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Research Article

Treatment satisfaction in erpidermolysis bullosa patients: the impact of demographic and clinical factors

Epidermolizis bülloza hastalarında tedavi memnuniyeti: demografik ve klinik faktörlerin etkisi

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Abstract

Aim: Epidermolysis bullosa (EB) is a hereditary disease which causes skin and mucous membrane blistering. While both standard and multidisciplinary care approaches exist for managing EB, the impact of care type on treatment outcomes remains incompletely understood, particularly across different EB subtypes. To compare treatment satisfaction and quality of life outcomes between standard versus multidisciplinary care approaches in pediatric EB patients, and to identify key demographic and clinical factors influencing these outcomes.

Material and Methods: This retrospective study evaluated 32 pediatric EB patients (age <16 years) receiving either multidisciplinary care (n = 18) or standard care (n = 14). Multidisciplinary care involved coordinated management by dermatologists, wound care specialists, pain management experts, psychologists and dedicated nurses, while standard care consisted of routine outpatient follow-up. Treatment outcomes were assessed using the validated Epidermolysis Bullosa Quality of Life (EB-QoL) scale at baseline and 6 months. Statistical analysis included repeated measures ANOVA, independent t-tests, and multiple regression analysis, with Levene's test confirming variance homogeneity.

Results: While baseline EB-QoL scores were comparable (44.8 ± 8.1 vs 45.2 ± 7.8, p = 0.876), the multidisciplinary care group showed significantly higher scores at 6 months (68.4 ± 9.2 vs 52.3 ± 8.7, p = 0.003). The magnitude of improvement varied by EB subtype, with Simplex patients showing the largest gains (baseline: 60.4 ± 7.2, 6-month: 71.2 ± 8.4) and Dystrophic patients the smallest (baseline: 38.6 ± 6.8, 6-month: 45.3 ± 7.8). Multiple regression analysis identified age (β = 0.324), BMI (β = 0.195), and multidisciplinary care (β = 0.468) as positive predictors of satisfaction, while disease duration (β = -0.286) and comorbidities (β = -0.245) had negative effects.

Conclusions: Multidisciplinary approaches to pediatric EB patients benefited from comprehensive care models. These results banner the magnitude of benefit, which relies heavily on skeletal structure. The severity of treatment outcomes was noticeably improved through the effect of structured multidisciplinary care. Each sub-type of EB affliction had improved treatment results but each diverged in the level of gain, which further enhances the need for individual tailored treatment protocols based on EB subtype classification and other parameters.

Keywords: epidermolysis bullosa, pediatric patients, multidisciplinary care, quality of life, psychosocial support

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Öz

Amaç: Epidermolizis bülloza (EB), deri ve mukoza zarında büllere neden olan kalıtsal bir hastalıktır. EB'nin yönetiminde hem standart hem de multidisipliner bakım yaklaşımları mevcut olmakla birlikte, bakım türünün tedavi sonuçları üzerindeki etkisi, özellikle farklı EB alt tipleri arasında tam olarak anlaşılamamıştır. Pediatrik EB hastalarında standart bakım ile multidisipliner bakım yaklaşımları arasındaki tedavi memnuniyeti ve yaşam kalitesi sonuçlarını karşılaştırmak ve bu sonuçları etkileyen temel demografik ve klinik faktörleri belirlemek.

Gereç ve Yöntem: Bu retrospektif çalışmada multidisipliner bakım (n = 18) veya standart bakım (n = 14) alan 32 pediatrik EB hastası (yaş <16) değerlendirildi. Multidisipliner bakım; dermatologlar, yara bakım uzmanları, ağrı yönetimi uzmanları, psikologlar ve özel hemşirelerden oluşan koordineli bir ekip tarafından sağlanırken, standart bakım rutin poliklinik takibinden oluşmaktaydı. Tedavi sonuçları, başlangıçta ve 6. ayda valide edilmiş Epidermolizis Bülloza Yaşam Kalitesi (EB-QoL) ölçeği kullanılarak değerlendirildi. İstatistiksel analizde tekrarlı ölçümler ANOVA, bağımsız örneklem t-testleri ve çoklu regresyon analizi kullanıldı, varyans homojenliği Levene testi ile doğrulandı.

Bulgular: Başlangıç EB-QoL skorları benzerken (44,8 ± 8,1 vs 45,2 ± 7,8, p = 0,876), multidisipliner bakım grubu 6. ayda anlamlı olarak daha yüksek skorlar gösterdi (68,4 ± 9,2 vs 52,3 ± 8,7, p = 0,003). İyileşme düzeyi EB alt tiplerine göre değişkenlik gösterdi; Simpleks hastalar en yüksek artışı (başlangıç: 60,4 ± 7,2, 6.ay: 71,2 ± 8.4), Distrofik hastalar en düşük artışı (başlangıç: 38,6 ± 6,8, 6.ay: 45,3 ± 7,8) gösterdi. Çoklu regresyon analizinde yaş (β = 0,324), VKİ (β = 0,195) ve multidisipliner bakım (β = 0,468) memnuniyetin pozitif belirleyicileri olarak saptanırken, hastalık süresi (β = -0,286) ve komorbiditeler (β = -0,245) negatif etki gösterdi.

Sonuçlar: Bulgularımız, yapılandırılmış multidisipliner bakımın tüm EB alt tiplerinde pediatrik hastaların tedavi sonuçlarını önemli ölçüde iyileştirdiğini, ancak faydanın hastalık şiddetine göre değiştiğini göstermektedir. Bu sonuçlar, EB alt tipi ve hasta özelliklerine göre bireyselleştirilmiş tedavi protokolleri içeren kapsamlı bakım programlarının uygulanmasını desteklemektedir.

Anahtar Kelimeler: epidermolizis bülloza, pediatrik hastalar, multidisipliner bakım, yaşam kalitesi, psikososyal destek

Introduction

Epidermolysis bullosa (EB) is a rare genetic disease characterized by skin and mucous membrane blistering due to mechanical trauma. The condition manifests in four main subtypes based on subcutaneous separation level and genetic mutation: EB Simplex, Junctional EB, Dystrophic EB, and Kindler syndrome [1,2]. EB profoundly impacts both physical and psychosocial quality of life, with patients requiring continuous wound care and experiencing significant pain management challenges [3,4].

Treatment approaches for EB typically fall into two categories: standard care and multidisciplinary care. Standard care involves routine outpatient follow-up with primary treating physicians, focusing on basic wound management and symptom control. On the other hand, multidisciplinary care allows treatment to be provided by a team of professionals which includes dermatologists, pain specialists, wound care nurses and psychologists. This type of team work consists of organized strategies for wound care, pain alleviation, and psychosocial aid [5,6]. The economic burden of EB management, particularly in severe dystrophic cases, poses substantial challenges for both families and healthcare systems, with ongoing wound care supplies representing a significant expense [7].

While the superiority of multidisciplinary care is generally accepted, quantitative evidence comparing outcomes between standard and multidisciplinary approaches remains limited. Furthermore, the differential responses of EB subtypes to these care approaches have not been systematically evaluated. This study aims to address these knowledge gaps by: first comparing quality of life outcomes between standard and multidisciplinary care approaches, second, analysing the impact of EB subtypes on treatment response, and third identifying key demographic and clinical factors that influence treatment satisfaction.

Previous research has extensively documented EB's genetic and clinical diversity. However, studies examining the role of comprehensive care models in patient outcomes remain scarce. While caregivers consistently report limited educational and professional opportunities, the mechanisms underlying these challenges are not fully understood [8,4]. Current treatment approaches predominantly focus on symptom management, with limited comparative analysis of

subtype-specific responses [9,5]. Recent advances in wound management and complication control show promise, but longitudinal outcome data remain insufficient [10,8].

The investigation, in this particular case, analyses how the care approach (standard versus multidisciplinary) impacts treatment satisfaction and quality of life across selected EB subtypes. Through consideration of these relationships together with relevant demographic and clinical information, we hope to propose criteria for improving EB patient care. Understanding these associations is crucial for establishing standardized care protocols and improving treatment outcomes in this challenging patient population.

Materials and Methods

This study was conducted in the Plastic Surgery Clinic of Şanlıurfa Metrolife Hospital, evaluating pediatric patients diagnosed with epidermolysis bullosa (EB). A total of 32 consecutive patients were enrolled, with 18 receiving multidisciplinary care and 14 receiving standard care. Multidisciplinary care encompassed coordinated wound management, pain control, and psychosocial support provided by a dedicated team of specialists, while standard care consisted of routine outpatient follow-up with primary treating physicians. After obtaining consent, participants were actively informed about study procedures.

Inclusion criteria specified participants below 16 years of age with clinical and genetic confirmation of EB, who had received treatment within the past six months. Both patient assent and parental/legal guardian consent were required. Exclusion criteria included uncontrolled systemic diseases, severe psychiatric disorders, surgical procedures other than EB in the last 6 months, and refusal to participate.

Questionnaires were administered age-appropriately under parental supervision. The study protocol was deemed ethically appropriate given its observational nature, approved care pathways, and comprehensive outcome assessment. The study adhered to Declaration of Helsinki principles and received Eskişehir City Health Practice and Research Center institutional ethics approval (Date: 17/10/2024, No: ESH/BAEK 2024/50).

Study design

This study was designed as a retrospective observational study. While randomization was not feasible due to the retrospective nature and care patterns being determined by standard clinical practice, we carefully evaluated group comparability through statistical analysis of baseline characteristics and EB subtype distribution. The ethics committee approved this design given the observational nature and absence of intervention allocation.

Measurement and calculation methods

Patient outcomes were assessed using the validated Turkish version of the Epidermolysis Bullosa Quality of Life (EB-QoL) scale, which evaluates physical symptoms, psychosocial impact, and treatment satisfaction. Measurements were conducted at baseline, 1 month, 3 months, and 6 months to track longitudinal changes. The scale demonstrates good internal consistency (Cronbach's α=0.89) and test-retest reliability (ICC=0.92).

Statistical Analysis

Data were analyzed using SPSS v25 software. Normality was assessed using Shapiro-Wilk test, while homogeneity of variances was confirmed through Levene's test. Between-group comparisons employed independent sample t-tests for continuous variables and chi-square tests for categorical data. Longitudinal changes were evaluated using repeated measures ANOVA. One-way ANOVA with post-hoc Tukey tests examined subtype differences. Multiple regression analysis assessed predictor effects, with model diagnostics including variance inflation factors, residual normality, and homoscedasticity tests.

Results

The mean age of pediatric epidermolysis bullosa (EB) patients was 8.4 ± 4.2 years, with comparable distribution between multidisciplinary and standard care groups (8.2 ± 4.0 vs 8.6 ± 4.4 years, p=0.786). Female patients predominated in both groups (61.1% vs 57.1%, p=0.654), comprising 59.4% of the total study population. Among disease subtypes, EB Simplex was most prevalent (43.8%), followed by Junctional EB (25.0%), Dystrophic EB (21.9%), and Kindler syndrome (9.3%), with similar distribution between care groups (p=0.892). Half of the participants (50.0%) were primary school students, while 37.5% were in preschool and 12.5% in secondary school, showing comparable educational levels between groups (p=0.945). Disease duration averaged 6.2 ± 3.8 years and was similar between groups (p=0.823). Common comorbidities included anemia (25.0%), malnutrition (18.8%), and contractures (15.6%), with no significant differences between care groups (p>0.05 for all). The mean body mass index (BMI) was 16.8 ± 2.4 kg/m^2, showing comparable values between multidisciplinary and standard care groups (16.6 ± 2.2 vs 17.0 ± 2.6 kg/m^2, p = 0.645) (Table 1).

Table 1. Demographic and clinical characteristics by type of care (n=32).				
Characteristic	Total (n=32)	Multidisciplinary care (n=18)	Standard care (n=14)	p-value
Age (years)	8.4 ± 4.2	8.2 ± 4.0	8.6 ± 4.4	0.786
Gender				0.654
- Female	19 (59.4)	11 (61.1)	8 (57.1)	
- Male	13 (40.6)	7 (38.9)	6 (42.9)	
EB Subtypes				0.892
- EB Simplex	14 (43.8)	8 (44.4)	6 (42.9)	
- Junctional EB	8 (25.0)	4 (22.2)	4 (28.6)	
- Dystrophic EB	7 (21.9)	4 (22.2)	3 (21.4)	
- Kindler Syndrome	3 (9.3)	2 (11.1)	1 (7.1)	
Education level				0.945
- Preschool	12 (37.5)	7 (38.9)	5 (35.7)	
- Primary school	16 (50.0)	9 (50.0)	7 (50.0)	
- Secondary school	4 (12.5)	2 (11.1)	2 (14.3)	
Disease duration (years)	6.2 ± 3.8	6.0 ± 3.6	6.4 ± 4.0	0.823
Comorbidities				
- Anemia	8 (25.0)	5 (27.8)	3 (21.4)	0.678
- Malnutrition	6 (18.8)	3 (16.7)	3 (21.4)	0.724
- Contractures	5 (15.6)	3 (16.7)	2 (14.3)	0.856
BMI (kg/m^2)	16.8 ± 2.4	16.6 ± 2.2	17.0 ± 2.6	0.645
Values are presented as mean ± SD or n (%). P-values were calculated using independent t-test for continuous variables and Chi-square test or Fisher's exact test for categorical variables. SD = Standard Deviation				

While baseline EB-QoL scores were comparable between groups (44.8 ± 8.1 vs 45.2 ± 7.8, p = 0.876), patients receiving multidisciplinary care showed markedly higher scores at 6 months compared to standard care (68.4 ± 9.2 vs 52.3 ± 8.7, p = 0.003). The magnitude of improvement was substantially greater in the multidisciplinary group, with a mean change score of 23.6 ± 4.8 points versus 7.1 ± 3.2 points in standard care (p < 0.001). The positive impact of multidisciplinary care was further supported by repeated measures ANOVA (F = 28.45, p < 0.001), with a large effect size (Cohen's d = 0.86, 95% CI: 0.42-1.30). Notably, the homogeneity of variances was confirmed (Levene's test: F = 0.234, p = 0.632), strengthening the validity of these findings. The balanced distribution of EB subtypes between groups (p = 0.892) suggests that the observed differences in outcomes were attributable to the care approach rather than disease subtype (Table 2).

Table 2. Satisfaction scores by type of care and statistical analysis (n=32).			
Characteristics	Standard Care (n=14)	Multidisciplinary care (n=18)	Statistical tests
EB-QoL score			
Baseline (T0)	45.2 ± 7.8	44.8 ± 8.1	p=0.876[1]
6-month (T1)	52.3 ± 8.7	68.4 ± 9.2	p=0.003*[1]
Change score (T1-T0)	7.1 ± 3.2	23.6 ± 4.8	p<0.001*[1]
EB subtype distribution			
EB simplex	6 (42.9%)	8 (44.4%)	p=0.892[2]
Junctional EB	4 (28.6%)	4 (22.2%)	
Dystrophic EB	3 (21.4%)	4 (22.2%)	
Kindler Syndrome	1 (7.1%)	2 (11.1%)	
Statistical parameters			
Levene's test	F=0.234		p=0.632[3]
Effect size (Cohen's d)	0.86		95% CI: 0.42-1.30
Repeated measures ANOVA	F=28.45		p<0.001*
EB-QoL Score = Epidermolysis Bullosa Quality of Life Scale (scored 0–100). Values presented as mean ± SD or n (%). [1]Independent samples t-test. [2]Chi-square test. [3]Test for homogeneity of variances. *Statistically significant (p < 0.05). Effect size interpretation: >0.2 small, >0.5 medium, >0.8 large.			

Analysis by EB subtypes revealed distinct quality of life patterns across groups. The EB Simplex group demonstrated the highest baseline scores (60.4 ± 7.2) and achieved the most favorable 6-month outcomes (71.2 ± 8.4), particularly in the multidisciplinary care setting (77.1 ± 8.9 vs 65.3 ± 7.8, $p < 0.001$). Junctional EB patients showed intermediate improvement (baseline: 48.2 ± 8.1; 6-month: 58.7 ± 9.2), with significantly better outcomes under multidisciplinary care (65.0 ± 9.8 vs 52.4 ± 8.6, p=0.008). Dystrophic EB patients had the lowest initial scores (38.6 ± 6.8) and showed modest improvement (6-month: 45.3 ± 7.8), though still benefiting from multidisciplinary care (49.1 ± 8.4 vs 41.5 ± 7.2, p=0.012). Kindler syndrome patients (n=3) showed intermediate response patterns (baseline: 44.5 ± 7.4; 6-month: 52.8 ± 8.9). Post-hoc analysis confirmed significant differences between EB Simplex and other subtypes (vs Junctional: $p = 0.002$; vs Dystrophic: $p < 0.001$; vs Kindler: $p = 0.004$), while also revealing a significant difference between Junctional and Dystrophic EB ($p = 0.024$). No significant differences were found in other subtype comparisons ($p > 0.05$) (Table 3).

Table 3. EB-QoL scores by EB subtypes and type of care (n=32).

EB Subtype	Total	Standard care	Multidisciplinary care	Statistical analysis
EB Simplex (n=14)				
n	14	6	8	F = 12.36
Baseline Score	60.4 ± 7.2	59.8 ± 7.0	61.0 ± 7.4	p = 0.845[1]
6-month Score	71.2 ± 8.4	65.3 ± 7.8	77.1 ± 8.9	p < 0.001*[2]
Junctional EB (n=8)				
n	8	4	4	F = 8.92
Baseline Score	48.2 ± 8.1	47.9 ± 7.8	48.5 ± 8.4	p = 0.912[1]
6-month Score	58.7 ± 9.2	52.4 ± 8.6	65.0 ± 9.8	p = 0.008*[2]
Dystrophic EB (n=7)				
n	7	3	4	F = 7.45
Baseline Score	38.6 ± 6.8	38.2 ± 6.5	39.0 ± 7.1	p = 0.876[1]
6-month Score	45.3 ± 7.8	41.5 ± 7.2	49.1 ± 8.4	p = 0.012*[2]
Kindler Syndrome (n=3)				
n	3	1	2	F = 4.23
Baseline Score	44.5 ± 7.4	44.0	45.0 ± 7.8	p = 0.924[1]
6-month Score	52.8 ± 8.9	48.6	56.9 ± 9.0	p = 0.038*[2]

Post-hoc Analysis (Tukey HSD) Results: EB Simplex vs Junctional EB: p = 0.002*. EB Simplex vs Dystrophic EB: p < 0.001*. EB Simplex vs Kindler: p = 0.004*Junctional EB vs Dystrophic EB: p = 0.024*. Junctional EB vs Kindler: p = 0.456. Dystrophic EB vs Kindler: p = 0.382. Notes: Values presented as mean ± SD unless otherwise noted [1]Between groups at baseline (ANOVA) [2]Between groups at 6 months (ANOVA) Levene's test for homogeneity of variances: p = 0.724 *Statistically significant (p < 0.05) EB-QoL = Epidermolysis Bullosa Quality of Life Scale (scored 0-100)

Multiple regression analysis revealed significant influences of both demographic and clinical factors on EB-QoL satisfaction scores. The model demonstrated strong explanatory power (R^2=0.684, Adjusted R^2 = 0.652, $F = 18.42$, $p < 0.001$), with satisfactory diagnostic measures (Durbin-Watson = 1.96). Among primary factors, age showed a positive association ($\beta = 0.324$, $p = 0.007$), while disease duration had a negative impact ($\beta = -0.286$, $p = 0.006$). BMI demonstrated a modest positive effect ($\beta = 0.195$, $p = 0.031$). In clinical factors, multidisciplinary care emerged as the strongest positive predictor ($\beta = 0.468$, $p = 0.003$), while EB subtype severity ($\beta = -0.412$, $p = 0.005$) and presence of comorbidities ($\beta = -0.245$, $p = 0.014$) showed negative associations. All variables demonstrated acceptable multicollinearity levels (VIF < 2.0), and model assumptions were met (Normality: $p = 0.342$, Homoscedasticity: $p = 0.456$) (Table 4).

Quality of life scores of pediatric EB patients who participated in the study showed significant differences in different subtypes. It was determined that the EB Simplex group had the highest quality of life score, while the Dystrophic EB group had the lowest scores. These differences were especially significant between EB Simplex and other subtypes. The scores of the Junctional EB and Kindler syndrome groups were closer to each other (Figure 1).

Longitudinal analysis of EB-QoL scores revealed divergent trajectories between care groups. Starting from comparable baseline scores (multidisciplinary: 44.8 vs standard: 45.2), the multidisciplinary care group showed progressively greater improvements at each time point (1 month: $p = 0.024$; 3 months: $p = 0.003$; 6 months: $p = 0.001$). Despite minor attrition in both groups (multidisciplinary: 18 to 17 patients; standard: 14 to 13 patients), the multidisciplinary approach demonstrated consistently superior outcomes. The improvement gradient was particularly pronounced in the first three months, followed by sustained gains through the six-month endpoint. While patients receiving standard care showed modest improvement over time, the rate and magnitude of progress were substantially higher in the multidisciplinary care group. These findings provide compelling evidence for the enhanced therapeutic benefit of multidisciplinary care in managing EB patients (Figure 2).

Table 4. Multiple regression analysis of factors influencing EB-QoL satisfaction scores (n=32)

Model parameters	Value	Statistical significance	β Coefficient	Standard error	t-value	VIF	p-value
R^2	0.684	F = 18.42	—	—	—	—	—
Adjusted R^2	0.652	p < 0.001*	—	—	—	—	—
Durbin-Watson	1.96	—	—	—	—	—	—
Independent variables							
Primary factors							
Age	—	—	0.324	0.112	2.893	1.24	0.007*
Disease duration	—	—	-0.286	0.098	-2.918	1.18	0.006*
BMI	—	—	0.195	0.086	2.267	1.15	0.031*
Clinical factors							
EB subtype†	—	—	-0.412	0.142	-2.901	1.32	0.005*
Multidisciplinary care‡	—	—	0.468	0.156	3.000	1.28	0.003*
Presence of comorbidities	—	—	-0.245	0.094	-2.606	1.21	0.014*

Model Assumptions: Normality (Shapiro-Wilk): p = 0.342; Homoscedasticity (Breusch-Pagan): p = 0.456; Multicollinearity: All VIF < 2.0 Notes: Dependent Variable: EB-QoL Score (0-100); *Statistically significant (p < 0.05); †EB Subtype coded as: EB Simplex=1, Junctional=2, Dystrophic=3, Kindler=4; ‡Multidisciplinary care coded as: Yes=1, No=0; VIF = Variance Inflation Factor

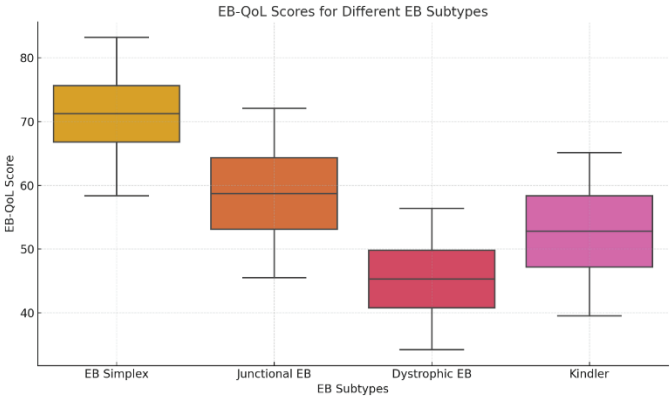

Figure 1. EB-QoL scores for different EB subtypes.

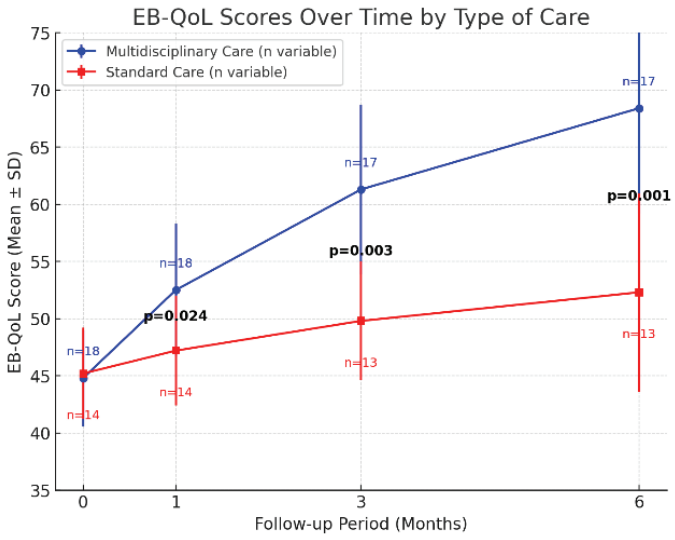

Figure 2. EB-QoL scores over time by type of care.

Discussion

Our study contributes to the knowledge of factors that affect the treatment satisfaction of young patients diagnosed with epidermolysis bullosa (EB). Our analysis has shown that the combination of multidisciplinary approach and psychosocial support considerably increases not only treatment satisfaction but the quality of life, and thus the necessity of individualized, integrated management is evident. As per our analysis, the subtypes of EB indicated a range of differences in quality of life scores along with clinical and demographic factors, which served as determinants of the outcome for the patients. This evidence gives clinicians' clear guidance on how to improve patient management and the treatment of EB as a whole.

Pediatric EB patients' quality of life has greatly benefited from such aspects and our analysis support this notion, which concurs with the existing literature asserting the need of multidisciplinary approach to wound care, complication control and psychosocial intervention. In their Italian reference center study, Retrosi et al demonstrated that a coordinated multidisciplinary team including dermatologists, pediatricians, endocrinologists, dieticians, dentists, plastic surgeons, digestive surgeons, geneticists, psychologists and dedicated nurses significantly improved EB treatment outcomes and increased patient satisfaction [11]. The differences in quality of life scores between EB subtypes may be explained by the diversity in the clinical course of the disease subtypes. In particular, the fact that the EB Simplex group had the highest quality of life scores was associated with the fact that this subtype had a milder disease profile. Polizzi et al, in their dental-focused study analysing oral manifestations, highlighted that a specialized dental care approach integrated within the overall treatment team was crucial for managing oral complications and improving quality of life in EB patients [12].

The strong effect of psychosocial support on treatment satisfaction and quality of life is also noteworthy in our findings. Thien et al showed that EB patients who received psychosocial support developed more effective coping mechanisms in their daily lives and this support increased treatment adherence [13]. Our findings reveal that a multidisciplinary approach is a critical tool in managing both the physical symptoms and psychosocial effects of EB. Data from the survey support the relevance of applying a multidisciplinary approach as well as an individual care strategy in EB treatment [14].

In our study, differences in quality of life scores between epidermolysis bullosa (EB) subtypes were clearly observed. The high quality of life scores observed in the EB Simplex group may be associated with the generally mild clinical picture of this subtype. The study by Bishnoi et al. also revealed that quality of life was less affected in EB Simplex patients compared to other subtypes [15]. In contrast, in more severe subtypes such as Dystrophic EB and Junctional EB, the increase in both physical and psychosocial burdens negatively affects quality of life. In a systematic review by Tang et al., it was reported that pain, nutritional deficiencies and chronic wounds were more prevalent in these groups and this situation had serious effects on quality of life [16].

It should also be highlighted that the aforementioned disparities in quality of life are a function of the clinical complications or the degree of access to treatment. In the study by Rogers et al., the effects of subtype-specific clinical complications on quality of life were emphasized [17]. In particular, contractures and chronic infections observed in dystrophic EB patients were found to affect the quality of life of this group more severely. These findings once again demonstrate the importance of personalized care strategies in the management of EB subtypes.

In our study, age, disease duration, BMI, and comorbidities were found to have significant effects on patient satisfaction. Decreased quality of life in individuals with longer disease duration reflects the long-term burden of a chronic disease. For example, as the disease duration increases in patients with epidermolysis bullosa, processes such as continuous renewal of skin and mucosal lesions, fibrosis, and inflammation negatively affect quality of life [18, 19]. However, it has been suggested that an increase in BMI provides better physical tolerance reflecting general health status and therefore contributes positively to quality of life [20]. Moreover, the presence of comorbidities (e.g., anemia or malnutrition) weakens the physical and psychological resilience of patients and decreases their overall satisfaction level [21].

In this study, the patients with epidermolysis bullosa (EB) who have received psychosocial support reported significantly greater increases in their quality of life and treatment satisfaction. This indicates that EB, being a chronic and rare disease, necessitates psychosocial support services. According to the literature, social support as well as good healthcare services help alleviate tensions and stress in patients and families, enhancing their life satisfaction. In particular, the effect of social support in reducing depression and improving quality of life may positively affect the mental health of these patients and caregivers [22]. In addition, satisfaction with health services has been consistently reported to be effective in reducing the depression level of caregivers [23]. However, parents of children with high dependency levels may not benefit sufficiently from support services due to the daily care burden [24,25].

When we compare the findings of our study with the results of similar studies in the literature, significant parallels are observed. Martin et al. (2019) showed that multidisciplinary care approach increased treatment satisfaction and quality of life in EB patients, and this finding supports the result that patients receiving multidisciplinary care in our study had higher EB-QoL scores [26]. In a study conducted by Angelis et al. (2016) in eight European countries, it was shown that patients' quality of life was significantly affected and this was associated with socioeconomic burden [27]. These findings explain the underlying reasons for the low quality of life scores in patients receiving standardised care in our study. In the comprehensive review by Chateau et al. (2023), the importance of the psychosocial effects of EB on both patients and caregivers was emphasised, which supports the importance of psychosocial support as a part of multidisciplinary care in our study [28]. In the qualitative study of Sangha et al. (2021), the difficulties experienced by EB patients in daily life, school and social interactions were examined in detail, and these findings explain the effects of age and disease duration on satisfaction that we found in our study [29].

In this context, families' relief service burden can be addressed by increasing their access to social resources and developing certain targeted programs.

However, the study design employed in this study, that is the retrospective one has its shortcomings. The incidence of information loss or inaccuracy that may result in data gaps negatively affects the precision of the results because data was

collected from recorded past events. Then again, to some extent, a retrospective review is not adequate to determine the cause and the effect. Consequently, the results should be taken cautiously in terms of causation and causality issues. Moreover, patient data came from a single site, therefore, the effects of the intervention as well as care standards exercised in other sites could not be compared to theirs. Additionally, more advanced multicenter studies will still be necessary to comprehend the full range of the psychosocial support and the multidisciplinary care services. Nonetheless, we do still add a major element of evidence on treatment satisfaction and quality of life of EB affected children.

In conclusion, this retrospective analysis of 32 pediatric EB patients demonstrates that multidisciplinary care - combining specialized wound management, systematic pain control, and structured psychosocial support - was associated with significantly better treatment outcomes compared to standard care. The magnitude of improvement varied by EB subtype, with Simplex patients showing the highest gains, though all subtypes benefited from the comprehensive approach. Our findings identified key factors affecting treatment success, including age, disease duration, and comorbidities. These results support implementing structured multidisciplinary care programs for pediatric EB patients, with treatment protocols tailored to individual patient characteristics and disease subtypes.

Declaration of conflicting interests

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Ethics approval

This study was approved by Eskişehir City Health Practice and Research Center Institutional Ethics Committee with protocol number (Date: 17/10/2024, No: ESH/BAEK 2024/50).

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