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A medico-economic study of trabectedin compared with end-stage treatment in soft tissue sarcomas

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ABSTRACT

The development of new anticancer drugs has strongly contributed to improve patients' outcomes. However, the question can be raised whether the sometimes minor benefits are worth the cost to both the individuals and the society. The aim of this medico-economic study was to evaluate trabectedin cost-effectiveness compared with end-stage care in the treatment of soft-tissue sarcoma. We retrospectively analyzed data from 45 patients treated with trabectedin for soft tissue sarcoma. Real-life data of clinical benefits and costs of trabectedin were compared with simulated end-stage treatment. Univariate and probabilistic sensitivity analyses identified key drivers of the incremental cost-effectiveness ratio. Trabectedin was associated with longer progression-free survival (4.0 months versus 1.6 months) and higher costs (€24,780 versus €15,490) compared with end-stage treatment. The incremental cost per year of progression-free survival gained with trabectedin was €46,104 (95% confidence interval; €45,039–€47,169). The results were relatively insensitive to changes. Trabectedin was cost-effective in the context of an orphan disease, when used to treat patients with a short life expectancy and few therapeutic options.

1. Introduction

Trabectedin is a marine-derived antineoplastic drug isolated from the Caribbean organism *Ecteinascidia turbinata* and currently produced synthetically. It is indicated for the treatment of unresectable or metastatic soft tissue sarcomas (STS) that have failed a prior anthracycline-containing regimen. The prognostic of progression-free survival (PFS) and overall survival (OS) for patients who relapse after first-line rarely exceed 6 months or 1 year [1,2]. Before the approval of trabectedin in Europe in 2007, a few agents have shown promising results when given as second-line treatment [3,4]. The antitumor activity of trabectedin has been demonstrated in several preclinical models and in clinical trials [5–8]. The clinical studies have provided data of efficacy for its marketing authorization [9] and have confirmed the clinical benefits of the drug when used in STS [10]. Because cancer drugs are used to treat patients with generally incurable diseases, new drugs have been rapidly adopted. The cost of new cancer treatments has however rarely been questioned. Today, high costs may prove a key limitation for accessibility. Indeed, health care in general and cancer care in particular can be expensive. While outcomes have improved in many areas due to new cancer treatments and technologies, the question can be raised whether the sometimes minor benefits are worth the cost to both the individuals and the society.

Regarding trabectedin, limited number of data is available on its use in routine clinical practice [11–13] or on its cost-effectiveness [14]. The aim of this study was to assess the cost-effectiveness of trabectedin against end-stage treatment (EST) when trabectedin was used in daily clinical practice to treat non-selected patients who were, for most of them, not eligible for inclusion in clinical trials. To do that, we conducted a retrospective analysis of data from STS patients treated in our institution from 2005 to 2014.

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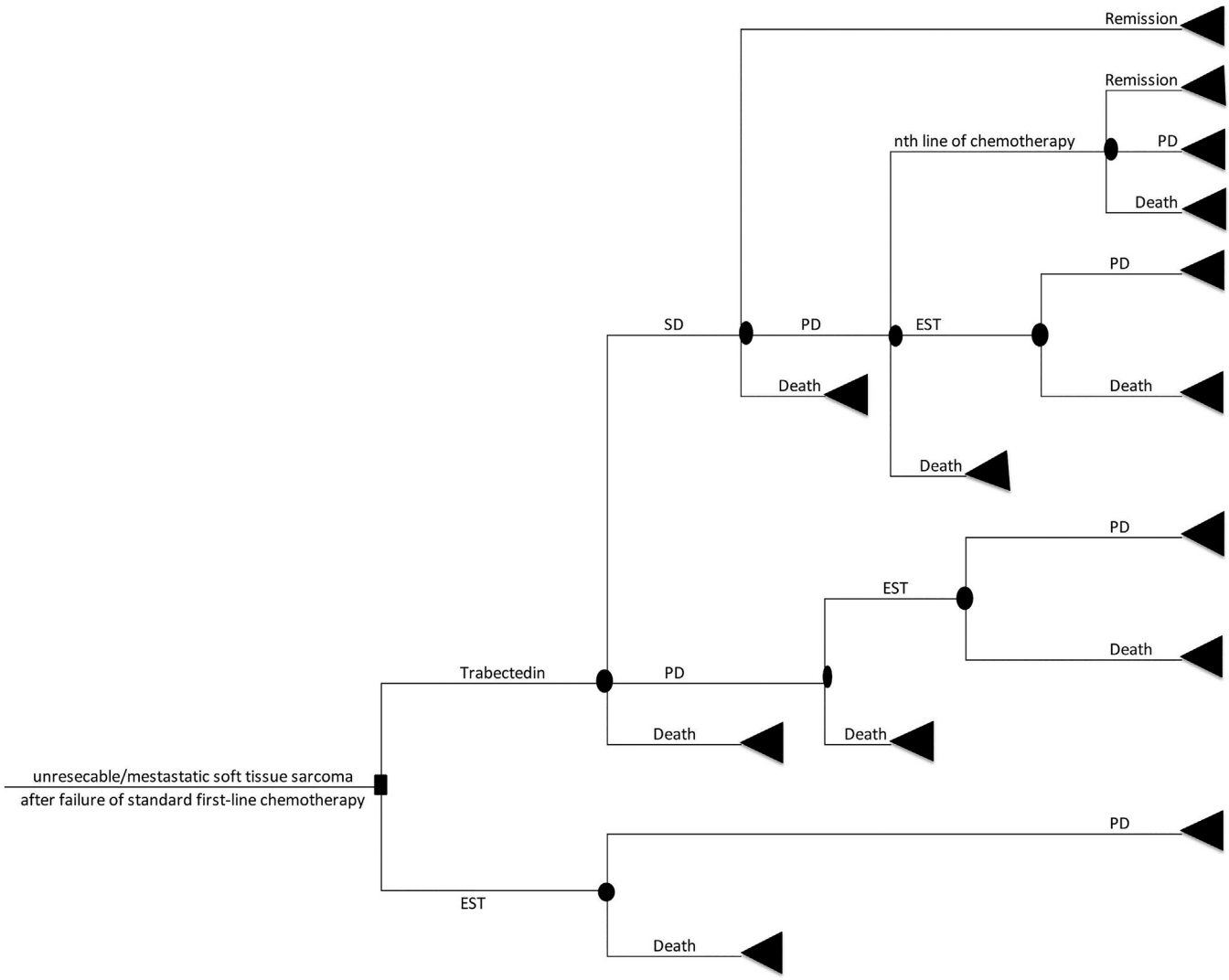

Fig. 1. Model structure and health states. EST: End-Stage Treatment, PD: Progressive Disease, SD: Stable Disease.

2. Methods

2.1. Model structure

The study population comprised adult patients with advanced STS, previously treated with CT. Because current evidence does not support any other second-line of CT for advanced STS, trabectedin treatment (24-h infusion every 21 days) was compared with EST (ie palliative medicine and care) initiated after the later line of CT.

A simplified schematic picture of the model structure is presented in Fig. 1. The model started with the failure of a previous CT for the treatment of advanced STS. The two alternatives are trabectedin or EST. In this model, patients could present 4 health states: remission, stable disease (SD), progressive disease (PD) and death. Patients on trabectedin could have a treatment response (remission or SD), disease progression or they could die. Patients on EST stayed in PD until death.

2.2. Effectiveness data

To assess trabectedin effectiveness, medical records of all patients with STS, having received trabectedin between January 2005 and May 2014, in Tenon hospital, Paris, were analyzed retrospectively until the cut-off date of August 1st 2014. The end-point of trabectedin effectiveness was progression-free survival (PFS). The durations of PFS were calculated according to the Kaplan-Meier method, from the date of inclusion to the date of progression or the most recent evaluation.

Effectiveness in the EST arm was estimated according to data published in the literature. Results of efficacy obtained in the placebo arm of a phase III trial of pazopanib versus placebo, conducted in patients with STS [15], were used to estimate the effectiveness of EST in our study.

2.3. Resources uses and costs

This medico-economic study was performed from the healthcare payer perspective. All pricing data used were based on regulated tariffs in the French Healthcare system. Drugs and biological analyses' prices corresponded to tariffs established by the French public health insurance applicable in 2014. Hospitalization costs were based on the French reimbursement tariff in 2014, according to the diagnosis-related group (DRG) in the French Agency for Information on Hospital Care (AIHC) classification, version 11f [16]. Pricing data of patients' transportations came from a report made in 2012 by the National Audit Office in France [17].

Concerning the cost of the trabectedin treatment strategy it included the cost of the drug itself, the cost of the inpatient adminis-