogeneity in findings that may be explained by underlying differences in the healthcare systems of the countries and between the populations under study.

PSY21

DELETERIOUS EFFECT OF BLOOD TRANSFUSIONS IN A COHORT OF ANAEMIC INPATIENTS

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OBJECTIVES: Almost 50% of anaemic cases are due to iron deficiency, in which guidelines recommend the administration of intravenous (IV) iron. Blood transfusions should be left for critical patients, as they provide faster haemoglobin (Hb) levels recovery, however they have also been associated with deleterious clinical outcomes. We aimed to characterize the clinical impact of blood transfusions in a population of hospitalised anaemic patients treated with IV iron. **METHODS:** This was a retrospective cohort study. Patient records from a general Portuguese Hospital, with at least one inpatient administration of iron sucrose in 2014-2015, were reviewed. Adult anaemic patients with at least one Hb evaluation before and after the administration of IV iron were included. Endpoints assessed comprised Hb level (baseline and 8 weeks after), transfusions, length of stay (LOS) and inpatient mortality. Statistical analysis included generalized linear models, using a 5% significance level. **RESULTS:** Data was collected for 878 patients (61.4% female; mean age (SD): 63.9 (20.6) years) and 945 IV iron administration episodes. Average baseline Hb level was 8.4 g/dl and increased to 10.3 g/dl at week 8. A total of 16.8% of patients corrected their anaemia by week 8. Blood transfusions were performed in 58.0% of the episodes. Having a blood transfusion was not significant for anaemia correction (OR: 0.9; 95% confidence interval [95%CI]: 0.5-1.7). Moreover, receiving blood transfusions was significantly associated with longer LOS (OR: 1.2; 95% CI: 1.1-1.3) and with a 2.6 fold increase in the risk of inpatient mortality (95%CI: 1.6-4.4), when controlled for age and sex. **CONCLUSIONS:** Adding blood transfusion to IV iron therapy did not increase anaemia correction, but further deteriorated other patient health outcomes.

PSY22

A REVIEW ON EPIDEMIOLOGY, RISK FACTORS AND TREATMENT PATTERNS IN MODERATE AND SEVERE PAINFUL DIABETIC NEUROPATHY AND POST-HERPETIC NEURALGIA

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OBJECTIVES: The aim of this review is to identify epidemiology, treatment pattern and risk factors for moderate and severe pain in painful diabetic neuropathy (PDN) and post-herpetic neuralgia (PHN). **METHODS:** A narrative review was conducted through searching Embase®, MEDLINE® and Cochrane from 1996 to April 2018 through Ovid® platform. The epidemiology, risk factors and treatment pattern are summarised for moderate and severe PDN and PHN. **RESULTS:** Of 1,430 citations reviewed, 11 studies were identified (PDN:7, PHN:4). Prevalence of severe-PDN in diabetic patients was 5.2% in the US. In UK, overall prevalence of PDN was 26.4%; of this the proportion of moderate-PDN was 47% and severe-PDN was 33%. Incidence of moderate-to-severe PHN increased with age, 9.6(60-69 years), 12.9(70-79 years) and 12.6(≥80 years) per 1000 person-years; for >60 years the incidence of severe-PHN was ~7%(at 3 months) and ~3%(at 12 months). Risk factors for severity of PDN were female, race, diabetic duration, thermal sensory loss and nephropathy. Risk factors for PHN were acute severe pain during herpes infection and age of patient. Analgesic use was greater in moderate-to-severe PDN than mild-PDN. Opioids were the most frequently prescribed medication in severe-PDN, followed by anticonvulsants and NSAIDs. As PDN severity increased, the proportion of prescription of ≥1 medications increased significantly (P=0.0004). Proportion of patients taking ≥2 pain medications increased with severity of PDN (mild: 22.1%, moderate: 52.2%, severe: 71.6%) and also increased mean number of pain medications (mild: 0.9, moderate: 1.5, severe: 2.2). With severity of PDN use of opioids increased (mild: 9.1%, moderate: 31.6%, severe: 53.1%). In both moderate-PHN and severe-PHN pregabalin was the preferred choice of drug, followed by opioids. **CONCLUSIONS:** This review provides spectrum of moderate-to-severe PDN and PHN in terms of epidemiology, risk factors and treatment patterns. In both moderate and severe PDN the prescription of pain medications increased with severity of pain.

PSY23

AGALSIDASE ALFA AND AGALSIDASE BETA FOR TREATMENT OF FABRY DISEASE

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OBJECTIVES: A review of the literature was performed to evaluate whether agalsidase alpha and agalsidase beta are effective and safe for the treatment of Fabry disease. **METHODS:** A review of the literature was conducted through a search in the Medline, Embase, Lilacs, Center for Reviews and Dissemination (CRD) and Cochrane databases, without restrictions. Manually, publications of the National Institute for Health and Care Excellence (NICE) and national and international guidelines have been consulted. Quality of evidence was assessed by GRADE (Grading of Recommendations Assessment, Development and Evaluation). Were estimated the costs of treatments. **RESULTS:** The search strategy recovered 2,337 articles and 10 were selected for evaluation. A Cochrane review of 2017 was used as the basis. Outcomes evaluated: renal complications, agalsidase alfa 15.3% [95% CI: 0.048, 0.303; p = 0.0005]; agalsidase beta 6% [95% CI 0.04, 0.07; and untreated patients 21.4% [95% CI: 0.1522; 0.2835 p <0.0001]; cardiovascular complications, agalsidase alfa 28% [95% CI: 0.07, 0.55; p <0.0001]; agalsidase beta 7% [95% CI 0.05, 0.08;]; and untreated patients 26.2% [95% CI: 0.149, 0.394; p <0.0001]; and for cerebrovascular complications, agalsidase alfa 11.1% [95% CI 0.058, 0.179; p = 0.0024]; agalsidase beta 3.5% [95% CI: 0.024, 0.046 p = 0.4209]; and untreated patients 18.3% [95% CI: 0.129; 0.245; p <0.0001]. All-cause mortality rate was higher in untreated patients (10.8%), followed by agalsidase alfa (9%) and agalsidase beta (4.4%), there was no significant difference. The treatment of an adult (70 Kg) for a month will cost about USD 8,109.00 for agalsidase alfa and about USD 10,243.00 for agalsidase beta. The quality of evidence was assessed as very low. **CONCLUSIONS:** The balance between the limited quality of evidence and the benefits demonstrated was

favorable to both technologies. The differences in effect favored agalsidase beta. The cost of agalsidase alfa was about 21% lower.

PSY26
OPIOID PRESCRIBING IN A SOUTH AFRICAN MEDICAL INSURANCE SCHEME SETTING
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OBJECTIVES: Opioid analgesics are reported to be overprescribed in various parts of the world. Since data for South Africa in its entirety is not available, studies on available electronic dispensing databases can add a valuable insight into opioid prescribing patterns in the country. The primary aim of the study was to determine which opioids are prescribed in a medical insurance scheme setting in South Africa. **METHODS:** A retrospective drug utilisation study was conducted on a South African medical insurance administrator database for 2017. The database contained 3 898 535 records for medicine, medical devices and procedures. All products in ATC subgroup N02A (opioids) were extracted and analysed. **RESULTS:** A total of 102 255 opioids were dispensed to 33 249 patients (72.47% male patients). The average age of patients was 40.80 (SD=12.26) years. Patients received on average 3.08 (SD=5.57) opioid prescriptions over the year. Most opioids were dispensed by private hospitals (46.45%), followed by general medical practices (43.38%) and pharmacies (9.74%). Dihydrocodeine and paracetamol (N02AJ06, 45.09%) were dispensed the most, followed by tramadol (N02AX02, 28.99%), and tramadol and paracetamol combined (N02AJ13, 9.67%). These three agents together accounted for 83.75% of all opioid analgesics dispensed. Pethidine (N02AB02) accounted for 4.74% and morphine (not in combination) (N02AA01) for 4.48%. A total amount of R2 560 040.07 was claimed by patients, of which R2 004 941.77 was reimbursed. Generic substitution is compulsory in South Africa. The average amount claimed per opioid was only R25.04 (SD=R56.72). Tramadol was, for example, dispensed as its originator, in addition to seven branded generics. **CONCLUSIONS:** Opioid prescribing seemed to be in line with the South African guidelines, and overuse could not be detected. A follow-up study focusing specifically on codeine is recommended, since South Africa is often portrayed in the media as one of the codeine hotspots in the world.

PSY27
SPINAL MUSCULAR ATROPHY: A REVIEW OF EPIDEMIOLOGY, BURDEN AND UNMET NEEDS
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OBJECTIVES: Spinal Muscular Atrophy (SMA) is a rare and severe neuromuscular autosomal recessive disorder characterized by degeneration of alpha motor neurons in spinal cord. It is the most frequent genetic cause of infant death. The objective of this literature review was to understand the epidemiology, humanistic and economic burden of disease and unmet medical needs in SMA Type 1, 2 and 3. **METHODS:** A literature search of Medline®, Cochrane Library and Embase® was conducted using disease related search terms and outcomes of interest. A hand search was also undertaken to ensure completeness. Articles published in English worldwide up to 16 April 2018 were included. **RESULTS:** 413 articles were retrieved from which 37 were included in this study. Estimated SMA (all types) incidence ranged from 5.1-27.7 per 100,000 live births, with highest incidence rates reported in Europe and Type 1 SMA being the most common sub-type. The worldwide registry-based prevalence estimates of SMA (all types) ranged from 0.01-4.11 per 100,000 persons. Studies estimating health-related quality of life (HRQoL) in SMA demonstrated a strong deterioration in HRQoL in SMA Type 1, 2 and 3 patients, and their caregivers relative to the general population. Limited cost of illness studies in SMA (all types) estimated annual per capita costs (both direct and indirect costs) as high as €70,566 in Europe to $184,647 in United States with one study noting the high costs compared to those without SMA. Substantial unmet needs exist in the diagnosis, cure, and supportive care of SMA patients and there is a dearth of robust treatment guidelines. **CONCLUSIONS:** SMA incurs significant healthcare costs and imposes a substantial economic and humanistic burden on SMA patients and their families. Severe burden associated with SMA necessitates concerted efforts towards developing disease modifying therapies and enhanced supportive mechanisms for patients.

PSY28
EPIDEMIOLOGY OF RPE65 GENE MUTATION RELATED INHERITED RETINAL DYSTROPHIES: A SYSTEMATIC LITERATURE REVIEW
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OBJECTIVES: Inherited retinal dystrophies (IRDs) are a heterogeneous group of diseases resulting from over 220 genetic mutations and are generally characterized by retinal degeneration ultimately leading to severe visual impairment or blindness. The aim of this study was to collect epidemiology data on RPE65 mutation associated IRDs, most often reported as Leber Congenital Amaurosis 2 (LCA2) and Retinitis Pigmentosa 20 (RP20). **METHODS:** Publications were reviewed up to March 2018 in MEDLINE, Embase, and Cochrane. Data collected included the prevalence and incidence of RP and LCA, and the proportion caused by RPE65 mutations within each disease. A quality assessment was conducted, and low ranked studies were excluded from the analysis. **RESULTS:** From the 2,571 citations screened, 49 studies presented relevant epidemiology data. The prevalence of LCA and RP ranged between 1 in 81,000 and 1 in 33,000, and between 1 in 8,357 and 1 in 3,454 respectively. There is a scarcity of incidence data for RP (Korea has 1.64 in 100,000 cases per year and US has 0.6 in 100,000 cases per year) and no data sources were found for LCA. The proportion of RPE65 mutations in LCA and RP patients varied from 1.0% to 16.6% and 1.0% to 6.0%, respectively. Regarding LCA, the proportion of RPE65 mutations ranged between 2.38% and 16% in Europe, 3.8% and 16% in the US, and 1% to 16.6% in the rest of the world. The proportion of RPE65 mutations in RP patients ranged between 1.8% and 6% in European countries, while in the US, the proportion ranged between 1.0% and 3.0%. **CONCLUSIONS:** Data on prevalence and incidence of RPE65 is scarce but indicates significant variation among countries. Further research is needed to generate consistent epidemiology figures for RPE65 mutation associated IRDs.

PSY29
REAL-WORLD DISCONTINUATION OF BIOLOGICAL TREATMENTS IN MODERATE-TO-SEVERE PLAQUE PSORIASIS OVER THE FIRST 5 YEARS OF TREATMENT – A LITERATURE REVIEW OF DRUG SURVIVAL
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OBJECTIVES: During the past decade, drug survival has become a real-life measurement for the success of treatments for plaque psoriasis. The objective of this study was to synthesize and quantify published evidence on real-world drug discontinuation for biological treatments used in moderate-to-severe plaque psoriasis over the first 5 years of treatment. **METHODS:** A literature review was conducted in May 2018. The search was limited to the last 5 years. Case review studies with small study samples and single-center cohort studies (< 300 patients) were excluded. Drug survival rates at specific timepoints were extracted or calculated from the included studies and weighted according to the sample size of each study. For each timepoint, a weighted average drug survival rate was calculated, which was then transformed into 4-week discontinuation rates. **RESULTS:** The identified studies reported drug survival rates for adalimumab (ADA), etanercept (ETA), infliximab (INF), ustekinumab (UST), and secukinumab (SEC). ETA and SEC had the longest and shortest follow-up time of 10 and 1.5 years, respectively. In the first year, 4-week discontinuation rates were 2.6%, 2.4%, 2.4%, 2.2%, and 1.2% for ETA, INF, ADA, SEC, and UST, respectively. For each year, a declining trend in discontinuation rates was seen for ETA, ADA, and UST (range: 0.5-0.7% at year 5). Similarly, a declining trend was observed for INF except for year 5. The discontinuation rates for SEC at year 1 and 1.5 were high at comparable levels (2.3% vs. 2.2%). **CONCLUSIONS:** This study indicates a declining trend in discontinuation rates for ETA, ADA, and UST over time, and for INF for all years except the last. Discontinuation rates for SEC are still uncertain due to the lack of evidence, however, the findings indicate early signs of high discontinuation for SEC. Further high quality observational studies are needed to confirm this.

PSY30
INCIDENCE AND PREVALENCE OF NONTUBERCULOUS MYCOBACTERIAL LUNG DISEASE IN US MEDICARE, 2008-2015
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OBJECTIVES: Previous research has reported nontuberculous mycobacterial lung disease (NTMLD) prevalence of 47 per 100,000 among US Medicare beneficiaries ≥65 years in 2007, with an average increase of 8.2% annually between 1997 and 2007. In this study, we have evaluated NTMLD incidence and prevalence in Medicare between 2008 and 2015. **METHODS:** Patients diagnosed for NTMLD with an ICD9 031.0 were identified from the Medicare database (N≈30 million yearly), not including the Part C portion. Individuals who incurred at least 2 medical claims ≥30 days apart between 2007-15 were considered as a positive NTMLD case, yielding 58,294 patients. All individuals fulfilling the case definition each calendar year were considered as prevalent cases. Incident cases included those meeting the case definition each year and having no medical claim for NTMLD in the prior year. **RESULTS:** Patients with NTMLD in the US Medicare database had a mean age of 74 (±10) years. Sixty-nine percent were women and 89% white. Yearly NTMLD incidence increased from 20.7 (95% CI: 20.2-21.3) in 2008 to 28.1 (27.5-28.7) in 2013 per 100,000 Medicare beneficiaries and leveled to 27.6 (26.9-28.2) in 2014 and 25.9 (25.3-26.5) in 2015 per 100,000. Yearly NTMLD prevalence increased from 41.6 (40.9-42.3) in 2008 to 63.1 (62.2-64.0) in 2015 per 100,000 Medicare beneficiaries. In 2015, incidence was 28.1 vs 14.7 per 100,000 Medicare beneficiaries ≥65 years vs those <65 years, respectively. Prevalence was 70.2 vs 27.9 per 100,000 Medicare beneficiaries ≥65 years vs those <65 years, respectively. Incidence and prevalence were 33.9 vs 16.0/100,000 and 86.2 vs 34.6/100,000 in women vs men and 41.1 vs. 27.6/100,000 and 89.4 vs. 68.7/100,000 in beneficiaries of Asians vs White origin. **CONCLUSIONS:** In US Medicare beneficiaries, NTMLD incidence increased from 2008 through 2013 and leveled off in 2014 and 2015, while NTMLD prevalence continued to rise through 2015.

PSY31
COMPARISON OF THE ESTIMATED PREVALENCE OF DIAGNOSED HOMOCYSTINURIA AND PHENYLKETONURIA IN THE UNITED STATES
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OBJECTIVES: Homocystinuria (HCU) may be underdiagnosed at birth due to inadequately sensitive newborn screening. This study estimated the prevalence of diagnosed HCU in the United States (U.S.) population across age groups, compared with that of diagnosed phenylketonuria (PKU) in similar age groups. **METHODS:** In the IBM MarketScan® insurance claims database (MSN) (1/1/2010–12/31/2016), patients with a diagnosis of PKU were identified using standard diagnostic coding systems (ICD-9 270.1 or ICD-10 E70.0), while those with a diagnosis of HCU were identified using solely the more specific ICD-10 code ((E72.11). U.S. census data