Mean numbers of patients by recommendation type: Standard = 7,838; Special = 3,935; Research Only = 2,423. There is also a clear trend over time: Standard recommendations decrease for all IPGs from 63% in 2003-2009 to 40% in 2014-2018; and the evidence threshold for Standard recommendations increases over time from 56% based on systematic reviews and/or RCTs in 2003-2009 to 85% in 2014-2018; mean numbers of patients per Standard recommendation also increase from 2,002 to 6,098 over this period.

Conclusions. Higher levels evidence and numbers of patients increase the likelihood of the most positive recommendation. However, this evidence might still lack sufficient quality or certainty to answer a policy question. The evidence threshold to achieve a Standard recommendation has also increased markedly over time. As with other NICE committees, factors other than cost and perceived hierarchies of evidence clearly act as drivers of decisions.

VP46 German Claims Data In Rare Disease HTA: Diffuse Large B-cell Lymphoma

Aljoscha Neubauer (aljoscha.neubauer@ifgph-muenchen.de), Susanne Guthoff-Hagen, Jacob Menzler, Carsten Schwenke, Markus Rueckert and Axel Boehnke

Introduction. In rare disease areas representative data are scarce. Routine sick fund claims data provide a meaningful and reliable base for the in- and outpatient treatment landscape. This real-world data (RWE) from Germany was used to describe treatment patterns for Diffuse Large B-cell Lymphoma (DLBCL), the most frequent and aggressive non-Hodgkin lymphoma type in adults.

Methods. Claims data from several sick funds of 4.8 Million insured were analyzed. Diagnosis of non-follicular Lymphoma (C83) was confirmed in 2.178 patients, DLBCL (C83.3) in 819 patients. The analysis was age- and gender-adjusted, observational period was 2014 and 2015. Treatments were analyzed for hospitalization and medication based on ATC-Code, Pharma Central Number and coded diagnoses (per ICD).

Results. Mean age of DLBCL patients was 60.3 years, with two peaks at 50-54 and 70-74 years. Total costs for patients with DLBCL averaged 25.048 EUR versus 1.259 EUR in healthy insured. Charlson comorbidity index (CCI) of 4.58 indicates clinical relevance and severity. Comorbidities included several psychiatric diagnoses such as depression in every fifth patient. Mean 3.2 hospitalizations with average 31.5 hospital days were observed in DLBCL patients. Forty-seven percent of patients during observational time-frame did not receive oncological treatment, including relapsed / refractory patients. Only few patients received stem cell transplantation (2.6 percent) or radiation (3.9 percent). Most pharmacological treatments were Rituximab (RTX) + CHOP (57 percent), followed by RTX mono therapy (25 percent) or RTX in combination with Bendamustine (8 percent).

Conclusions. Despite limitations in sick fund claims analyses, these provide a reasonable database for rare diseases. They allow standard treatment pathway- and longitudinal analyses. All DLBCL patients frequently required hospitalization and generated significant costs. A high unmet medical need exists for treatments other than palliative care, especially for a tolerable and effective outpatient therapy in elderly relapsed / refractory DLBCL.

VP47 Secondary Prevention For CV Disease: Population And Outcomes Using RWD

Paolo Sciattella (paolo.sciattella@uniroma2.it) and Francesco Mennini

Introduction. The study goal was to estimate prevalence of population in secondary prevention for Atherosclerotic Cardiovascular Disease (ASCVD) stratified by the pharmacological treatment and related outcomes using Health Information Systems (HIS).

Methods. From HIS of Marche and Umbria Regions (1.8 millions of inhabitants) which collect information related to hospitalizations, drugs prescriptions, outpatient visits and results of laboratory tests, we identified all patients aged ≤ 80 years with one or more hospitalization with DRG related to Acute Coronary Syndrome, Peripheral Artery Disease, Ischemic Stroke and Transient Ischemic Attack and discharge date between 2011 and 2014 (study period). Pharmacological treatment for each subject was defined selecting all prescriptions of Statins, Ezetimibe and Simvastatin/Ezetimibe, retrieved between the date of the last prescription in the study period and the previous 90 days. We stratified patient in no-treated, treated with low/medium intensity statins (LMS), high-dose statins (HDS) and other Lipid-Lowering Therapies (LLTs). Furthermore, for Umbria region, we selected the last blood levels test of LDL-cholesterol occurred in period 2011-2016. Starting from test date, we defined the pharmacological treatment in the previous 90 days. Subject were stratified based on LDL-C levels in target (<70) and not at-target (≥70) patients.

Results. Population in secondary prevention for ASCVD in period 2011-2014 in Marche and Umbria was estimated in 23,043 (prevalence: 4.3 x 1,000 inhabitants), corresponding to more than 800,000 subjects in Italian population. Within treated patients: 51.3% received LMS, 38.1% HDS and 10.6% other LLTs. No-treated patients were 27.8%. LDL-C target was achieved by 34.9% of patients treated with LMS and by 46.1% of patients treated with other LLTs.

Conclusions. The study, based on Italian administrative databases, allowed to estimate the very high risk population in secondary prevention for ASCVD. It highlighted a relevant proportion of no-treated patients, and an high proportion of patients that did not achieve recommended LDL-C target.

VP49 Real-world Evidence For Economic Evaluation Of Medical Devices

Benedetta Pongiglione (benedetta.pongiglione@unibocconi.it) and Aleksandra Torbica

Introduction. Randomized controlled trials (RCTs) are considered the gold standard in the hierarchy of research designs for

https://doi.org/10.1017/S0266462319003167 Published online by Cambridge University Press

evaluating the efficacy and safety of a treatment intervention. The low external validity of RCTs and the general shortage of clinical evidence available to support the use of many medical devices have emphasized the necessity for exploring the use of real-world data (RWD) as a complementary source to RCTs data for establishing a more robust evidence base on the effectiveness of medical devices. The aim of the present project is to assess in a comprehensive way the existing sources of real world data on medical devices in Europe.

Methods. The guidelines to the mapping exercise have been outlined in a research protocol. First, all national relevant sources (e.g. website of Ministry of Health, national institutions, research bodies) are screened, both in local language and English. Second, we perform a systematic search on PubMed using a set of key words for each case study, adapted to each country setting. Finally, we seek advice from key actors in the field of the device and clinical conditions, such as manufacturers or clinicians.

Results. Information on existing sources of RWD for each case studies are provided in a template including details on the key features of the source (e.g. data producer, data collection period, sample size, study design, geographical coverage) and the main content of the dataset, distinguishing socio-demographic information, clinical and epidemiological data, data on resource use and health outcomes. The data mapping includes all countries of the project participants, i.e. Italy, UK, Netherlands, Switzerland, Germany, Hungary, and we enlarge the scope of our mapping including other countries: Spain, France, Denmark, Finland, Sweden, Poland and Hungary as well as international databases at pan-EU level. The number of available sources of RWD and their quality vary depending on case study and across countries. For example, in the case of orthopaedics, many countries have a national registry and administrative data, such as hospital discharge, contain useful information, although not as detailed. When a registry is not available, it is often the case that more observational studies are available; this occurs for example in France.

Conclusions. In this work we shows the importance of RWE and map in an accurate and comprehensive way which source of RWD are currently available and to what extent they are known and used in medical, epidemiological and economic research.

VP53 Long-Acting Insulin Analogues In Brazil: Clinical And Economic Impact

Lays Pires Marra (laysmarra@yahoo.com.br), Daniel da Silva Pereira and Gustavo Laine Araújo de Oliveira

Introduction. The aim was to evaluate the effectiveness, safety and economic impact of long-acting insulin analogues (LAIA) compared to NPH for type 1 diabetes mellitus (DM1).

Methods. A search was performed in five electronic databases to find systematic reviews (SR) comparing at least a LAIA to NPH insulin for DM1. Budget impact analysis was performed from the perspective of Brazilian public health system (SUS), with NPH insulin as the base scenario. The costs were extracted from the Integrated System of Administration of General Services (SIASG). The market share was calculated per month, using a logarithmic function with maximum diffusion of 50% at the end of the time horizon - five years.

Results. A total of 160 studies were identified and seven SR of low to uncertain risk of bias were selected. LAIA have shown modest clinical benefit and its effect is more prominent for the control of severe and nocturnal hypoglycaemia. Insulins glargine and detemir compared to NPH were associated with reduction in HbA1c levels between 0.16% and 0.40% and associated with lower risk of episodes of severe hypoglycemia. Insulin degludec compared to NPH showed no statistically significant difference in the reduction of HbA1c levels and in the episodes of severe hypoglycemia. The budget impact ranges from USD 210 million (detemir) to USD 670 million (degludec) over five years.

Conclusions. The use of LAIA as a basal insulin regimen for DM1 may benefit more patients with recurrent episodes of hypoglycemia. However, the fragility of the outcomes considered to evaluate the clinical impact of LAIA and the high budget impact with its use should be considered, and may compromise SUS sustainability. In view of these aspects, CONITEC recommended the incorporation of one of the LAIA, if the treatment is equal to or less than that of NPH insulin and according to the criteria established by a guideline.

VP54 Digital Tools For More Efficient Conduct Of RCTs: Trials Unit Survey

Jacqui Nuttall (J.Nuttall@soton.ac.uk), Athene Lane, Amanda Blatch-Jones, Gareth Griffiths and Jeremy Wyatt

Introduction. Recruitment of participants to, and their retention in, Randomized Controlled Trials (RCTs) is a key determinant of research efficiency, but is challenging. Digital tools and media are increasingly used to reduce costs, waste and delays in the conduct and delivery of research. The aim of this UK Clinical Trials Unit (CTU) survey was to identify which digital recruitment and retention tools are being used to support RCTs, their benefits and success characteristics.

Methods. A survey was sent to all UK Clinical Research Collaboration (UKCRC)-registered CTUs with a webinar to help increase completion. A logic model and definitions of a "digital tool" were developed by iterative refinement by project team members, the Advisory Board (NIHR Research Design service, NHS Trust, NIHR Clinical Research Networks and patient input) and CTUs.

Results. A total of 24/52 (46%) CTUs responded, 6 (25%) of which stated no prior use. Database screening tools (e.g. CPRD, EMIS) were the tool most widely used (45%) for recruitment and were considered very effective (67%). The most mentioned success criteria were saving GP time and reaching more patients. Social media was second (27%), but estimated effectiveness varied considerably, with only 17% stating very effective. Fewer retention tools were used, with SMS / email reminders reported most (10/15 67%), but certainty about effectiveness varied. A detailed definition on what constitutes a digital tool with examples and a logic model showing relationships between the resources, activities, outputs and outcomes for digital tools was developed.

https://doi.org/10.1017/S0266462319003167 Published online by Cambridge University Press