OBJECTIVES: In most developed countries, policies incentivize manufacturers to invest in developing orphan drugs (OD) as the return on investment is unlikely. The objective of this study was to assess the overall performance of OD policies on development and revenue of OD. METHODS: We identified on FDA website the yearly number of OD approved. We gathered revenue associated to OD from companies and analyzed its perspective in databases, such as the Datamonitor, and companies’ websites and annual reports. We reviewed published OD forecasts. RESULTS: In the US, the number of yearly approved drugs grew from less than 5 in the 1980s, to more than 50 in the last 5 years. In 2017, the turnover of OD business reached US$ 83 billion. The growth of this part of the pharmaceutical market is very fast with a sales forecasting of US$ 127 billion in 2018 (153%) compared to 2012. In 2010, 65 products generated more than US$ 100 million revenue, 13 more than US$600 million, and 24 more than US$ 1 billion. Seven leading companies generate from US$9.9 to 1.7 billion yearly revenue with OD portfolio. Factors related to rare diseases such as severity of disease, socioeconomic status, rarity, and adherence. Rarity had a relative weight of 0.02, the most important factors in the top 5 for the majority of our subjects. Our study found that the most important factors Canadians feel should be used in decision making, the relative weight of these values, and whether there is an impact on the access of the population to DRD.

PSY62 TRENDS OF ORPHAN DRUGS APPROVALS OVER TIME IN THE UNITED STATES Thokagevist K1, Dorey J1, Tessella F2, Méruazat C3, Thiuri M4
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OBJECTIVES: To understand real-world treatment patterns of CLL, the use of agents/regimens across all lines of therapy was evaluated in EU5 countries during the winters 2010-2013. The IPSOS Health care projected rituximab usage for treatment of CLL based on patient diaries. Representative samples of oncologists (N = 509) completed forms directly from reviews of patients’ charts and provided information on how they manage patients in everyday practice. A total of 104 physicians provided CLL patient information across first-, second-, and third-line CLL treatments. Important data such as age, comorbidities, and therapy goals were also abstracted. RESULTS: According to the most recent annually projected data, a total of 37,119 patients with CLL are estimated to have been treated in the EU5 with any anticancer drug therapy between July 2012 and June 2013. The majority of these patients (23.1%) are estimated to have received fludarabine-cyclophosphamide-rituximab followed by bendamustine-rituximab (17.4%), chlorambucil (16.2%), fludarabine (7.2%), single-agent rituximab (8.8%), and chlorambucil-rituximab (5.6%). The data show higher rituximab monotherapy share in later lines of therapy for CLL (first-line, 0.3%; second-line, 8.2%; third-line or more, 22.1%) irrespective of age. Approved monoclonals (alemtuzumab, 2%, and ofatumumab, 4%) are not widely used in relapsed CLL. Our data also indicates that single-agent rituximab is now more commonly used in clinical practice than chlorambucil (21.1% vs 9.2%, respectively) as third-line treatment and above in patients with CLL. Among patients with CLL (using this subset of patients, diabetes is the most common comorbidity (13%)). CONCLUSIONS: Rituximab is utilized either as a combinational or single-agent regimen across all lines of therapy in CLL and more commonly used in the setting of first-line treatment. No standard of care for relapsed elderly or more comorbid CLL patients. This data may serve as real-world outcomes which can help in designing appropriate or interpreting outcomes of clinical trials programs.

PSY64 THE ASSOCIATION BETWEEN CONTINUITY OF CARE AND POTENTIALLY INAPPROPRIATE CONCOMITANT MEDICATION IN CONTINUOUS NON-STEROIDAL ANTI-INFLAMMATORY DRUG USERS Lin V1, Haoih C2, Kao FS1
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OBJECTIVES: The development of orphan drugs (OD) has substantially increased in the last decade. The objective of this study was to describe the trends in OD approvals from 2002 to present in the United States. METHODS: We selected all OD approved between 2002 and 2013 from the FDA Orphan Products database. We extracted the ODs characteristics, such as therapeutic area, prevalence of disease, current treatment, and OD approvals proportion in therapeutic areas, prevalence of disease [rare vs. ultra rare (≤500 patients/million)] and duration of substance patent. Number of orphan indications by drug was also assessed over the last decade. RESULTS: Since 2002, 1,433 OD were approved with 162 added in the last 5 years, of which 32 (8%) were granted an orphan status. The total number of approved indications has increased by 76% when comparing 2011-2013 period to 2008-2010 period. Oncology was found to be the major indication area, accounting for 36% of overall indications, followed by gastroenterology (18%) and dermatology (13%). Across all indication areas, OD approvals proportion in oncology remained relatively stable over time, while other areas appeared randomly distributed over the years. About 12% of OD was approved in 2 or more indications, with a stable number over time. Half of approved OD displayed less than 7 years of patent protection, or the period of exclusivity granted by the Orphan Drug Act. Ultra OD indications represented 27% of overall approvals, with 41% of ultra orphan indications approved in the last 3 years. CONCLUSIONS: This analysis confirmed the increase of ODs in the last decade, and particularly in oncology area between 2002 and 2013. The 7 years exclusivity is critical for half of the products. No specific trends could be captured in the studied period.