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COST-EFFECTIVENESS OF AZACITIDINE IN THE REAL-WORLD: ANALYSIS IN HIGH-RISK PATIENTS WITH MYELODYSPLASTIC SYNDROMES FROM THE PERSPECTIVE OF A EUROPEAN PUBLIC HOSPITAL

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BACKGROUND

- Azacitidine is the recommended treatment of higher-risk myelodysplastic syndromes (MDS) in patients who are not candidates for hematopoietic transplantation. It is also used in low-risk MDSs where supportive treatment fails.
- Despite its widespread use, there are no pharmacoeconomic data of azacitidine based in the real-world setting, outside the context of clinical trials.

OBJECTIVE

- To evaluate the incremental cost-effectiveness ratio of azacitidine versus supportive care in patients with MDS treated in a public hospital, from the payers' perspective.

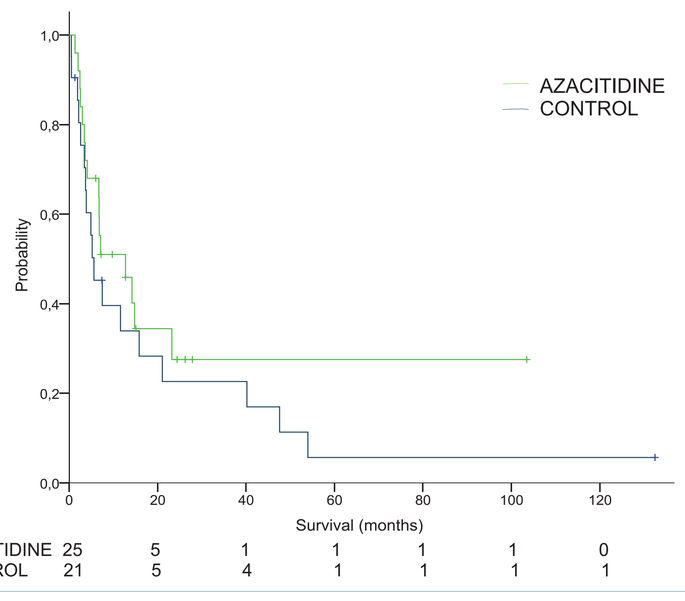
METHODS

- Type of study: Observational retrospective study
- Population: 2 cohorts of patients with MDS (n=53 patients each one), with similar demographic, clinical, biological and haematological characteristics. Patients were stratified according to the International Prognostic Score System for risk: 25 high-risk/intermediate-2 were treated with azacitidine, and 21 with supportive care.
- Medication consumption, transfusion support and hospital resources related to MDS were accounted for each patient, according to the Valencian Community Fees Law (2016 fiscal year), and to the 2017 final hospital sale price for medicines. Overall survival since diagnosis was the measure of effectiveness.
- Mean based incremental cost-effectiveness ratio (ICER) was estimated with the bootstrapping resampling technique.
- The average cost was calculated with the Bang-Tsiatis[1] reweigthed estimator and restricted mean survival time (RMST) was used for effectiveness.

RESULTS

- Patients treated with azacitidine showed improved survival in high-risk/intermediate-2 patients.
- Kaplan-Meier median survival was 13 months in azacitidine group versus 6 months in control group. (Figure 1)
- RMST, which is defined as the area under the survival curve up to truncation time (102 months in this study), was 33 months in azacitidine treated patients and 19 months in control group.
- Statistical significance was not reached (log rank p= 0.346, test of differences in RMST p= 0.171) even when the magnitude of survival differences is important, probably due to the small number of patients in this subgroup.

FIGURE 1: Kaplan- Meier survival curve (subgroup of high risk / intermediate-2)



- The mean based cost-effectiveness ratio was 16.812€ per life-year gained (LYG). (Figure 2)
- According to the cost-effectiveness plane 91% of values lie in the northeast quadrant, where increased survival is achieved at increased cost.
- 68% of the values are within the threshold (30.000€ per LYG) of willingness to pay commonly accepted for cost-effectiveness in Spain.

FIGURE 2: Cost-effectiveness plane

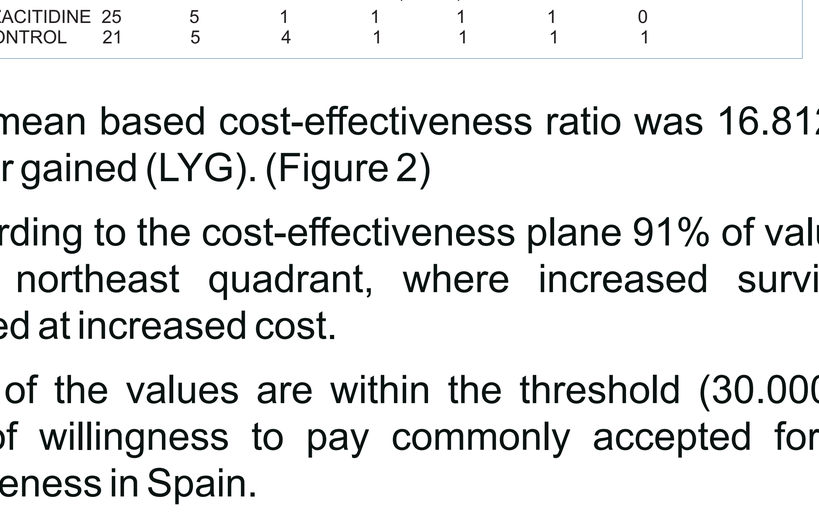
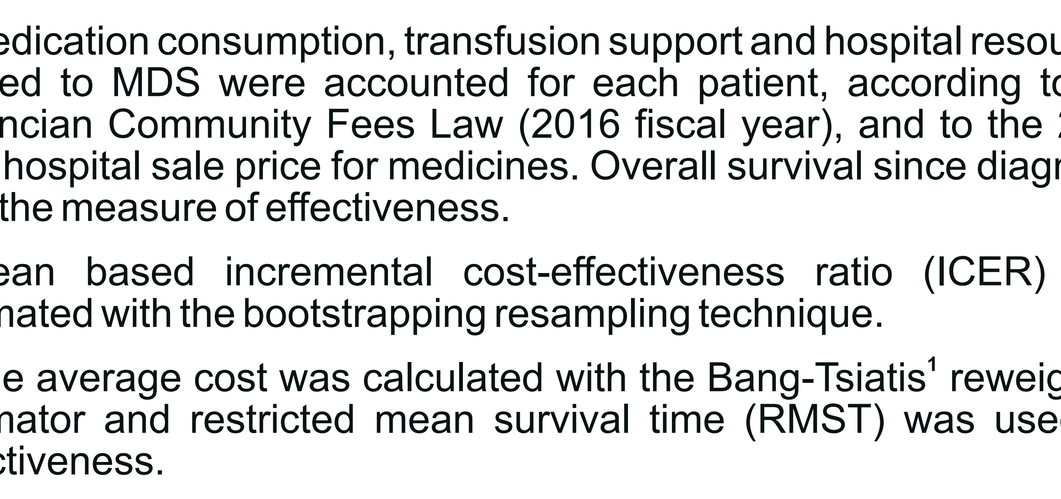


FIGURE 3: Distribution of the average cost per patient and year of the components of the total cost (subgroup of high risk / intermediate-2)



	Treatment Pharmacological*	Transfusions	Hospital Resources	Total Cost
Control Group	368 €	13.443 €	26.323 €	40.134 €
With Azacitidine	23.674 €	10.162 €	30.619 €	64.455 €

- The mean annual cost was significantly higher in patients treated with azacitidine compared to the control group (64.455€ versus 40.134€, p = 0,003). (Figure 3)
- In both groups, the main component was derived from hospital resources, which includes office and emergency department visits, hospital stays, day hospital and home hospitalization.

CONCLUSIONS

- Azacitidine shows a favorable cost-effectiveness ratio in high-risk intermediate-2 patients, although with the uncertainty derived from the small sample size.
- This result corroborates what is reflected in the bibliography for azacitidine cost-effectiveness, but the strength of this study is that is based on data obtained from the usual healthcare practice. On the contrary, azacitidine cost-effectiveness publications are usually based on mathematical models and data from clinical trials, which shows more favorable results than real-world practice.

REFERENCES

1- Bang H, Tsiatis AA. Estimating medical costs with censored data. Biometrika 2000;87(2):329-343.

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