

Convenience and Preferability Among Patients with Sick Cell Anemia on Iron Chelator

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Abstract

Background: Sickle Cell Disease (SCD) is a chronic hemolytic disease that is significant and has a broad spectrum of complications. The value of blood transfusion in SCD is becoming increasingly evident. If left untreated, the resulting iron excess can be life-threatening. Parenteral infusions are necessary for chelation therapy with deferoxamine, which can have a detrimental effect on drug adherence and quality of life.

Methods: Deferoxamine (20-50 mg/kg/day) or oral deferasirox (20-40 mg/kg/day) was administered to 97 patients in a 2:1 randomized fashion. Sixty patients have received this medication previously.

Results: At the end of each interval of questioning, a significantly greater number of people who passed the time up and took medication were either (satisfied\ delighted) with deferasirox or identified the treatment as {preferable\very preferable} compared to deferoxamine ($p = 0,001$). Deferasirox resulted in

fewer hours lost from daily activities than deferoxamine in those patients. At the conclusion of the investigation, most patients expressed their willingness to continue their oral therapy (84 vs 11%).

Conclusions: Therefore, patients with SCD are more satisfied with deferasirox, as it exerts a lesser impact on daily activities than deferoxamine. Enhanced quality of life is associated with a high level of satisfaction. The improved treatment outcome is associated with high oral chelation adherence.

Introduction

It has been scientifically proven that blood transfusions are very beneficial in preventing stroke in patients with sickle cell anemia (SCD) [1]. Blood transfusions have also been shown to be essential in helping to prevent acute chest syndrome, as well as pain attacks and failure to thrive [2, 3]. Upon reaching adulthood, many patients with sickle cell disease will require blood transfusions [4, 5]. Iron increases due to repeated blood transfusions. Failure to administer regular iron chelation therapy may lead to iron overload from frequent blood transfusions, resulting in potentially fatal consequences and premature death [6, 7]. Transfusion therapy is gaining popularity in SCD due to recent research suggesting it can prevent organ injury and improve the prognosis of complications in certain patients [8–11]. Deferoxamine (DFO; Desferal) is the most commonly used long-term chelation therapy for iron overload. Its administration can prevent iron-induced organ damage and associated complications, while also improving survival in patients with analogous transfusion-dependent illnesses [6, 12]. Some patients may experience a range of local reactions to gas injections, including skin rash, bruising, inflammation, and allergies [13].

One study found that over 40% of patients cited drug-related side effects as the primary cause of missing a DFO dose [14]. The administration of DFO involves multiple subcutaneous injections over 5 to 7 days, with an interval of 8 to 12 hours [15]. This method is exceedingly taxing; this is especially critical because, at least in the context of thalassemia, there is no evidence that adherence to chelation therapy has a detrimental effect on the patients undergoing blood transfusion [16]. Additionally, this substantial encumbrance can significantly impair patients' ability to comply with their treatment regimens. Taking the drug has a detrimental effect on the lives of patients who are accustomed to blood transfusions [17]. Despite patients adhering strictly to the treatment regimen, they may experience a significant decline in health [18] and daily activities [19]; therefore, maintaining blood iron levels is essential. Deferasirox treatment is considered necessary, as it is the standard of care, and its side effects are clinically manageable [20, 21].

Aim of the study: This study aimed to examine the satisfaction, convenience, activity limits, and patient preferences between deferasirox and DFO in individuals with SCD.

Methods and Patients

This study was conducted over 1 year and included all patients with SCD attending hospitals in NAJAF city.

Study design to evaluate the safety and tolerability of oral deferasirox in relation to Desferal infusion in SCD with hemosiderosis. In this study, all patients are assessed by questionnaire at baseline, after week 2, 3 months, 6 months, and finally. Patients were randomly assigned in a 2:1 ratio and then received either deferasirox (5-30 mg/kg) or DFO (20-50 mg/kg), with the dosage determined by baseline ferritin levels. Deferasirox was administered orally once daily, while DFO was administered via pump every 8 to 12 hours for five consecutive days.

All patients with SCD aged 2 years or older with serum ferritin levels greater than 1000 ug/L were informed about the study, and written consent was obtained. All patients had a history of more than 12 blood transfusions, and previous chelating therapy was permitted. At baseline and in weeks 2, 3 months, 6 months, and at the conclusion of the trial (1 year), patients assessed their overall satisfaction with the present study drug. The rating scale was five points: (very satisfied), (satisfied), (neutral), (unsatisfied), and (completely unsatisfied). Impaired in routine activities due to therapy, assessed via questionnaire. All patients were requested to report the number of hours spent away from their regular daily activities due to chelation therapy at 2 weeks, 3 months, 6 months, and at the conclusion of the study.

Patients randomized to receive deferasirox, who were previously undergoing deferoxamine therapy, were queried regarding their preference to either maintain the existing treatment or transition to deferasirox. Upon completion of the research, all patients were surveyed using a questionnaire regarding their willingness to continue the chelation therapy administered during the trial.

Statistical analysis:

Statistical analysis was conducted utilizing SPSS software version 17. The analysis encompassed the patient characteristics of individuals receiving deferasirox or DFO. All analyses were performed in patients who received at least one dose of the study drug. The patient's global assessment of treatment satisfaction and convenience was documented by treatment group and assessment time point, using appropriate descriptive statistics for patients previously treated with DFO and for those not receiving any iron chelator before study enrollment. Convenience and satisfaction were assessed using uncomplicated single items with a 5-point answer scale. The analysis was conducted at each time point for satisfaction and convenience. A Chi-square test with Bonferroni correction was used to compare the two treatment groups. P-values less than 0.05 were deemed statistically significant.

Results

A total of 103 individuals were randomized in a 2:1 ratio to receive treatment with oral deferasirox or DFO infusions. A total of 97 patients underwent treatment (66 with deferasirox and 31 with DFO). The treatment groups showed substantial comparability for demographic features (Table 1). At baseline, 41 patients previously treated with DFO were randomized into the deferasirox group, whereas 19 patients were assigned to the DFO group. Among the patients not undergoing chelation therapy before the research commenced, 26 were assigned to the deferasirox group and 12 to the DFO group. Table 1:

Table 01 Baseline Demographic and Clinical Characteristics of the Study Participants

DFO no 12	New DFXj25	Prev on DFO19	Previo on DFX41	DFXno 66	DFO no31	
22±12	20±11.7	17.5±9.6	17±9.2	17.1±9.1	17.2±9.7	Age yr
17(3-50)		14(6-46)	14(4-49)	15(3-44)	14(3-45)	Mean SD
5(41.6%)	9(36%)	9(47.3%)	18(43.9%)	14(43.4%)	26(39%)	Male
7(58.4%)	16(64%)	10(52.7*)	23(56.1%)	17(56.6%)	40(61%)	Femal
0	1(4%)	2(10.5%)	2(4.8%)	2(6.4%)	7(10.7%)	Rural
12(100%)	24(96%)	17(89.5%)	39(95.2%)	29(93.7%)	59(89.3%)	urban

Satisfaction:

Among patients who had previously been treated with DFO, there was no significant difference in the proportion of "very satisfied" or "satisfied" patients between those assigned to DFO and those assigned to deferasirox ($p = 0.13$; Table 1). Thereafter at three mth, six mth, and end of study, more than 83% of patients were very satisfied/satisfied for DFX than DFO (p value 0.001-0.0001). For all patients, at weeks 2, 3mth, 6mth, and end-of-study, significantly more patients in the deferasirox group reported being 'very satisfied' or they would be "satisfied" when compared to the patients detailed in Table 1 who given the treatment. There was no "dissatisfaction" or "complete dissatisfaction" among patients receiving deferasirox, whereas 40-50% of DFO patients were dissatisfied, as shown in Table 2. More than half of the patients in both the deferasirox and DFO groups reported being "dissatisfied" or "completely dissatisfied" with their previous DFO treatment, as shown in Table 2.

Table 02 Patient satisfaction with deferasirox versus deferoxamine (DFO) at follow-up visits and satisfaction with prior DFO therapy.

end 22%	6 months 36%	3 months 40%	2WK 23%	START 28%	Satisfied For DFO
83%	88%	90%	86%	16%	For DFX
>0,001	>0,0001	>0.0001	Less 0.0001	P=0.13	P value

Table 3: Patients who accepted chelation therapy:

Contentment	Pt in the Past Time with DFO				Pt No Chelation Prior				
	Deferosirax 41		DFO 19		Deferasirax 25		DFO 12		
	no	%	no	%	No	%	no	%p value	
Start									
Very sat-sat	7	17,7	6	31.5					
Neutral	9	21.9	1	5,2					
Dissat\very	22	53.6	11	57.9		na			
No answer	3	7.3	1	5.2					
Wk2									
Very sat-sat	35	85.3	5	26.3	18	72	3	25	less0.01
Neutral	2	4.8	2	10.5	3	12	1	8.3	
Dissat\very	0	0	8	42.1	0	0	1	8.3	less0.01
No answer	4	9.7	4	21	4	16	7	58.3	
3mth									
Very sat-sat	36	87,8	8	42.1	17	68	5	41,6	less0.01
Neutral	2	4.8	1	5.2	4	16	3	25	
Dissat\very	0	0	8	42.1	0	0	2	16.6	less0.01
No answer	3	7.3	1	5.2	4	16	2	16.6	
6 mth									
Very sat-sat	35	85.8	7	36.8	17	68	4	33.3	less0.01
Neutral	3	7.3	2	10.5	2	8	2	16.6	
Dissat\very	0	0	8	42.1	0	0	2	16.6	less0.01
No answer	3	7.3	2	10.5	6	24	3	25	
End of study									
Very sat-sat	34	82,9	5	26.3	15	60	3	25	less0.01
Neutral	2	4.8	2	10.5	3	12	3	25	
Dissat\very	0	0	9	47.3	3	12	2	16.6	less0.01
No answer	5	12,1	3	15.7	4	16	4	33.3	

For those who were pDFO (ously on DFO, and chosen to take DFX, at 2 wk, three mth, six mth, and end of study most of them (83%,87%,83%, and 82%), reported that they are delighted with treatment, compare with those who were chosen for DFO(26%,43%,36%, AND 26%) at three mth,6mth, and end of study respectively, which is statically significant(p value 0.01).

Patients who were not receiving chelation therapy and then took the treatment within weeks or months in the DFO group were "very satisfied" or less satisfied" than those in the deferasirox group (p < 0.01). At any given time point, the number of patients in the DFO group who were

"dissatisfied" or "very dissatisfied" was greater than those taking deferasirox ($p < 0.01$), as shown in Table 3.

Preferability:

Initially, a proportion of patients, including those currently receiving DFO, were randomly assigned to deferasirox or DFO. The previous DFO treatment was either "highly preferred" or "preferred" at rates of 17% and 10%, respectively ($p = 0.49$; Table 3). It was found that approximately two-thirds of the patients in the two groups were 63.4% and 68.4%, respectively. In the first six weeks, with patients in the deferasirox group, the treatment was "highly preferred" or "preferred" compared to the DFO group. The probability value is < 0.0001 , and at each time interval, fewer patients in the deferasirox group were "undesirable" or "not at all preferred" compared to the DFO group, as shown in Table 4.

Table 4: Patients prefer the ability for DFO and deferasirox:

preferability	Pt previously on DFO					Pt on no chelation before				
	DFX no 41		DFO 19			DFX 25		DFO 12		
	no	%	no	%	P val	no	%	no	%	P value
Start										
Very preferable\preferable	7	17	2	10.5						0.49
Neutral	5	12.1	3	15.7						
Not preferable\unpref	26	63.4	13	68.4						
No answer	3	7.3	1	5.2						
2WK										
Very preferable\preferable	33	80.4	3	15.7	0.001	18	72	4	33.33	0,0001
Neutral	3	7.3	2	10.5		3	12	3	25	
Not preferable\unpref	1	0.24	10	52.6		0	0	3	25	0,0001
No answer	5	12.1	4	21		4	16	2	16.66	
3month										
Very preferable\preferable	34	82.9	7	36.8	0.001	18	72	3	25	0,0001
Neutral	3	7.3	2	10.5		3	12	3	25	
Not preferable\unpref	1	0.24	8	42.5		0	0	4	33.3	0,0001
No answer	3	7.3	2	10.5		4	16	2	16.6	
6month										
Verypreferable\preferable	33	80.4	7	36.8	0.001	17	68	4	33.3	0,0001
Neutral	5	12.1	3	15.7		1	4	2	16.66	
Not preferable\unpref	0	0	8	42.5		0	0	2	16.66	0,0001
No answer	3	7.3	1	5.2		7	28	4	33.33	
END										
Very preferable\preferable	33	80.4	3	15.7	0.001	16	64	2	16.66	0,0001
Neutral	3	7.3	2	10.5		2	8	4	33.3	
Not preferable\unpref	0	0	12	63.1		1	4	3	25	0,0001
No answer	5	12.1	2	10.5		6	24	3	25	

Among patients who were not undergoing treatment before the trial, at weeks 2, 3 months, 6 months, and at the conclusion of the study, a substantially lower percentage (16.66-33.3%) in the DFO group compared to the deferasirox group (64%-72%) indicated that the treatment was 'extremely preferable' or 'preferable' ($p < 0.0001$), as shown in table 3. A significantly larger proportion of patients receiving DFO compared to those on deferasirox categorized their treatment as 'not preferable or unpreferable' ($p < 0.0001$), as illustrated in Table 4.

Time is extended on routine activities because of treatment:

At 3m, 6m, and the end of the year, those randomized to DFO reported more time loss (37, 34, and 4 hours) in the deferasirox group (2, 2, and 1 hour) per week.

Probability of ongoing therapy. Of the 31 patients undergoing DFO therapy, three patients (9.6%) expressed a willingness to continue the treatment at the conclusion of our study (p value 0.0001), whereas 66 patients were receiving oral therapy. Fifty-five patients (83.4%) are inclined to continue deferasirox therapy. Sixteen patients (51.3%) on DFO expressed unwillingness to continue the medication, whereas only two patients (1.3%) indicated a preference against continuing deferasirox. The principal reasons for the preferences included deferasirox being more convenient to administer (39%), causing less disturbance to patients' daily routines (18%), injection site pain (DFO) (16%), reduced sleep disturbance (6%), and the presence of other factors was estimated at (15%). In comparison, the absence of specific causes was 7%.

Discussion

The principal objective of chelation therapy is to sustain safe iron levels in the body consistently. DFO has been used clinically since the 1970s and has been administered by subcutaneous infusion since around 1980 [27]. DFO is a recognized and efficacious therapy for iron overload, demonstrating favorable long-term safety outcomes. Treatment is time-consuming, uncomfortable, and can negatively impact lifestyle and well-being [12]. Treatment alternatives remain scarce, placing a burden on patients [14]. Deferasirox is a suitable oral iron chelator used once daily worldwide, particularly in the United States and Europe. Published results have shown that deferasirox is equally effective as [20].

Our study found that our patients preferred deferasirox as chelation therapy at 3 m (86%), 6 m (90%), and the end of the study (88% and 83%), respectively. (p -value significant at all points, as being more satisfied and feeling higher convenience as less impact on daily life than DFO, which can be explained by less disturbance, and avoiding annoying pain associated with injections, and sleep disturbance during DFO therapy.

Over 80% of patients who had previously received DFO and were selected for deferasirox reported 'satisfaction' or 'high satisfaction' with the medication, compared with those continuing on DFO. For drug convenience, most patients on deferasirox report greater preference and convenience than those on DFO (80% versus 16.6-33.3%), respectively; the difference is significant. Those patients who prefer to continue DFO therapy explained that their good feeling is due to a change in urine color following subcutaneous injection, which, for them, indicates good excretion of iron from their bodies.

Vikiniscky et al. demonstrate in their multicenter study that more than 60% of patients were satisfied with deferasirox, and about 75% reported greater drug convenience with deferasirox than with DFO [10].

The findings align with a prior study indicating that thalassemia patients reported greater satisfaction with deferasirox than with DFO [22].

Most patients who were on DFO reported greater loss of daily activities (37, 34, and 26 hours) than those on deferasirox (2, 2, and 1 hours) at 3m, 6m, and the end of study, respectively; this is due, of course to much more infusion time of subcutaneous DFO, preparation, and drug injection. This may explain many of the differences in time lost at the end of the study among those on DFO.

55 patients (83%) of those selected for deferasirox were willing to continue their therapy and cited less disruption to their day, less sleep disturbance, and no injection-related soreness as reasons.

They anticipated improved outcomes, enhanced quality of life, and reduced impact on daily activities. Patients who report greater satisfaction with their treatment, as it has shown minimal impact on their lives, tend to adhere to the prescribed regimen and dosage [23].

Treatment adherence is not limited to oral therapy but also includes intravenous therapy, as supported by research conducted in France and Italy through satisfaction questionnaires distributed to patients with iron overload. Researchers have demonstrated that increased satisfaction and potential improvements in lifestyle and quality of life enhance long-term health benefits for patients [24, 25].

This study reveals that 83.4% of patients on oral deferasirox were willing to continue therapy, suggesting that a considerable proportion of patients may remain non-compliant with the newly prescribed medication. As a result, 9.6% of patients on DFO were willing to continue their treatment; this was due to a feeling of better, faster chelation with DFO, as they saw changes in urine color following each injection, which they believed reflected greater iron elimination than with oral therapy. Deferoxamine has been utilized for over 30 years, exhibiting an outstanding safety and efficacy profile. The experience with follow-up trials is insufficient, and the findings supporting its efficacy in eliminating myocardial iron are preliminary [26]. Consequently, patients who exhibit excellent compliance with deferoxamine may opt to continue its use until additional follow-up data are available.

Conclusion:

1. Once-a-day oral deferasirox significantly improves quality of life by increasing patient satisfaction.
2. The preference of most SCD patients for oral deferasirox came from fewer disturbances to their daily activities.
3. deferoxamine (DFO) remains the mainstay of treatment for a significant proportion of patients, as they feel psychological relief from being actively treated and greater effectiveness in chelating iron and promoting its excretion.

Recommendation:

treatment type should be better explained to all patients, and their drug choice should be considered.

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