Use of a Pediatric Bleeding Questionnaire in the Screening of Von Willebrand Disease in Young Females at Menarche in the Primary Care Setting

Joana Duran, DrNP, CPNP, CNS, Joseph L. Lasky III, MD, & Cheryl Rodgers, PhD, RN, CPNP, CPON

ABSTRACT

Von Willebrand disease (VWD), the most common inherited bleeding disorder, is caused by deficiency or dysfunction in von Willebrand factor. Assessment of hemorrhagic symptoms is essential for early diagnosis, although bleeding histories are taken in a nonstandardized manner. Validated bleeding assessment tools provide objectivity in evaluating bleeding patterns of females at menarche and may improve provider confidence in screening for VWD. Utilizing a pretest/posttest design, in this project we implemented and evaluated the use of a pediatric bleeding questionnaire in eight pediatric primary care clinics for 3 months. Results indicate improved provider knowledge, confidence, and skills after implementation. The importance and quality of the tool were rated highly by the providers, while the ease of use was rated low. Providers were satisfied with the practice change and believed that it improved their clinical abilities. The use of this tool can improve VWD screening in this practice setting. J Pediatr Health Care. (2016) *30*, 408-413.

KEY WORDS

Von Willebrand disease, hematology, pediatrics, menorrhagia, bleeding assessment

Joana Duran, Student, School of Nursing, Duke University, Durham, NC.

Joseph L. Lasky III, Chief, Division of Pediatric Hematology/Oncology, Harbor UCLA Medical Center, Torrance, CA.

Cheryl Rodgers, Assistant Professor, School of Nursing, Duke University, Durham, NC.

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Correspondence: Joana Duran, DrNP, CPNP, CNS, School of Nursing, Duke University, 307 Trent Dr, Durham, NC 27710; e-mail: joana.duran@duke.edu.

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Von Willebrand disease (VWD) is an inherited bleeding disorder that is caused by a deficiency or dysfunction in von Willebrand factor (VWF), a plasma protein that mediates primary hemostasis by facilitating platelet adhesion and stabilizes blood clotting factor VIII in circulation (Berntorp et al., 2012). VWD, which

is the most common bleeding disorder, is estimated to affect 1% of the general population. Given its primarily autosomal dominant inheritance pattern, VWD is characterized by evidence of a family history of the condition, along with a personal history of excessive mucocutaneous bleeding and abnormal VWF laboratory studies (Nichols et al., 2008). Because of the complex nature of the assessments required to confirm diagnosis, VWD often is not diagnosed in the primary care setting. Accurate assessment of hemorrhagic symptoms is a key component in the early diagnosis of VWD. A bleeding history is often taken in a nonstandardized manner, leaving interpretation to vary according to the experience of the provider (Sadler, 2009). Defined criteria and screening tools for this initial assessment are poorly understood by pediatric primary care providers (Diaz, Laufer, & Breech, 2006). One of the objectives of the U.S. Department of Health and Human Services' Healthy People 2020 framework is to increase the percentage of patients with VWD receiving a timely and accurate diagnosis from the present 28.4% to a minimum of 31.2% (Healthy People 2020, 2014).

Pediatric patients are particularly difficult to assess for this disease, because they may not have been exposed to a large number of hemostatic challenges at their young age. Symptoms of potentially problematic bleeding patterns will become evident with significant hemostatic challenges, such as menorrhagia upon onset of menstruation, so this is an appropriate time for the detection of an underlying bleeding disorder. The American Academy of Pediatrics and the American College of Obstetricians and Gynecologists have jointly stated that pediatric providers should consider the menstrual cycle as another "vital sign" in the assessment of developing females. However, young adolescent females and their parents are often unsure about what represents normal or abnormal menstrual patterns, and clinicians also may be unsure about how to quantify normal ranges for the length, amount, and duration of menstrual flow through adolescence (Diaz, Laufer, & Breech, 2006). Even when abnormal menstrual bleeding is identified, research indicates that providers underestimate inherited bleeding disorders as an underlying cause of menorrhagia in their patients (Chi et al., 2006). Validated assessment tools can assist in accurately evaluating the bleeding patterns of young females at menarche and improve the confidence of primary care providers in screening for VWD.

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The standardized bleeding assessment tool was originally developed by hematologists as a research tool to evaluate bleeding severity in adults with known VWD. Rodeghiero and colleagues (2005) were the first to assess the discriminant power of this tool in the diagnosis of Type 1 VWD (the most mild form) in adult patients. Mild VWD was chosen because it represents 80% to 90% of cases registered at specialized hematology centers, and it can be the most difficult to detect, because bleeding symptoms may vary and are often subtle. This tool was also validated through international, multicenter studies (Goodeve et al., 2007; Tosetto et al., 2006) and prospective clinical trials in previously undiagnosed patients (Tosetto, Castaman, Plug, Rodeghiero, & Eikenboom, 2011). These studies concluded that patients with a diagnosis of VWD and their affected family members have a different phenotype measured by bleeding scores and laboratory values compared with healthy control subjects. Bleeding scores demonstrated an inverse relationship with levels of VWF antigen, VWF ristocetin cofactor, and factor VIII levels. Higher bleeding scores correlated with an increased likelihood of VWD. A normal bleeding score reasonably excluded the presence of mild VWD, with a consistently high negative predictive value (NPV) greater than 95%.

Bowman and colleagues (2009) administered a modified version of the bleeding assessment tool to undiagnosed pediatric patients in the primary care setting with the addition of assessments that are specific to the hemostatic challenges that young children would likely face (i.e., postcircumcision bleeding, cephalohematoma, umbilical stump bleeding, postvenipuncture bleeding, macroscopic hematuria, and conjunctival hemorrhage). This modified bleeding assessment tool became known as the Pediatric Bleeding Questionnaire (PBQ). A score of greater than 2 was abnormal, and an NPV of 0.99 indicated that the tool could accurately rule out VWD 99% of the time. Most recently, Marcus, Nire, Grooms, Klima, and O'Brien (2011) prospectively assessed the PBQ's predictive power in identifying type 1 VWD in pediatric patients. Results showed that the tool was effective in excluding the presence of type 1 VWD. Considering the lack of practicality in administering the full PBQ, Marcus and colleagues also evaluated the diagnostic utility of a simplified yes/no assessment of the bleeding criteria (> 2 bleeding symptoms) contained in the PBQ, which would not take as long as administering the full PBQ. Results showed that this simplified assessment had an NPV comparable with that of the formalized PBQ (93.8% vs. 96.7%, respectively) when ruling out type 1 VWD, while being easier to administer.

PROJECT AIMS AND OBJECTIVES

The purpose of this quality-improvement project was to evaluate a 3-month implementation of the simplified