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Cost-effectiveness of the treatment of sickle cell anemia with hydroxyurea: an economic evaluation study

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ABSTRACT

Objective: to analyze the cost-effectiveness of the treatment of sickle cell anemia (SCA) using hydroxyurea. **Method:** economic evaluation study, with a quantitative, descriptive and analytical approach, through a retrospective cohort. To be developed in the main reference services in Campo Grande/MS. Through three phases: systematic literature review, with analysis by odds ratio, model of inverse random effect and chi-square; secondary data collection from medical records and DATASUS with chi-square analysis or Fisher's exact test; cost-effectiveness analysis using the Markov analytical decision model and Student's t test for all variables. This research is approved by the UFMS Ethics Committee, under opinion no. 2,892,100. **Expected results:** Produce indicators to support the improvement of public policies, assist in decision-making and therapeutic conduct, in addition to promoting the improvement of patients' quality of life.

Keywords: Cost-Benefit Analysis; Sickle Cell Anemia; Hydroxyurea, Sickle Hemoglobin; Costs and Cost Analysis.

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INTRODUCTION

Estimating the cost of treating patients with a specific medical diagnosis in specific therapeutic regimes is complex, but extremely important to support the development and implementation of public policies.

Thus, the cost-effectiveness of a treatment involves from the real global cost of a given treatment, which must estimate how effective the result is, to the point of justifying high investments to obtain improvements in quality of life, in addition to years of experience. saved lives[1].

Among chronic diseases, hemoglobinopathies deserve to be highlighted, mainly because they deal with conditions that accompany patients since birth. Among these, sickle cell anemia (SCA) counts as a form of drug treatment practically a single high-cost drug, widely used in the world, Hydroxyurea[2].

The challenge for improving the treatment of SCA consists of planning by managers, as well as estimates of the costs and economic benefits needed to improve care[2]. Thus, due to the lack of studies that establish the cost-effectiveness of treatment with the use of hydroxyurea for SCA in Brazil, this study is justified as a way of producing indicators for public policies for SCA patients, family members, managers and professionals.

OBJECTIVE

To analyze the cost-effectiveness of the treatment of sickle cell anemia using hydroxyurea compared to those who do not use this medication.

METHOD

It is a complete economic evaluation study of the type of cost-effectiveness analysis[3], with a quantitative, descriptive and analytical approach, through a retrospective cohort.

It will be developed in the main reference services for SCA in the state of Mato Grosso do Sul: Maria Aparecida Pedrossian University Hospital; Regional Hospital of Mato Grosso do Sul Rosa Pedrossian and State Coordination of Specialized Pharmaceutical Assistance. It will be organized in three phases:

I - Systematic review: developed based on the PRISMA recommendations and will use the PICO strategy, with a protocol to guide searches in the indexed bases of the CAPES Journal Portal. The data will be extracted by two researchers. The Cochrane form will be used, quality of evidence by GRADE and the risk of bias by the ROBIS tool. Analysis in the RevMan® software, the dichotomous outcomes by calculating the odds ratio, the continuous outcomes by the inverse random effect model. Chi-square tests and analysis of variance to analyze heterogeneity.

2 - Secondary data collection: Medical records will be collected retrospectively, using a structured form. Approximately 80 patients with SCA, of all ages and sex attended from 1980 to 2019 will participate. Illegible medical records with a lack of data will be excluded. Based on the data, the AF DALY will be stipulated. Data will be collected from the Reduced Authorization Hospital Information System DATASUS, historical series of the last 10 years (2008

to 2018) registered in the Brazilian states. The analysis with the SPSS® software, to check the association with the chi-square test, for the tendency the chi-square test or Fisher's exact test.

3 - Modeling System: Markov's analytical decision model will be adopted, and the hypothetical cohort will consist of individuals with SCA of all ages and both genders. The validation of parameters with the participation of experienced researchers. The perspective will be from SUS (Brazilian Public Health System) and health plans, with a 20-year time horizon. For clinical parameters, the results of RS and data collection will be considered. The monetary value in Reais and per micro-cost. The degree of stability and robustness will be analyzed by the Incremental Cost Effectiveness Ratio. Use of the TreeAge Pro® software and univariate analysis using Student's t test. In all tests, the level of significance adopted will be 0.05 ($p \leq 0.05$).

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EXPECTED RESULTS

It is expected to carry out a cost-effectiveness analysis of the treatment of SCA with Hydroxyurea in a robust model in order to produce indicators that can be used to improve public policies in Brazil and other services around the world. The data can be used for decision making within the scope of management and clinical practice, as well as in future research.

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- Cardoso AIQ, Ferreira Júnior MA, Barbieri AR, Carvalho AMA, Grande AJ and Ivo MA contributed to the construction of the project, research, analysis and interpretation of data, writing of the article, critical review and approval of the final version.

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