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# Different desferrioxamine usage in the patients with thalassemia major: a cost-effect analysis

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## ABSTRACT

Regular desferrioxamine (DFO) usage in patients with thalassemia major (TM) ameliorates hepatic, cardiac and endocrine dysfunction, improves growth and sexual maturation and prolongs survival. The difficulties of administering DFO with classic pumps are well known. The aim of this study was to compare the iron accumulation and cost effects between the continuous 48 hours infusion of DFO with infusor pump and the intermittent 40 hours infusion with classic pump in patients with TM. A total of 54 patients with TM were divided to two groups, first group includes 27 patients (18 female, 9 male) aged between 5.5 and 20.5 years, and were infused a total of 100 mg/kg DFO with infusor in 48 hours. Second group includes 27 patients (18 female, 9 male) aged between 6 and 22 years, were infused a total of 200 mg/kg DFO in 4 days with an intermittent infusions for about in 40 hours. After one year of treatment, the patients were compared from a clinical view point and cost of medical treatment. No statistical difference was found between infusor pump and classic pump in terms of cost-effectiveness.

**Key Words:** Desferrioxamine, Infusion pumps, Thalassemia, Cost-effectivity.

## ÖZET

### Talassemi majörlü hastalarda farklı desferrioksamin kullanımı: Maliyet-etkinlik analizi

Talassemi majörlü hastalarda düzenli desferrioksamin (DFO) kullanımı hepatik, kardiyak ve endokrin bozuklukları düzeltir, büyüme ve seksüel olgunlaşmayı artırır ve sağkalımı uzatır. DFO'yu klasik pompa ile vermenin zorlukları bilinmektedir. Bu çalışmanın amacı sürekli 48 saat infüzyon pompası ile DFO verilmesi ile aralıklı 40 saat klasik pompa infüzyonu arasında demir birikimi ve maliyet-etkinlik karşılaştırması yapmaktır. Talassemi majörlü 54 hasta iki gruba ayrılmıştır. Birinci grupta yaşları 5.5 ile 20.5 arasında değişen 27 hasta (18 kadın, 9 erkek) 48 saatte infüzyon pompası ile toplam 100 mg/kg DFO, yaşları 6 ile 22 yıl arasında değişen 27 hastalık (18 kadın, 9 erkek) ikinci gruba ise aralıklı infüzyon ile 4 günden toplam 200 mg/kg DFO verilmiştir. Tedaviden bir yıl sonra gruplar klinik ve maliyet-etkinlik açısından değerlendirilmiştir. Bir yılın sonunda iki grup arasında klinik ve maliyet-etkinlik açısından anlamlı bir fark bulunmamıştır.

**Anahtar Kelimeler:** Desferrioxamine, Infüzyon pompası, Talassemi, Maliyet-etkinlik.

## INTRODUCTION

Conventional treatment of thalassemia major (TM) includes regular transfusion and iron chelation therapy. Continuous chelator infusion or slow release oral preparation have theoretical advantages over discontinuous regimens with respect to iron detoxification and removal of plasma on transferrin bound iron (NTBI) and this should be considered particularly when designing regimens for high risk patients<sup>[1]</sup>.

It is clear that compliance with desferrioxamine (DFO) treatment is a major determinant of survival for patients. DFO ameliorates hepatic, cardiac and endocrine dysfunction, improves growth and sexual maturation and prolongs survival on iron overloaded patients<sup>[2]</sup>. DFO is infused with classic pump in 10-12 hours for 4-5 days per week or continuously with the disposable infusor pump over 48, 72, 96 and 120 hours<sup>[3]</sup>.

The aim of this study was to compare the iron accumulation and cost effects between the continuous 48 hours infusion of DFO with infusor pump and the intermittent 40 hours infusion with classic pump.

## MATERIALS and METHODS

A total of 54 patients with TM were divided into two groups, first group which includes 27 patients (18 female, 9 male) aged between 5.5 and 20.5 years, were given DFO 50 mg/kg/day continuous subcutaneous 48 hours infusions by an infusor (C1083-Baxter). They were infused a total of 100 mg/kg DFO in 2 days. Second group which includes 27 patients (18 female, 9 male) aged between 6 and 22 years,

were infused DFO 50 mg/kg/day for 4 days per week by classic pump. They were infused a total of 200 mg/kg DFO in 4 days with intermittent infusions.

After one year of treatment, the patients in two groups were compared from a clinical view point and according to the cost of the medical treatment.

## RESULTS

Mean serum ferritin levels of both groups at before, 3<sup>rd</sup>, 6<sup>th</sup> and 12<sup>th</sup> month of therapy were compared and the difference was not statistically significant ( $p > 0.05$ ) (Table 1).

Therapy costs including outpatient visit, inpatient visit, consultants, laboratory investigations, blood transfusion, chelation, treatment of complications were calculated according to the figures used by ministry of health and compared between infusor and pump group. When the total costs were examined, the pump using groups' yearly treatment cost was 9.647 USD, while the infusor groups' yearly treatment cost was 7070 USD. As it was seen in the table the cost of the patients who use infusor is less than the others who use pumps. Because of the DFO treatment cost, the pump using groups had a high cost (Table 2).

The compliance of the patients to infusor was 100%, while the compliance to the pump was 80%.

## DISCUSSION

When chelation therapy in patients with thalassemia major is needed, DFO is an excellent drug that has been available for al-

Table 1. Mean ferritin levels during therapy

Ferritin level (ng/mL)	Infusor group	Pump group
Before (mean $\pm$ SD)	4782.1 $\pm$ 2254.6	3609.8 $\pm$ 2079.3
3 <sup>rd</sup> month after therapy (mean $\pm$ SD)	3316.6 $\pm$ 1218.1	2869.5 $\pm$ 1434.3
6 <sup>th</sup> month after therapy (mean $\pm$ SD)	3830.9 $\pm$ 1844.8	3447.6 $\pm$ 1670.2
12 <sup>th</sup> month after therapy (mean $\pm$ SD)	3707.3 $\pm$ 1670.8	3070.3 $\pm$ 1442.9

**Table 2. Therapy costs of patients (YTL)\***

The cost	Infusor group (n: 27)	Pump group (n: 27)
Out patient visit	980.1	980.1
In patient visit	1312.7	21.6
Consultants	90.8	90.8
Laboratory	13108.6	13522.8
Transfusion	31655.5	27245.9
Chelation	198073.3	311170
Complication therapies	31571.2	24338.5
Total cost	276792.2	377369.8
The cost for each patient (YTL)	10251.6	13986.7
The cost for each patient (USD)	7070	9646

\* According to the budget data of ministry of health.

most 30 years. Its wide application caused an impressive improvement in life expectancy and a progressive lowering in the prevalence and severity of iron-related clinical complication<sup>[4]</sup>.

It is known that the iron binding effect of DFO depends on how long it stays in the body. Low dose application of DFO for a long period also reduces the toxicity due to DFO metabolites<sup>[5]</sup>.

A level of compliance with chelation above 60% and serum ferritin levels always below 2.500 ng/mL seem to be the best predictors of long term survival. Unfortunately, a significant proportion of patients fail to maintain a high compliance with DFO. This is due only in part to relevant local reactions. Main reasons are psychological, as this treatment is burdensome and from another side the patient can experience the effects of iron toxicity only after many years of poor chelation<sup>[4]</sup>.

It has been reported that continuous application of disposable infusor of DFO for 48-72-96-120 hours was more successful in urinary iron excretion and degreasing the iron load<sup>[6,7]</sup>. We also published that the compliance of the 48 hours infusor groups is 97% where as 120 hours infusor groups is 72%<sup>[8]</sup>.

In this study, it was observed the compliance to infusor is 100% while the compliance to the pump is 80%. With in two groups there were no meaningful distinctions due to the number and essence of the problems. There was not statistical significant at serum ferritin levels during therapy within two groups. Cost of using 200 mg/kg DFO 4 days per week with a classic pump was meaningfully higher than the cost of 100 mg/kg DFO once a week with infusor.

As a result, there were no statistical different about cost effect and iron loading between infusor pump and classic pump.

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