RESULTS:

In 2007, the use of eculizumab was approved by the United States Food and Drug Administration and the European Medicines Agency. In Brazil, despite the provision of eculizumab through judicial proceedings since 2009, the manufacturer of eculizumab only requested a licence for it in 2017, after several meetings with the government when the company agreed to provide the drug at approximately half the price of the imported product. The efficacy of eculizumab in PNH patients was assessed in one randomized, placebo controlled study, one single arm study, and one long-term extension study. The drug reduced hemolysis and the need for transfusion, although the studies had methodological problems. The efficacy of eculizumab in the treatment of aHUS was assessed in four prospective, controlled open-label studies, two long-term extension studies, and one retrospective study. Eculizumab normalized platelet counts and reduced the need for plasmapheresis, although the studies had no control group. Eculizumab was well tolerated, with no meningococcal infections occurring after patients were immunized.

CONCLUSIONS:

Some companies have no interest in licensing their products in Brazil because their provision by judicial proceedings is more lucrative. This situation promotes litigation and irrational prescription of drugs, and also obligates the Brazilian government to import expensive health products.

PD66 Indirect Comparison Of Treatments For Metastatic Melanoma

AUTHORS:

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INTRODUCTION:

Vemurafenib plus cobimetinib (VC) for the treatment of metastatic melanoma was requested to be included in the National Formulary in Uruguay. The standard of care for metastatic melanoma in Uruguay is dacarbazine. There is no published head-to-head trial assessing the effects of VC versus dacarbazine. The objective of this study was to perform an indirect comparison of the effects of dacarbazine, compared with VC, based on the results of trials that included both treatments versus the same comparator (vemurafenib alone).

METHODS:

We searched Pubmed and The Cochrane Library for trials comparing either VC or dacarbazine with vemurafenib. Trials were assessed in terms of risk of bias, similarity of interventions and inclusion and exclusion criteria, and comparability of characteristics of patients in the vemurafenib arm. We performed an indirect comparison using the Bucher method.

RESULTS:

From the literature search we retrieved two studies that met the inclusion criteria: a randomized clinical trial that assessed VC versus vemurafenib or placebo and another assessing dacarbazine versus vemurafenib. Both studies were similar in terms of methodological quality, inclusion and exclusion criteria, and comparability of the vemurafenib arms. However, the comparison of overall survival and progression-free survival curves for the vemurafenib arms were quite different between the two trials. At 9 months, overall survival was eighty-one percent and fifty-five percent and progression-free survival was thirty percent and fifteen percent, respectively. The indirect comparison provided the following hazard ratios: 0.24 (95% confidence interval [CI]: 0.14–0.48) for overall survival; 0.13 (95% CI: 0.09–0.19) for progression-free survival; and 0.15 (95% CI: 0.02–1.29) for grade 4 adverse events.

CONCLUSIONS:

Treatment with VC increased overall survival and progression-free survival, compared with dacarbazine. Severe adverse events were less frequent with the combined therapy. However, the differences in the vemurafenib survival curves increases doubts about the accuracy of the indirect estimators of overall survival and progression-free survival.

PD67 Strengthening And Accelerating Health Technology Assessments Through Artificial Intelligence

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INTRODUCTION:

Rising costs and the rapidly increasing volume of findings from research in health care are driving the demand for comprehensive information to inform the allocation of resources. Health technology assessment (HTA) applies rigorous processes to provide high-quality synthesized information to policymakers and healthcare payers. HTA involves combining large amounts of research publications to systematically evaluate the properties, effects, and impacts on a topic of interest.

METHODS:

The time and resources required to complete a full HTA are often demanding. There is an opportunity to apply high-performance computing (inclusive of artificial intelligence and machine learning disciplines) to HTA. This project applied high-computing technology to create a research synthesis tool to support HTA and then developed a service that integrates as much relevant data as possible to strengthen HTA. This was a joint project that combined expertise from the areas of health technology, machine learning, information technology, and innovation.

RESULTS:

The information gathered for this phased project from HTA subject matter experts and other stakeholders was collated to inform a research synthesis tool and a broader concept of the project.

CONCLUSIONS:

The results of this study will inform the design of a research synthesis tool that covers the entire HTA process (literature search, screening titles and abstracts, data extraction, quality assessment, and analysis). The collaborators included Alberta Innovates, the Alberta Machine Intelligence Institute, the University of Alberta, Cybera, and PolicyWise. Alberta Innovates, which is an accelerator and innovator of research in the province of Alberta, Canada, was the primary source of funding for this project.

PD70 Cost-Effectiveness Of Deep Brain Stimulation For Epilepsy In Australia

AUTHORS:

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INTRODUCTION:

Deep brain stimulation (DBS), which uses an implantable device to modulate brain activity, is an adjunctive treatment for partial-onset seizures in patients with medically refractory epilepsy. Our objective was to perform an exploratory cost-utility analysis of DBS in conjunction with medical therapy, compared with medical therapy alone, using the latest clinical data and costs for the Australian healthcare system.

METHODS:

A deterministic five-state Markov model was used to project treatment response and outcomes over the patients' lifetimes, based on 5-year data from the recent Stimulation of the Anterior Nucleus of the Thalamus for Epilepsy (SANTE) DBS trial and drug outcome data identified through a literature search. Costs were based on 2017 data for the Australian healthcare system, and response-specific utilities were derived from published literature. We estimated the lifetime discounted incremental cost-effectiveness ratio (ICER) in Australian dollars per quality-adjusted life-year (QALY) for patients 36 years of age, fifty-five percent of whom were men. Costs and effects were discounted at five percent per annum. The robustness of projections was evaluated through scenario and sensitivity analyses.

RESULTS:

Under assumed continued treatment benefit, DBS was projected to add 3.48 QALYs over the patients' lifetimes, at an increased cost of AUD 142,304 (USD 105,960), resulting in an ICER of AUD 40,951 (USD 30,492) per QALY gained. Reducing the analysis horizon to 20 years increased the ICER to AUD 49,803 (USD 37,083). Increasing the DBS generator life from 3 to 6 years decreased the ICER to AUD 23,956 (USD 17,838) per QALY. Longer follow-up periods and younger age at treatment were associated with greater cost effectiveness. Results were sensitive to assumptions about health state-specific utility estimates and long-term treatment effects.

CONCLUSIONS:

Our exploratory findings suggest that DBS is a cost-effective treatment strategy in the Australian healthcare system for patients with medically refractory epilepsy. DBS therapy might meaningfully improve patient outcome at a health economic profile that compares favorably to other well accepted therapies. Consideration of indirect costs would further add to this value proposition.