ANVISA to authorize the off-label use of health technologies provided that the analysis is supported by scientific evidence regarding effectiveness, accuracy, and safety for the intended purpose.

CONCLUSIONS:

The off-label use of health technologies is a worldwide practice that can favor vulnerable populations and neglected diseases. This practice should be seen as positive when there is evidence supporting off-label use, and such decisions should not be influenced by political, economic, or marketing considerations.

PD64 Diagnostic Accuracy Of The Nitrate Reductase Assay Technique

AUTHORS:

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INTRODUCTION:

The conventional drug sensitivity test is traditionally used in Brazil to diagnose drug-resistant tuberculosis. However, the test can take up to 60 days to return a diagnosis, which is considered too long for certain vulnerable populations. Therefore, this study analyzed the available scientific evidence on the accuracy and time to diagnosis of the nitrate reductase assay for diagnosing resistant tuberculosis, compared with the conventional drug sensitivity test.

METHODS:

We searched MEDLINE, Embase, and The Cochrane Library for systematic reviews with meta-analyses. The articles were screened by title and abstract. The full-texts of potentially relevant articles were then screened according to the inclusion criteria.

RESULTS:

Three systematic reviews with meta-analyses were selected that compared the nitrate reductase assay with the conventional drug sensitivity test. The accuracy of the nitrate reductase assay was satisfactory in most of the results when compared with the sensitivity test, except for one study that showed low sensitivity for the detection of streptomycin resistance. In addition, the nitrate reductase assay had a shorter time to diagnosis than the drug sensitivity test.

CONCLUSIONS:

The results of this study reinforce the idea that the nitrate reductase assay may diagnose drug-resistant tuberculosis earlier than the conventional drug sensitivity test and be a helpful strategy for controlling the disease, especially in vulnerable populations that are more likely to be affected by tuberculosis. For a broader analysis of the benefit of the assay, it is suggested that studies investigate the impact of the shorter time to diagnosis on morbidity and mortality in patients with drug-resistant tuberculosis. In addition, economic analyses comparing the nitrate reductase assay with the sensitivity test are recommended to evaluate the cost-benefit ratio.

PD65 The Acquisition Of Eculizumab By Judicial Proceeding In Brazil

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INTRODUCTION:

Eculizumab is a monoclonal antibody indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) or with atypical hemolytic uremic syndrome (aHUS). In Brazil in recent years eculizumab was the most expensive drug requested through court orders, obliging public health managers to import it from the USA. From 2012 to 2016, approximately BRL 424 million (USD 112 million) was spent on eculizumab. The purpose of this study was to assess the regulatory situation and the scientific evidence on the safety and efficacy of eculizumab.

METHODS:

A literature search was conducted in PubMed, The Cochrane Library, and the Centre for Reviews and Dissemination databases on September 2017. The websites of regulatory agencies were also searched.

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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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