

Original article

Validity and reliability of self-administered Thai Pediatric Bleeding Assessment Tool (Pediatric-BAT) application to predict the risk of bleeding in pediatric bleeding disorders

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Abstract:

Background: Congenital and acquired bleeding disorders present with various symptoms ranging from mild to severe manifestation. In recent years, attempts have been made to apply bleeding histories in questionnaires to predict the risk of bleeding and describe the symptoms' severity. The Pediatric Bleeding Assessment Tool (BAT) is a pediatric-specific bleeding checklist set that has been accepted worldwide and was translated to multiple languages including Thai. **Objectives:** (a) The study aimed to test the validity and reliability of the self-administered BAT and compare with the professional-administered BAT among Thai children with bleeding disorders, and (b) to descriptively evaluate severity using the self-administered BAT. **Method:** This is a descriptive study of patients receiving a diagnosis of bleeding disorders treated at the King Chulalongkorn Memorial Hospital between 2019 and 2020. The patients were interviewed by a physician using the Thai version of the BAT application followed by a self-administered assessment over the next two weeks. Results were analyzed using mean and test-retest reliability by intra-class correlation (ICC). **Result:** Totally, 41 eligible pediatric and adolescent patients, a median age of 10 years (ranged 2-20) were enrolled [17 with von Willebrand disease (VWD), 8 with severe hemophilia A, 3 with congenital factor VII deficiency and 13 with immune thrombocytopenia (ITP)]. The total mean scores of self- and professional-administered BAT were 8.88 and 8.98 respectively ($p = 0.819$). However, subgroup analysis showed statistically significant differences between the two groups in diseases that have high BAT scores (severe hemophilia A and chronic ITP). The mean duration of the interview was 9.68 and 3.51 minutes, respectively ($p < 0.001$). The score was also shown to depend on the disease and severity descriptively. **Conclusion:** Self-administered BAT application is comparable with interviews by physicians in identifying the bleeding disorders and evaluating severity, but higher score need to be interpreted more carefully. The nondiagnosed mild bleeder may be encouraged to find medical attention, and known bleeders could also assess any severity change by themselves.

Keywords : ● Pediatric Bleeding Assessment Tool (BAT) ● Pediatric Bleeding Questionnaire (PBQ)
● Self-administered ● Bleeding risk

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นิพนธ์ต้นฉบับ

ความถูกต้องและความน่าเชื่อถือของแบบประเมินเลือดออกด้วยตนเองภาษาไทยผ่านโปรแกรมเพื่อพยากรณ์ความเสี่ยงเลือดออกในผู้ป่วยโรคเลือดเด็ก

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¹แผนกโลหิตวิทยาและมะเร็งเด็ก ²หน่วยปฏิบัติการวิจัยเพื่อการบูรณาการและนวัตกรรมทางโลหิตวิทยาและมะเร็งเด็ก ภาควิชากุมารเวชศาสตร์ คณะแพทยศาสตร์ จุฬาลงกรณ์มหาวิทยาลัย

บทคัดย่อ

ความเป็นมา ภาวะเลือดออกผิดปกติแต่กำเนิดและเกิดภายหลังมักมีอาการหลากหลายตั้งแต่เล็กน้อยถึงรุนแรง ที่ผ่านมามีความพยายามที่จะนำอาการเลือดออกมาใช้สร้างแบบสอบถามเพื่อประเมินความเสี่ยงของการตกเลือดและความรุนแรง ชุดประเมินเลือดออก (Bleeding Assessment Tool: BAT) ในเด็กเป็นชุดคำถามเฉพาะสำหรับเด็กที่ได้รับการยอมรับทั่วโลกและได้รับการแปลเป็นหลายภาษารวมทั้งภาษาไทย **วัตถุประสงค์** (1) เพื่อทดสอบความถูกต้องและความน่าเชื่อถือของชุดประเมินเลือดออกที่บันทึกแบบทดสอบด้วยตนเองเปรียบเทียบกับบันทึกโดยแพทย์ในผู้ป่วยเด็กไทยที่มีโรคเลือดออกผิดปกติ (2) ประเมินความรุนแรงโดยใช้ชุดประเมินเลือดออกที่บันทึกแบบทดสอบด้วยตนเอง **กระบวนการ:** เป็นการศึกษาเชิงพรรณนาของผู้ป่วยที่ได้รับการวินิจฉัยว่าเป็นโรคเลือดออกผิดปกติได้รับการรักษา ณ โรงพยาบาลจุฬาลงกรณ์ ระหว่างปี พ.ศ. 2562-2563 ผู้ป่วยจะได้รับการสอบถามตามคำถามในชุดประเมินเลือดออกและบันทึกโดยแพทย์ผ่านโปรแกรมประยุกต์เวอร์ชันภาษาไทย จากนั้นบันทึกแบบทดสอบด้วยตนเองอีกครั้งในสองสัปดาห์ถัดไป วิเคราะห์ผลลัพธ์โดยใช้ค่าเฉลี่ย และ test-retest reliability โดย intra-class correlation (ICC) **ผลลัพธ์** ผู้ป่วยเด็กและวัยรุ่นที่เข้าเกณฑ์ 41 ราย อายุมัธยฐาน 10 ปี (ช่วง 2-20 ปี) ได้รับการลงทะเบียน (17 รายวินิจฉัย von Willebrand disease 8 รายวินิจฉัยโรคฮีโมฟีเลีย A ชนิดรุนแรง 3 รายวินิจฉัยภาวะพร่องปัจจัยการแข็งตัวชนิดที่ 7 แต่กำเนิด และ 13 รายวินิจฉัยโรคเกล็ดเลือดต่ำจากภูมิคุ้มกัน) คะแนนเฉลี่ยของชุดประเมินเลือดออกของผู้ที่บันทึกแบบทดสอบด้วยตนเองและบันทึกโดยแพทย์เท่ากับ 8.88 และ 8.98 ตามลำดับ ($p = 0.819$) อย่างไรก็ตามการวิเคราะห์กลุ่มย่อยแสดงให้เห็นความแตกต่างที่มีนัยสำคัญทางสถิติในกลุ่มที่มีคะแนนสูง (โรคฮีโมฟีเลีย A ชนิดรุนแรงและ เกล็ดเลือดต่ำจากภูมิคุ้มกันเรื้อรัง) พบระยะเวลาการบันทึกชุดประเมินเลือดออกเท่ากับ 9.68 และ 3.51 นาทีตามลำดับ ($p < 0.001$) ระดับคะแนนยังขึ้นกับชนิดและความรุนแรงของโรคอีกด้วย **สรุป** การบันทึกชุดประเมินเลือดออกด้วยตนเองได้คะแนนเทียบเท่ากับการบันทึกโดยแพทย์ ในประเด็นเพื่อค้นหาผู้มีโรคเลือดออกผิดปกติและประเมินความรุนแรง แต่ควรแปลผลด้วยความระมัดระวังในกรณีที่มีคะแนนสูง ผู้ที่มีเลือดออกเล็กน้อยและยังไม่ได้รับการวินิจฉัยอาจไปพบแพทย์รวดเร็วขึ้น ผู้ป่วยโรคเลือดออกผิดปกติเดิมสามารถประเมินการเปลี่ยนแปลงความรุนแรงได้ด้วยตนเอง

คำสำคัญ : ● เครื่องมือประเมินภาวะเลือดออกในเด็ก ● ชุดคำถามประเมินภาวะเลือดออกในเด็ก ● การทดสอบด้วยตนเอง ● ความเสี่ยงเลือดออกง่าย

วารสารโลหิตวิทยาและเวชศาสตร์บริการโลหิต. 2567;34:.....

Introduction

Pediatric bleeding disorders are rare and consist of congenital and acquired causes including von Willebrand disease (VWD), congenital factor deficiencies, inherited platelet disorders, immune thrombocytopenia (ITP), and others. The symptoms' presentation varied ranging from mild to severe disease comprising mucocutaneous bleeding as epistaxis, heavy menstrual bleeding, bruising, hemarthrosis, gastrointestinal (GI) bleeding or bleeding just after a traumatic event/surgery. Unlike the severe bleeding episodes that urge patients to seek medical attention, mild bleeding disorders are often missed by their parents and these may disturb daily life activities.

In 2009, Bowman, et al.¹ created the Pediatric Bleeding Questionnaire (PBQ) containing five specific clinical aspects, i.e., umbilical stump bleeding, cephalhematoma, postcircumcision bleeding, post-venipuncture bleeding and macroscopic hematuria, aimed to screen children tending to have VWD. The PBQ exhibits a sensitivity of 83% and a specificity of 79%. The score also correlates well with disease severity. This study was modified from the MCMDM-1 VWD Bleeding Questionnaire² which was mainly based on adult patients with VWD. The adult questions were also assigned scores together with menorrhagia or postpartum hemorrhage events. Concerns have arisen that use of a bleeding questionnaire for mild bleeding disorders may become "saturated" (already high scoring) and thus uncorrelated with severity in other severe diseases.³

In 2016, Mahdi S, et al.⁴ proved the bleeding score (International Society on Thrombosis and Haemostasis/Scientific and Standardization Committee Bleeding Assessment Tool: ISTH-BAT)⁵ among adult and pediatric groups correlated to plasma levels of various diseases of clotting factor deficiencies (factor I, V, VII, VIII, IX, XI). However, any bleeding screening questionnaire was limited to mild bleeding disorders, because there might not be any bleeding events to document. Moreover, the bleeding severity was confused with "frequency" more than "site" of bleeding such as central nervous

system (CNS) or GI hemorrhage. The tool also included a PBQ but originally did not comprise postcircumcision bleeding, umbilical stump bleeding and cephalhematoma as seen in the pediatric group but later included in the "other bleeding problems" domain. An ISTH-BAT score of 5 or more indicates bleeding events risks by 8.1% showing incident rates of 3.7 per 100 person-years (95%CI: 1.8-6.6). The score above 3 also indicates the same incidental rates of bleeding.³

In Thailand, Pakdeeto S, et al.⁶ developed a Thai version (Thai pediatric-BAT) adopted from the ISTH-BAT which was available as a mobile application and certified by a professional English-Thai translator. The purpose was to use it as a screening tool for mild bleeding disorders. The result corresponded well with the original ISTH-BAT in all aspects.

The primary aims of BAT and other bleeding assessment tools are to avoid unwanted laboratory testing, predict the risk of bleeding, describe symptom severity and inform the need for treatment. Therefore, in our study, we performed BAT as a standard tool for pediatric bleeding disorders to bleeder detection and evaluated disease severity. The evaluation process was based on a comparison between professional- and self-administered and the reliability was verified. The self-administered BAT could be performed as an upfront strategy for motivating new bleeders to seek medical attention. The known bleeders can also determine bleeding severity by themselves.

The objectives were (a) to test the validity and reliability of the self-administered BAT and compare it with the professional-administered BAT among Thai children with bleeding disorders and (b) to descriptively evaluate severity using the self-administered BAT.

Materials and Methods

Participants

Forty-one eligible pediatric and adolescent patients (24 males and 17 females) at the median age of 10 years (ranging between 2 and 20) were enrolled at the

King Chulalongkorn Memorial Hospital between 2019 and 2020. The diagnoses included VWD (18 cases), ITP (13 cases), hemophilia A (8 cases) and congenital factor deficiencies (3 cases) shown in Table 1. The exclusion criterion considered any patient treated with any antiplatelet and anticoagulant agents.

Bleeding assessment tools

A questionnaire in the form of Thai pediatric BAT was used as a standard tool by combined PBO/ISTH-BAT. The questionnaire was a reported form of bleeding symptoms categorized in 14 parts including epistaxis, cutaneous bleeding, minor cutaneous wound, oral cavity bleeding, GI bleeding, hematuria, posttooth extraction bleeding, surgical bleeding, menorrhagia, postpartum bleeding, muscle hematoma, hemarthrosis, CNS bleeding and other pediatric-specific bleeding. The pediatric-specific bleeding includes postcircumcision, umbilical stump, scalp hematoma, bleeding after venipuncture and subconjunctival hemorrhage. These answers were assigned to a scoring system by one trained physician with ranges of 0 (no bleeding) to 4 (severe symptoms) in each domain rendering a total of 56 scores. The range 0 to 2 score in childhood was defined as normal.

Study protocol

The descriptive-analytic study was approved by the institute's ethics committee (RA510/63). All eligible

participants voluntarily joined the study, and no participants were excluded during the study period. The children and the parents (for patients aged below 15 years old) were interviewed using the BAT by one trained physician, and the duration was recorded. In the next two weeks but not more than four weeks, parents or patients were asked to complete the BAT application again without any supervision. The duration of the self-administered BAT was collected.

Statistical analysis

Scoring by BAT was compared between professional and self-administration. All of these data were represented in mean±standard deviation (SD) and frequency (%). The difference and correlation between the two methods were measured using a paired t-test. The *p*-value below 0.05 was defined as statistical significance. Test-retest reliability was confirmed with the intra-class correlation coefficient (ICC), and all statistical analyses were evaluated using SPSS software version 21.

Results

Baseline characteristics in this study are shown in Table 1. With the professional-administered BAT, the mean score was 8.98±5.73; this was quite similar to self-administered BAT with 8.88±6.40. The mean difference was 0.10±2.71 (*p* = 0.819, 95%CI: -0.76 to

Table 1 Demographic characteristics

Parameter	Value (n = 41)
Age (year), median (IQR)	10 (5-26)
Sex, n (%)	
- Male	24 (58.5)
- Female	17 (41.5)
Diagnosis, n (%)	
- Acute ITP	8 (19.5)
- Chronic ITP	5 (12.2)
- Congenital factor VII deficiency	3 (7.3)
- Severe hemophilia A	8 (19.5)
- Type 1 VWD	6 (14.6)
- Type 2 VWD	11 (26.8)

Abbreviation: IQR, interquartile range; ITP, immune thrombocytopenia; VWD, von Willebrand disease

0.95). However, subgroup analysis showed a statistically significant difference between professional- and self-administered versions in the high BAT score group. This included chronic ITP (11.60 ± 6.19 vs. 14.80 ± 7.01 ; $p = 0.020$, respectively) and severe hemophilia A (13.88 ± 3.91 vs. 11.38 ± 2.97 ; $p = 0.003$, respectively) except congenital factor VII deficiency (19.33 ± 4.16 vs. 23.33 ± 2.31 ; $p = 0.321$, respectively). The duration to fulfill BAT of both methods for all diseases showed a significant difference (3.51 ± 0.84 vs. 9.68 ± 3.68 min; $p < 0.001$, respectively) (Table 2). Using Bland Altman analysis, we found the limit of agreement (LOA) (SD) between the two methods was between -5.32 and 5.52 and a bias of +0.098 units as represented by the gap between the X axis, corresponding to the zero difference (Figure 1). The self-administered BAT showed a good correlation to the professional-administered BAT. The test-retest reliability was performed and the intra-class correlation (ICC) was

0.90 ($p < 0.001$; 95%CI: 0.82-0.94). The score showed descriptive differences between the diseases and tended to depend on the disease severity.

Discussion

The study proved the self-administered BAT, which was first approved for health care providers only, is reliable as a screening tool in defining the bleeder and assessing the severity of the disease when compared with the professional-administered BAT. The higher score can be demonstrated in a group of severe diseases as the scores of congenital factor VII deficiency were higher than VWD. Although a higher score raises the concern of inaccurate results compared with those of the professionally performed the positive questionnaire still warns medical providers to be more careful in supervision allowing for self-administered to be valid. Nevertheless, a patient with severe bleeding tends to

Table 2 BAT scores and duration to complete the questionnaire between professional- and self-administered BAT

Parameter	Professional-administered BAT	Self-administered BAT	p-value
	Mean scores $\pm$ SD	Mean scores $\pm$ SD	
Disease			
Total	8.98 $\pm$ 5.73	8.88 $\pm$ 6.40	0.819
Acute ITP	3.75 $\pm$ 0.71	3.88 $\pm$ 0.64	0.685
Chronic ITP	11.60 $\pm$ 6.19	14.80 $\pm$ 7.01	0.020
Congenital factor VII deficiency	19.33 $\pm$ 4.16	23.33 $\pm$ 2.31	0.321
Severe hemophilia A	13.88 $\pm$ 3.91	11.38 $\pm$ 2.97	0.003
Type 1 VWD	4.50 $\pm$ 1.64	3.33 $\pm$ 0.82	0.110
Type 2 VWD	7.64 $\pm$ 3.04	7.09 $\pm$ 3.02	0.311
Duration to complete the questionnaire (min)			
Total	3.51 $\pm$ 0.84	9.68 $\pm$ 3.68	< 0.001
Acute ITP	3.25 $\pm$ 0.89	9.25 $\pm$ 2.77	< 0.001
Chronic ITP	3.80 $\pm$ 0.84	10.00 $\pm$ 1.41	< 0.001
Congenital factor VII deficiency	3.67 $\pm$ 0.58	9.00 $\pm$ 7.94	< 0.001
Severe hemophilia A	3.87 $\pm$ 0.99	12.38 $\pm$ 3.46	< 0.001
Type 1 VWD	3.00 $\pm$ 0.63	7.33 $\pm$ 3.93	0.027
Type 2 VWD	3.55 $\pm$ 0.82	9.36 $\pm$ 3.14	< 0.001

Abbreviation: BAT, Bleeding Assessment Tool; ITP, immune thrombocytopenia; SD, standard deviation; VWD, von Willebrand disease

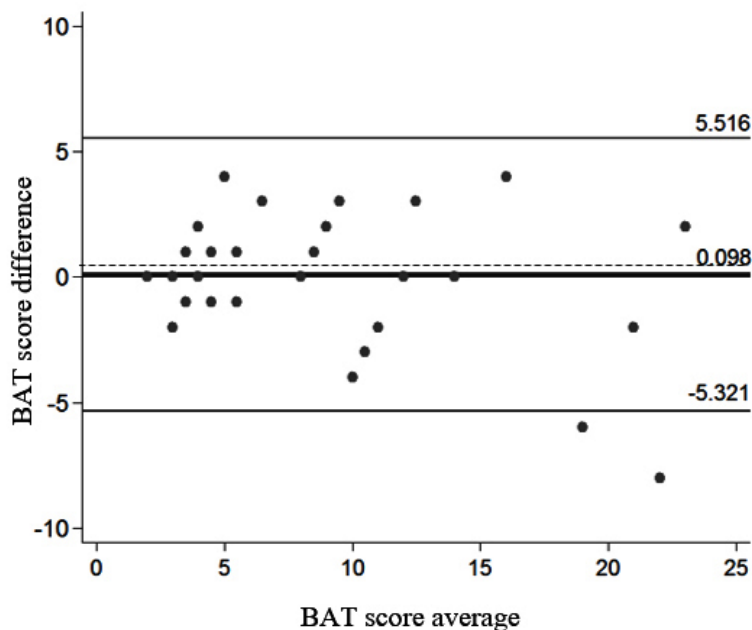


Figure 1 Bland-Altman plot showing the difference between professional- and self-administered-Bleeding Assessment Tool (BAT). Limit of agreement (LOA)(SD) between two methods was between -5.32 and 5.52 and bias of +0.098 units (dot line) as represented by the gap between the X axis, corresponding to the zero difference.

have obvious clinical presentation without BAT requirement. For milder bleeding disorders, the differences were not significant.

The related study compared the electronic self-administered BAT (eBAT) between the physician-administered version in 94 pediatric cases, one half had congenital bleeding disorders. The sensitivity of eBAT was 93.8% (95%CI: 82.8%-98.7%) and positive correlated to the physician-administered BAT.⁷ This is comparable to our study.

The minor differences in scores between the two groups may have stemmed from many factors including recall bias which might play some role in the two evaluations of the same patient. For the professional-administered version, professional respect may hasten the patient to miss some bleeding events when compared with the self-administered version where the patient had more time to recall. The difference in the parents' educational background may also lead to interpreting the same wording in different ways. With the second questionnaire set for the next two weeks, the patients or their parents may also have more time to recall bleeding events thus resulting in higher scoring.

With the upfront strategies for self-administered in a nondiagnosed mild bleeder, especially in VWD and congenital platelet disorders, the widespread use of the questionnaire may encourage parents to bring their children for medical attention sooner. The severity evaluation was also proposed to known bleeders so they could evaluate any severity change by themselves. The period time of evaluation depends on the course of illness.

For completion time, the duration of self-administered BAT was significantly higher compared with the professional-administered version. The reason could be that the questionnaire was not created for the general population. To answer the question without assistance might take much more time than directly clarifying by conversation. However, if the patient frequently self-administered as in every OPD visit, the completion time would be lower. Therefore, in the crowded outpatient clinical setting, the self-administered BAT remains a reasonable follow-up tool for each patient.⁷

Our study's limitation rests on the recall bias because both methods were two weeks apart. The recall bias may not be completely prevented when performing the

questionnaire twice. Because the hypothesis set as the professional-administered BAT has a shorter duration, the self-administered version was performed last to allow recall and prove the hypothesis. The timing was set to reassure the patient may not lose follow-up at the next visit (2-4 weeks apart). Because these are rare bleeding disorders, the sample population comprised a small group. The congenital factor VII deficiency has no significant difference in professional- and self-administered BAT scores compared with other higher score groups such as chronic ITP and severe hemophilia A, possibly due to the low case number. Some disease statuses may have different BAT interpretations, e.g., acquired and congenital causes. For example, epistaxis more than five times a year accounted for one score but for the acute events as acute ITP may differ. For some patients with acute bleeding, emergency treatment could result in rapid recovery and a lower score. In the future, we hope to have easier self-administered tools that can help our patients classify their bleeding risks by themselves at home.

Conclusion

The self-administered bleeding assessment tool (BAT) is verified as a useful method to assist patients in identifying the bleeders and evaluating severity by themselves. However, performing this in a highly severe disease raises the concern of inaccuracy compared with that of professional administration. In the future, an easier tool may be established specifically for self-performing.

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All authors had different duties and accepted the final version of the manuscript. DS suggested a hypothesis and designed the study. Nongnuch Sirachainan offered

the BAT in the original Thai version of the application. SS recruited the patients and drafted the first manuscript. PC revised the final version and supported publication assistance. DS supervised the study.

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