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ARTICLE

Investigation and Analysis of the Current Status of Health-Related Quality of Life in Adolescents with Hemophilia in Hubei Province

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Abstract

Aims/Background Reliable health-related quality of life data are critical in developing countries, in order to advocate for government agencies to develop national hemophilia care programmes. This study aims to explore the current status and influencing factors of health-related quality of life among adolescents with hemophilia in Hubei Province, so as to provide empirical data for professionals.

Methods A total of 84 children with hemophilia aged 8 to 18, who were registered in Tongji Hemophilia Treatment Center and Hubei Hemophilia Home, were selected using a cluster sampling method. The "General Situation Questionnaire of Hemophiliac Adolescents", designed by Tongji Hemophilia Treatment Center, and "the Chinese version of Canadian hemophilia outcomes-kid's life assessment tool (CHO-KLAT)", were used for this study conducted from June 1, 2022 to December 30, 2022.

Results 82 completed questionnaires were included. The average age of the 82 adolescents was 13.04 ± 3.29 years and all were males. Among them, 67 were hemophilia A and 15 were hemophilia B. 61 cases were severe type, 19 were moderate type and 2 cases were mild type. The average total score of the CHO-KLAT for adolescents with hemophilia in Hubei Province was 49.49, which was lower than their counterparts in developed countries. The statistically significant influencing factors included residence, annual family income, and disease type.

Conclusion This study provides empirical data support for the health management of adolescents with hemophilia, highlighting the importance of improving medical resource access, transfusion convenience, and psychological support in enhancing the quality of life for this group. The results emphasize the need for healthcare systems and policymakers to take specific measures to address these factors to improve the treatment and care conditions for adolescents with hemophilia.

Key words: Hubei Province; adolescents with hemophilia; health-related quality of life; influencing factors

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Introduction

Hemophilia is a group of X-chromosome-linked hereditary lifelong hemorrhagic disorders and is a rare disease worldwide (Thrombosis and hemostasis Group et al, 2020). Patients experience various degrees of bleeding according to the severity of the disease (mild type/moderate type/severe type), primarily affecting the joints, muscles, and soft tissues (Lee Mortensen et al, 2018). Recurrent joint bleeds cause long-term complications including pain, arthropathy, and disability (Breakey et al, 2014). Unfortunately, there is currently no radical cure for hemophilia. Patients need prophylactic or on-demand injections of the missing coagulation factors to prevent bleeding, or hemostatic treatment (Thrombosis and hemostasis Group et al, 2020; Srivastava et al, 2020).

Health-related quality of life evaluation is recognized as an important outcome indicator in the assessment of boys with hemophilia (Lambert et al, 2020). In fact, reliable health-related quality of life data are even more critical in developing countries, in order to advocate for government agencies to develop national hemophilia care programmes (Lambert et al, 2020). The investigation and analysis of factors influencing the quality of life among adolescents with hemophilia has emerged as a prominent research topic in developed countries (Lambert et al, 2020; Srivastava et al, 2020; Wu et al, 2012). However, there is limited research on this subject in China (Wang et al, 2020; Wu et al, 2012). This study aims to investigate the current quality of life among adolescents with hemophilia in Hubei Province, located in central China, and analyze the associated influencing factors. The results will serve as a reference for rare disease care organizations, Hemophilia Treatment Centers, and relevant policy-making institutions for the development of hemophilia care programmes.

Methods

Participants

We used cluster sampling to identify a total of 84 adolescents with hemophilia aged 8 to 18 years, who were registered in Tongji Hemophilia Treatment Center and Hubei Hemophilia Home and met the inclusion criteria. Inclusion criteria were: Diagnosed as hemophilia A or B according to the missing type of coagulation factors. The diagnostic criteria were from Chinese Guidelines for Hemophilia Treatment (2020 edition) (Thrombosis and hemostasis Group et al, 2020); Between 8 and 18 years of age; Had independent literacy and understanding skills and could complete the questionnaire independently or under the guidance of parents; Volunteered for this survey and provided informed consent. Exclusion criteria: Could not understand the content of the questionnaire; The child or caregiver did not agree to participate in the survey; Other congenital genetic diseases, developmental abnormalities, other serious diseases or complications unrelated to hemophilia; History of major bleeding in the past year.

Methods

Investigation Tools

The “General Situation Questionnaire of Hemophiliac Adolescents”, designed by the Tongji Hemophilia Treatment Center, and “the Chinese version of Canadian hemophilia outcomes-kid’s life assessment tool (CHO-KLAT) (Young et al, 2006)” were used for this study conducted from June 1, 2022 to December 30, 2022.

The questionnaire is composed of basic information, disease and diagnosis, self-management behavior, and difficulties and needs in diagnosis and treatment of children with hemophilia. The basic information includes a total of 10 items, such as the child's gender, date of birth, place of residence, medical insurance, family income, the proportion of treatment costs to family income, the relationship between the caregiver and the child, the caregiver's education and occupation, and health status. Diseases and diagnosis and treatment included a total of 10 items, including hemophilia classification, concentration of coagulation factors, time of first bleeding, location of bleeding, treatment plan, home treatment or nearby treatment, joint disability, status of inhibitors and concentration, and the time of first treatment. Self-management behaviors include self-injection behaviors, communication behaviors with doctors and nurses, disease-related knowledge acquisition behaviors, and independently access medical treatment behaviors. The difficulties and needs in the process of diagnosis and care include two open-ended questions: "what is the biggest difficulty in the process of diagnosis and care" and "what kind of help do you most want".

The CHO-KLAT questionnaire was developed in Canada by Young et al (2006). This questionnaire is a disease-specific, child-centric, health-related quality-of-life measure for boys with haemophilia (Young et al, 2006). The CHO-KLAT is used for children aged 4 to 18 years old and includes both children's self-reported and parent-proxy-reported questions (Young et al, 2006). The CHO-KLAT contains 35 items including treatment, physical health, family, future, feeling, understanding of hemophilia, others and friends, and taking control of your own life, and is scaled from 0 to 100 (Wang et al, 2020; Wu et al, 2012; Young et al, 2006). It has been validated against the Pediatric Inventory of Quality of Life (Wu et al, 2012). A 5-point Likert scale is used to evaluate the frequency of an event in the past 4 weeks from "never" to "always". Questions 22 to 35 utilize a 6-point Likert scale, from "never" to "always" in the past 4 weeks, and each response item also includes a "not applicable" option; when "not applicable" is chosen for questions 22, 25, 30, 31, 32, 33, and 35, the system will treat the response as a missing item. The total score of the questionnaire is the average score of each item, and the higher the score, the better the quality of life (Wang et al, 2020; Wu et al, 2012; Young et al, 2006).

Investigation Methods

Three nurses (ZLX and TZ and XZ) at Tongji Hemophilia Treatment Center informed all adolescents with hemophilia and their caregiving parents, who were registered in Tongji Hemophilia Treatment Center and Hubei Hemophilia Home, about the purpose and significance of the investigation through telephone or WeChat communication. For adolescents with hemophilia who met the inclusion criteria and their caregiving parents, nurses made an appointment with them to sign the informed consent and fill in the questionnaire under the guidance of the nurses during their outpatient visits, follow-up visits, hospitalization, or on-site public welfare activities such as self-management knowledge and skills training, family fellowship activities, multidisciplinary voluntary clinics, joint evaluation, and so on. The nurse was present during completion of the form, but for the items that were not understood, only objective explanation was given without any suggestion. A total of 84 adolescents with hemophilia met the inclusion criteria, among which 2 adolescents with mild hemophilia had not experienced joint bleeding and clotting factor infusion in the last year, and the questionnaire had 14 "not applicable" responses, which were judged to be invalid. The remaining 82 adolescents with hemophilia completely filled in the questionnaire, and the effective rate of the questionnaire was 97.62%.

Statistical Methods

Data were analyzed using SPSS 26.0 (IBM-SPSS Statistics, Chicago, IL, USA). Normality tests for continuous variables were performed using the Kolmogorov–Smirnov test. Normally distributed continuous variables were expressed as mean ± standard deviation (SD) and comparisons between two groups were performed using the *t*-test. Comparisons between multiple groups were performed using one-way analysis of variance with Least Significant Difference for post hoc multiple comparisons. p values < 0.05 were considered a statistically significant.

Results

General Situation of Adolescents with Hemophilia in Hubei Province

All 82 adolescents with hemophilia in this survey were male, with an average age of 13.04 ± 3.29 years old; There were 67 cases of hemophilia A and 15 cases of hemophilia B. 61 cases were diagnosed as severe type, 19 as moderate type, and 2 as mild type. See Table 1 for details.

Two open-ended questions included "what is the biggest difficulty in the process of diagnosis and care" and "what kind of help do you most want". 15 adolescents with hemophilia expressed the hope of psychological counseling and social support. Two of them thought their lives were a heavy burden on their family, and were hoping to die early, so that they and their families could be relieved. One adolescent admitted that he had failed to commit suicide. 21 adolescents said they were not understood by people around them and were kept away from their neighbors. They often felt helpless and lonely because they could not tell their true feelings to others.

CHO-KLAT Scores of Adolescents with Hemophilia in Hubei Province

In this study, the average total score of the CHO-KLAT was 49.49, with the maximum score being 78.13 and the minimum score being 20.71. These values are lower than their counterparts in developed countries, and are close to their domestic counterparts. The average score for 39 hemophilia adolescents living in cities was 54.08 ± 15.00 points, whereas that of their 43 counterparts living in rural areas was 45.33 ± 15.11 points. This difference was significant (p = 0.010), indicating that residence was a significant influencing factor on the quality of life of these adolescents. Annual family income had a proportion relation with the score. The average score of 49 adolescents whose annual family income <50,000 RMB was 45.02 ± 15.58 points, whereas the average for those with annual family income >200,000 was higher (67.50 ± 2.53 points, p = 0.003). This indicates that the

Table 1. General characteristics of hemophiliac adolescents in Hubei Province (n = 82).

	Case number	Percentage (%)
Age		
6–10 years	22	26.83
11–14 years	33	40.24
>14 years	27	32.93
Residence		
City	39	47.56
Rural areas	43	52.44
Annual family income (RMB)#		
<50,000	49	59.76
50,000–100,000	23	28.05
100,000–200,000	8	9.76
>200,000	2	2.44
Primary caregiver		
Father	8	9.76
Mother	70	86.58
Other	4	3.66
Annual treatment cost (RMB)#		
<10,000	2	2.44
10,000–50,000	47	57.32
50,000–100,000	22	26.83
>100,000	11	13.41
Hemophilia type		
Hemophilia A	67	81.71
Hemophilia B	15	18.29
Severity type		
Severe	61	74.39
Moderate	19	23.17
Mild	2	2.44
Treatment type		
On demand therapy only for severe bleeding	3	3.66
On demand therapy	23	28.05
Temporary prophylactic+On demand therapy	24	29.27
Intermittent prophylactic	4	4.88
Tertiary prophylactic	8	9.76
Secondary prophylactic	8	9.76
Primary prophylactic	12	14.63
Inhibitor status		
Negative	46	56.10
Low level (<5 BU)	7	8.54
High level (>5 BU)	4	4.88
Other	25	30.49

RMB, renminbi, Chinese monetary unit; BU, Bethesda Unit. # the exchange rate of RMB to US dollar is 7.1.

annual family income was another significant influencing factor on the quality of life of these adolescents. The third significant influencing factor on the quality of life of these adolescents was the disease type. The average score of 67 cases of hemophilia A was 51.59 ± 15.55 points, whereas that of 15 cases of hemophilia B was 40.12 ± 12.36. This difference was statistically significant ($p = 0.009$). See Tables 2,3 for details.

Discussion

Residence was a Significant Influencing Factor on the Quality of Life of Adolescents with Hemophilia

The Chinese Guidelines for the Treatment of hemophilia (2020 edition) pointed out that acute bleeding should be treated at a nearby professional medical institution as soon as possible or accept self-injection of the missing coagulation factors at home (Thrombosis and hemostasis Group et al, 2020). Early treatment can reduce the bleeding amount and associated pain, dysfunction, and long-term disability, and significantly reduce hospitalizations for complications (Thrombosis and hemostasis Group et al, 2020). To avoid the recurrent joint bleeding and prevent the long-term complications including pain, arthropathy and disability, the World Hemophilia Federation and the World Health Organization recommends preventive treatment as the first choice for children with severe hemophilia, so as to achieve final goal of musculoskeletal function reservation (Feldman et al, 2018; Srivastava et al, 2020).

However, lack of access to sufficient missing coagulation factors and convenient injection services is a common problem faced by adolescents with hemophilia in rural areas (Qin et al, 2012, 2022). The lack of nearby medical institutes in rural areas results in an inability to obtain sufficient coagulation factors in time (Qin et al, 2012, 2022). Moreover, the lack of hemophilia-related knowledge means that the professionals working in rural medical institutes are not able to provide hemostatic and prophylactic treatment for hemophilia patients such as calculation of the dosage and injection of the missing coagulation factors (Qin et al, 2012, 2022). The poor self-management knowledge and nursing skills of these adolescents limits the family treatment and family nursing behaviors. Inconvenient public transportation also limits the ability of adolescents with hemophilia living in rural areas to get preventive treatment or timely on-demand hemostatic treatment. These passive factors affect the quality of life of adolescents with hemophilia living in rural areas.

Thus, the local Hemophilia Treatment Center and policy-making agencies should pay attention to the accessibility to the coagulation factors and the injection services in rural areas.

Medical networks and patient support groups should be made available to facilitate injection of coagulation factors for the adolescents with hemophilia living in rural areas. Continued education regarding hemophilia for primary medical professionals should be provided by the Hemophilia Treatment Center to improve their attitude and ability to support these adolescents. Also, these adolescents and their

Table 2. Between-group comparison of CHO-KLAT questionnaire scores (n = 82).

	Case number	Average total score	Statistical value	*p* value
Age			0.511	0.602
6–10 years	22	52.26 ± 15.26		
11–14 years	33	47.95 ± 14.07		
>14 years	27	49.12 ± 17.80		
Residence			2.628	0.010
City	39	54.08 ± 15.00		
Rural areas	43	45.33 ± 15.11		
Annual family income (RMB)			5.061	0.003
<50,000	49	45.02 ± 15.58		
50,000–100,000	23	53.02 ± 11.54		
100,000–200,000	8	62.22 ± 16.48		
>200,000	2	67.50 ± 2.53		
Primary caregiver			0.955	0.389
Father	8	52.14 ± 11.18		
Mother	70	48.65 ± 16.09		
Other	4	58.97 ± 12.91		
Annual treatment cost (RMB)			1.656	0.183
<10,000	2	52.86 ± 26.26		
10,000–500,000	47	47.31 ± 14.89		
50,000–100,000	22	55.55 ± 15.74		
>100,000	11	46.10 ± 15.61		
Hemophilia type			2.670	0.009
Hemophilia A	67	51.59 ± 15.55		
Hemophilia B	15	40.12 ± 12.36		
Severity type			1.462	0.238
Severe	61	50.81 ± 16.19		
Moderate	19	44.46 ± 13.49		
Mild	2	57.14 ± 2.02		
Treatment type			2.071	0.067
On demand therapy only for severe bleeding	3	32.47 ± 5.28		
On demand therapy	23	50.28 ± 16.89		
Temporary prophylactic+on demand therapy	24	46.36 ± 13.82		
Intermittent prophylactic	4	43.57 ± 13.26		
Tertiary prophylactic	8	52.47 ± 16.57		
Secondary prophylactic	8	46.72 ± 12.22		
Primary prophylactic	12	60.33 ± 15.63		
Inhibitor status			0.398	0.755
Negative	46	49.85 ± 15.48		
Low level (<5 BU)	7	54.28 ± 19.66		
High level (>5 BU)	4	44.68 ± 22.27		
Other	25	48.26 ± 14.17		

CHO-KLAT, the Chinese version of Canadian hemophilia outcomes-kid’s life assessment tool; RMB, renminbi, Chinese monetary unit; BU, Bethesda Unit.

Table 3. Post hoc multiple comparison.

Multiple comparison	Mean value difference	*p* value
Age		
6–10 years VS 11–14 years	4.318	0.320
6–10 years VS >14 years	3.143	0.488
11–14 years VS >14 years	–1.174	0.774
Annual family income		
<50,000 VS 50,000–100,000	–8.004	0.032
<50,000 VS 100,000–200,000	–17.196	0.003
<50,000 VS >200,000	–22.479	0.035
50,000–100,000 VS 100,000–200,000	–9.192	0.128
500,00–100,000 VS >200,000	–14.476	0.181
100,000–200,000 VS >200,000	–5.284	0.647
Primary caregiver		
Mother VS Father	–3.488	0.551
Mother VS Other	–10.325	0.202
Father VS Other	–6.838	0.476
Annual treatment cost		
<10,000 VS 10,000–500,000	5.554	0.619
<10,000 VS 50,000–100,000	–2.687	0.814
<10,000 VS >100,000	6.757	0.570
10,000–500,000 VS 50,000–100,000	–8.240	0.042
10,000–500,000 VS >100,000	1.204	0.816
50,000–100,000 VS >100,000	9.444	0.101
Severity type		
Severe VS Moderate	6.346	0.123
Severe VS Mild	–6.333	0.571
Moderate VS Mild	–12.679	0.275
Treatment type		
On demand therapy VS Primary prophylactic	–10.055	0.064
On demand therapy VS Secondary prophylactic	3.556	0.566
On demand therapy VS Tertiary prophylactic	–2.197	0.722
On demand therapy VS Intermittent prophylactic	6.706	0.412
On demand therapy VS On demand therapy only for severe bleeding	17.806	0.057
On demand therapy VS Temporary prophylactic+On demand therapy	3.911	0.375
Primary prophylactic VS Secondary prophylactic	13.610	0.051
Primary prophylactic VS Tertiary prophylactic	7.858	0.255
Primary prophylactic VS Intermittent prophylactic	16.760	0.057
Primary prophylactic VS On demand therapy only for severe bleeding	27.860	0.005
Primary prophylactic VS Temporary prophylactic+On demand therapy	13.967	0.010
Secondary prophylactic VS Tertiary prophylactic	–5.753	0.446

Table 3. Continued.

Multiple comparison	Mean value difference	p value
Secondary prophylactic VS Intermittent prophylactic	3.150	0.733
Secondary prophylactic VS On demand therapy only for severe bleeding	14.250	0.165
Secondary prophylactic VS Temporary prophylactic+On demand therapy	0.356	0.954
Tertiary prophylactic VS Intermittent prophylactic	8.903	0.336
Tertiary prophylactic VS On demand therapy only for severe bleeding	20.003	0.053
Tertiary prophylactic VS Temporary prophylactic+On demand therapy	6.108	0.322
Intermittent prophylactic VS On demand therapy only for severe bleeding	11.100	0.336
Intermittent prophylactic VS Temporary prophylactic+On demand therapy	–2.794	0.731
On demand therapy only for severe bleeding VS Temporary prophylactic+On demand therapy	–13.894	0.135
Inhibitor status		
Negative VS Low level (<5 BU)	–4.425	0.491
Negative VS High level (>5 BU)	5.174	0.531
Negative VS Other	1.594	0.685
Low level (<5 BU) VS High level (>5 BU)	9.599	0.335
Low level (<5 BU) VS Other	6.019	0.375
High level (>5 BU) VS Other	–3.580	0.675

caregiving parents should be given health education about hemophilia and nursing skills so to implement self-management, including self-injection.

The Annual Family Income was Another Significant Influencing Factor on the Quality of Life of Adolescents with Hemophilia

Preventive treatment is recognized as the best treatment for hemophilia patients and is divided into the standard-dose, medium-dose and low-dose (Li et al, 2017; Thrombosis and hemostasis Group et al, 2020). Standard-dose preventive treatment specifies that children with hemophilia inject the missing coagulation factor at the dose of 25~40 IU/kg each time, three times a week (Feldman et al, 2018; Qin et al, 2022). The “China Guidelines for the Treatment of Hemophilia (2020 edition)” recommend that the medium-dose program be implemented in children with hemophilia starting at a dose of 15 to 30 IU/kg each time, 3 times a week, for children with Hemophilia A, and 2 times a week for children with Hemophilia B. The low-dose program, taking into account China’s national medical and economic conditions, requires children with Hemophilia A to be injected with 10 IU/kg each time, twice a week, or once every 3 days, and children with Hemophilia B to be injected 20 IU/kg each time, once a week (Qin et al, 2022; Thrombosis and hemostasis Group et al, 2020).

However, the annual treatment costs accounts for a large portion of the annual family income for most adolescents with hemophilia (Guo et al, 2019; Li et al, 2017). It is almost impossible for those adolescents with hemophilia whose family annual income is less than 50,000 RMB to afford even the low-dose preventive treatment (Qin et al, 2012). Without preventive treatment, the adolescents experience limitation of their normal activities and recurrent bleeding.

The Third Significant Influencing Factor on the Quality of Life of these Adolescents was the Disease Type

Hemophilia is a rare disease worldwide and is divided into two types: Hemophilia A (Lack of coagulation factor VIII) and Hemophilia B (Lack of coagulation factor IX) (Makris, 2023; National Health Commission Website, 2019). Hemophilia A accounts for 80~85% of all cases of hemophilia, whereas hemophilia B accounts for 15~20% of all cases (Makris, 2023; National Health Commission Website, 2019). Compared with their hemophilia A counterparts, adolescents with hemophilia B have more difficulties in obtaining peer education support. Moreover, coagulation factor IX is difficult to store and has high requirements for transfusion. Thus, the adolescents with Hemophilia B seldom implement family treatment or self-injection of coagulation factors (Qin et al, 2012; Qin et al, 2022).

The Mental Health of Adolescents with Hemophilia should be Prioritized

Responses to open-ended questions in the “General Situation Questionnaire of Hemophiliac Adolescents” revealed the true thoughts of adolescents with hemophilia. Some of them were bothered by the limited social activities and alienation from neighbors. Some felt the economic burden they brought to their families. Some of them even committed suicide. We should recognize their desire for social support and professional psychological assistance. The Hemophilia Treatment Center should routinely do psychological assessment for all the patients in their follow-up clinic to provide professional help.

A limitation of the study is that it was conducted in only one Hemophilia Treatment Center and thus is representative of only one Province (Hubei). Future study should include additional Hemophilia Treatment Centers to broaden the study range so as to obtain more representative data and to enrich the findings of the survey.

Conclusion

This study provides empirical support for the health management of adolescents with hemophilia, highlighting the importance of improving medical resource access, transfusion convenience, and psychological support in enhancing the quality of life for this group. Both training programmes aim to improve the ability of medical professionals to care for hemophiliac patients, and patients’ health education programmes aim to improve their confidence and capacity for self-management should be designed and implemented in rural areas. The results of this study emphasize the need for healthcare systems and policymakers to take specific measures to address those influencing factors to improve the treatment and care conditions for adolescents with hemophilia.

Key Points

- This study revealed that residence, annual family income, and disease type were three significant influencing factors on the quality of life of adolescents with hemophilia in Hubei Province.
- The adolescents with hemophilia showed desire for social support and professional psychological assistance.
- The results were improtant for healthcare systems and policymakers to take specific measures to address those influencing factors to improve the treatment and care conditions for adolescents with hemophilia.

Availability of Data and Materials

Available data are presented in the manuscript. The Chinese Version of CHO-KLAT is not available due to copyright restrictions.

Author Contributions

XLQ designed and initiated the study, and participated in its coordination and drafted the manuscript. AZ participated in the coordination of the study and performed the statistical analysis. XZ, TZ and ZLX conducted the survey. GZY participated in the design and oversight of the study and helped draft the manuscript. All authors contributed to editorial changes of important content in the manuscript. All authors read and approved the final manuscript. All authors have participated sufficiently in the work and agreed to be accountable for all aspects of the work.

Ethics Approval and Consent to Participate

The study was conducted in accordance with the Helsinki Declaration. The full protocol was approved by Tongji Hospital Affiliated to Tongji Medical College Ethics Committee (TJ-IRB20220316). Written consent was obtained from participants and their parents or legal guardian.

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Conflict of Interest

The authors and contributors declare no conflict of interest.

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