VP39 The Alphabet Lottery? How NICE Outcomes Vary By Appraisal Committee

Richard Macaulay (richard.macaulay@parexel.com), Lok Wan Liu, Cornelia Roibu and Andrea Berardi

Introduction. NICE (National Institute for Health and Care Excellence) makes recommendations on the public reimbursement of medicines based on their clinical- and cost-effectiveness. The recommendation is made by an Appraisal Committee (comprising a multi-disciplinary group of independent experts) as part of a technology appraisal. There are four Appraisal Committees (A,B,C,D); this research investigates whether appraisal outcomes vary by committee.

Methods. All publicly-available Final Appraisal Determinations from NICE Single Technology Appraisals (STA) were screened (01/10/2009-14/11/2018) and key data were extracted. Homogeneity in rates of acceptance or rejection across the committees was assessed using Chi-squared tests.

Results. The Appraisal Committee was identified for 298 technologies, 56% (168/298) of which were 'recommended'. The number of technologies assessed by each committee was similar (A:79, B:62, C:91, D:66). However, STAs conducted by Committee D were significantly less likely to receive 'recommended' outcomes (A:68% [54/79], B:65% [40/62], C:53% [48/91], D:39% [26/66]; $p < 0.01$). STAs for oncology indications had higher 'not recommended' outcomes than those for non-oncology indications (25% vs. 9%). The lower 'recommendation' rates for committee D persisted across oncology (A:60%, B:83%, C:50%, D:38%; $p = 0.01$) and non-oncology indications (A:73%, B:53%, C:55%, D:40%; $p < 0.01$). However, STAs conducted by Committee D were significantly more likely to receive 'optimized' recommendations (A:16%, B:21%, C:33%, D: 36%; $p < 0.01$) and when considering the rates of 'recommended' and 'optimized' outcomes compared to 'only in research' and 'not recommended' outcomes, no significant differences were found (A:85%, B: 85%, C:86%, D:76%; $p = 0.27$).

Conclusions. STAs undertaken by NICE Appraisal Committee D was associated with a significantly lower rate of 'recommended' outcomes but tended to an 'optimized' recommendation significantly more than the other committees. Further research is needed to determine if this reflects any deviation in uniform implementation of NICE methodology between Committees.

VP40 Increasing Divergence Of IQWiG & G-BA Benefit Assessments Over Time?

Richard Macaulay (richard.macaulay@parexel.com) and Zainab Mohamoud

Introduction. Since 2011, new pharmacological therapies in Germany are subject to an early benefit assessment (EBA) upon launch. The Institute for Quality and Efficiency in Health Care (IQWiG) usually conducts an initial assessment, followed by the Federal Joint Committee (G-BA) issuing a final resolution. If the G-BA deem a new therapy offers no additional benefit over relevant comparators, it cannot attain premium-pricing through price negotiations. This research compares G-BA and IQWiG assessment outcomes over time.

Methods. All EBA resolutions were extracted from the G-BA website alongside corresponding IQWiG assessments (01/01/2011-19/09/2018) and key information compared. For extracted outcome data, the focus was the subgroup of greatest additional benefit.

Results. Of 261 identified EBAs with both G-BA and IQWiG assessment outcomes published, 59% (155/261) did not differ in their additional benefit. The G-BA concluded on an additional benefit where IQWiG deemed none in 13% (34/261) of cases, which was consistent pre-2015: 13% (11/87) and 2015-onwards: 13% (23/174). Conversely, IQWiG deemed an additional benefit where the G-BA concluded on none in 3% (8/261) of cases, none of which were pre-2015 (0/87) vs. 5% (8/261) for 2015-onwards. G-BA and IQWiG both agreed that additional benefit was offered but differed in its extent in 14% (37/261; in 23 cases: G-BA's rating was lower, 14 cases: G-BA's was higher) with 19% (17/87) pre-2015 vs. 8% (14/174) 2015-onwards.

Conclusions. The G-BA has deviated from IQWiG's initial assessment in around one-third of resolutions, with potential significant rebate negotiation consequences. The divergence in extent of additional benefit (where both agree on additional benefit) appears to be becoming less common over time. However, a slight converse time-trend appears regarding divergence on whether any additional benefit is offered, driven by increased incidence of G-BA deeming no additional benefit contrary to IQWiG. This emphasizes that companies should fully engage with the EBA consultation process post-IQWiG appraisal.

VP41 NICE Interventional Procedures Advisory Committee Recommendations

Christopher Carroll (c.carroll@shef.ac.uk), Rumona Dickson, Angela Boland, Rachel Houten and Matthew Walton

Introduction. This study explores the factors (principally evidential) that predict guidance recommendations by this NICE committee. There are three main types of recommendations: Standard/normal arrangements (can be done without restriction in the NHS); Special arrangements (can be done under certain conditions); and Research only.

Methods. The following data were extracted from all published pieces of Interventional Procedure Guidance (IPGs) produced by this committee: year, IPG number, recommendations, evidence base (numbers and types of included studies, numbers of included patients etc.). All data were extracted independently by two researchers, and any disagreements clarified by consensus. Data were tabulated and descriptive statistics produced. Regression analyses will be performed using these data to identify any statistically significant predictors of recommendations.

Results. IPG recommendations (n = 496); year range: 2003-2018. Proportion of IPGs by each recommendation: 50% Standard; 38% Special; 11% Research Only; 2% Do Not Do. Proportion of IPGs with highest level evidence (i.e. systematic review and/or RCT) by recommendation type: Standard = 64% (152/239); Special = 43% (77/180); Research Only = 48% (26/54); Do Not Do = 75% (6/8).

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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

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