

Received Date : 26-Sep-2014

Revised Date : 25-Nov-2015

Accepted Date : 11-Mar-2016

Article type : Original Article

**Predictors of Non-Adherence to Prescribed Prophylactic Clotting-Factor Treatment Regimens
among Adolescent and Young Adults with a Bleeding Disorder**

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This is the author manuscript accepted for publication and has undergone full peer review but has not been through the copyediting, typesetting, pagination and proofreading process, which may lead to differences between this version and the [Version of Record](#). Please cite this article as [doi: 10.1111/hae.12951](https://doi.org/10.1111/hae.12951)

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Introduction

Adherence to clotting-factor treatment regimens, especially among adolescents and young adults (AYAs), is under-researched.

Aim

We determined factors associated with better adherence to prophylaxis.

Methods

From April through December 2012, a convenience sample of AYA (aged 13-25 years) persons with haemophilia (PWH) or von Willebrand disease (VWD) completed an online survey that assessed adherence to prescribed prophylactic treatment regimens (VERITAS-Pro). Logistic regression analysis assessed demographic and clinical factors related to non-adherence (VERITAS-Pro \geq 57).

Results

Seventy-three prophylactically-treating AYAs participated. Of which, 88%, 8%, and 4% had haemophilia A, B, and VWD, respectively. Almost all (90%) had severe disease and 58% had never

developed an inhibitor. Most were aged 13-17 years (56%), white (78%), non-Hispanic (88%), never married (94%) and had some type of health insurance (96%).

Median VERITAS-Pro score was 48 (range=25–78) and 22 (30%) participants were non-adherent to prophylaxis (VERITAS-Pro \geq 57). Final logistic regression modeling suggested that, compared to those aged 13-17 years, participants aged 18-25 years were 6.2 (95%CI: 1.8–21.0; $p < .01$) times more likely to be non-adherent. Compared to respondents whose mother had at least a Bachelor's degree, respondents whose mother did not were 3.8 (95%CI: 1.0–14.3; $p = .05$) times more likely to be non-adherent.

Conclusions

Results suggest that adherence efforts should be especially targeted to young adults as they transition from adolescence (i.e., parental supervision) and assume primary responsibility for their bleeding disorder care. Healthcare providers should be mindful of AYAs whose mothers have less formal education and ensure that adequate time and resources are dedicated to family adherence education.

Key Words adherence, prophylaxis/prophylactic, adolescents, young adult, predictors

Introduction

Research over the past 50 years has demonstrated that using prophylactic treatment regimens in children with haemophilia can prevent repeated bleeding into the joints; subsequently reducing hospitalization, chronic pain, and disability—ultimately resulting in improved quality of life (QoL) [1-4]. Prophylaxis in children with severe haemophilia is a *grade 1A* recommendation based on strong evidence from both randomized controlled trials (RCTs) and observational research [5]. There is less consensus, however, about whether prophylaxis is ideal for adolescent and adult persons with haemophilia (PWH) [6-8]. The clinical advantages of continuing prophylaxis into adulthood have to be weighed against more pragmatic considerations like more frequent infusions and greater medical expense [7]. There is, however, a growing body of evidence suggesting that continuing prophylaxis, or starting secondary prophylaxis, in AYA PWH reduces the risk of bleeding and helps decrease chronic pain and preserve joint health and quality of life [3, 8-11].

Despite evidence demonstrating the benefits of prophylaxis, most data suggest that less than 50% of patients actually follow their prescribed treatment regimens in the United States [12-15]. Many factors are thought to influence haemophilia treatment adherence, including knowledge about the disease

and the importance of treatment, disease severity and the frequency of haemophilia symptoms, treatment satisfaction, frequency of administration, the amount of time spent in an HTC, reminder telephone calls, the age of the PWH, and the quality of relationships among patients and their health care professionals [13, 16-20]. Previous studies, however, have not used standardized definitions of adherence, which makes contextualizing and comparing results difficult [12, 13, 17, 21], and adherence among AYA PWH remains particularly under-researched. Exploring adherence among AYA PWH may be especially important given that adolescence and young adulthood represents an inflection point, where PWH start to take more responsibility for the management of their own disease and develop treatment habits that can carry over into adult life [13]. This survey determined factors associated with better adherence to prescribed prophylactic regimens among AYAs with a bleeding disorder using a standardized and validated tool.

Materials and Methods

Study Population and Recruitment

Data describing AYA PWH's adherence to prescribed treatment regimens and level of chronic pain were obtained as part of the larger Interrelationship between Management of Pain, Adherence to Clotting-factor Treatment, and Quality of Life (IMPACT QoL) study, which has been previously described [3]. Data were collected via a one-time, cross-sectional, online survey from a convenience sample of AYA patients with a bleeding disorder. To be eligible to complete the survey, participants had to i) be aged 13-25 years, ii) read, write, and speak English, and iii) have Haemophilia A, Haemophilia B, or VWD. Recruitment occurred at major US haemophilia meetings (e.g., Inhibitor Summits and national and state haemophilia society meetings), US haemophilia treatment centers (HTC), and through a Facebook™ (Facebook, Menlo Park, CA, USA) page dedicated to the study from April through December of 2012. All surveys were completed electronically using SurveyMonkey™ (SurveyMonkey, Palo Alto, CA, USA) and Apple iPads™ (Apple, Cupertino, CA, USA). The study was approved by the Munson Medical Center (Traverse City, MI, USA) institutional review board prior to data collection. All data were de-identified prior to analysis [3].

The current study uses a patient subset of the IMPACT QoL survey data to determine factors associated with better adherence to prophylactic clotting-factor treatment regimens among AYA (aged 13-25 years) PWH or von Willebrand disease (VWD). Patients who treated with on-demand regimens were excluded from this analysis because of small sample size and to minimize heterogeneity in the assessment of the primary hypothesis. Furthermore, unlike the VERITAS-Pro for prophylactic patients,

a validated cut-off of adherence vs non-adherence using the VERITAS-PRN for on-demand patients has not been established [22].

Measurement

Prophylactic adherence was assessed using the Validated Haemophilia Regimen Treatment Adherence Scale (VERITAS)-Pro [23]. Possible subscale scores range from four points (most adherent) to 20 (least adherent), and possible total scores ranged from 24 (most adherent) to 120 (least adherent) [23]. Scores were calculated for the overall VERITAS-Pro and for each of the six VERITAS-Pro subscales (Time, Dose, Plan, Remember, Skip, and Communicate) which are designed to capture the diverse dimensions of adherence [23]. The cutoff for non-adherent prophylactic patients was a total VERITAS-Pro score ≥ 57 as established previously [23]. VERITAS-Pro scores were self-reported. Other self-reported data collected including information about participant age, gender, race, ethnicity, health insurance status/type, and the educational level of the participants' parents. Data were also collected about bleeding disorder type (Haemophilia A or B, or VWD), whether or not the participant ever developed an inhibitor to treatment, and bleeding disorder severity. For Haemophilia A and B, severity was classified as mild, moderate, or severe corresponding to 6%-50%, 1-5%, and <1%, respectively, of the normal amount of clotting factor VIII/IX. VWD was classified as mild, moderate, or severe corresponding to Type I (lower than normal levels of von Willebrand factor), Type II (lower than normal levels and improper functioning of von Willebrand factor), and Type III disease (absence of von Willebrand factor in the blood).

Statistical Analysis

Descriptive statistics and univariate relationships were assessed by tabulating adherence status (adherent vs non-adherent) by patient sociodemographic and clinical characteristics. Percentages were used to describe categorical variables and statistical association was assessed using Fisher's exact test because of small sample size. Multivariable, parsimonious logistic regression models were constructed to predict prophylactic clotting-factor non-adherence (vs adherence). Factors assessed for their relationship with being adherent included: age, gender, race/ethnicity, parent's education level, bleeding disorder type and severity, and history of inhibitor development. Due to the large number of variables collected as part of the survey and because of the small sample size inherent in rare disease research, in addition to the fully adjusted models, final parsimonious models were constructed. In the final parsimonious models, we decided, *a priori*, to include covariates in the model only if they i) were statistically significant at a two-tailed alpha level of .05, ii) changed the odds ratio (OR) of another

statistically significant model parameter by at least 10-15% (i.e., confounded) [24], or iii) improved the precision of another statistically significant parameter already in the model. All statistical analyses were performed using SAS 9.4 (Cary, NC) and Stata 12 (College Station, TX). All p-values were calculated using two-sided tests.

Results

Overall, 108 persons aged 13-25 years with haemophilia or VWD participated in the IMPACT QoL study. Thirty-five (32%) participants who reported treating on-demand (i.e., episodically) were excluded from the analysis. The vast majority (95%) of AYAs who self-reported treating their bleeds prophylactically reported that they 'use factor regularly or on a set schedule for prevention' compared to only 5% who reported that they 'use factor before physical activity for prevention.' Of the 73 prophylactically-treating AYAs remaining, 88%, 8%, and 4% had haemophilia A, B, and VWD, respectively. Almost all (90%) had severe disease and 58% had never developed an inhibitor. Most were aged 13-17 years (56%), white (78%), non-Hispanic (88%), never married (94%) and had some type of health insurance (96%) (Table 1). Although nearly all respondents were insured, insurance type differed significantly by several factors. Adolescents (vs young adults) (61% vs 34%, $p=.03$), whites (vs non-whites) (58% vs 19%, $p=.01$), and respondents whose mothers had at least a bachelor's degree (vs less than bachelor's) (68% vs 32%, $p=.03$) were significantly more likely to have private/commercial insurance only (vs public/government insurance, a combination of private and public insurance, or no health insurance. Among respondents, 34% had a mother who completed at least a bachelor's degree and 36% had a father that did so. The education status of the respondents' parents was closely related ($p<.01$), and data showed that if a respondent's mother had less than a Bachelor's degree, 85% of the time so too did the father. Likewise, if a respondent's mother had at least a Bachelor's degree, 76% of the time the father did as well.

Median VERITAS-Pro score was 48 (range=25–78) and 22 (30%) patients were non-adherent (VERITAS-Pro \geq 57) (Figure 1). At the univariate level, only age was statistically significantly related to non-adherence to prophylactic treatment regimens, with young adults (aged 18-25 years) having a higher percentage of non-adherence compared to adolescents (aged 13-17 years) (47% vs 17%, $p=.01$) (Table 1) and significantly higher (worse) median VERITAS-Pro scores (54.5 vs 45.0, $p=.01$) (Table 2). Final logistic regression modeling (Table 3) suggested that, compared to those aged 13-17 years, participants aged 18-25 years were 6.2 (95%CI: 1.8, 21.0; $p<.01$) times more likely to be non-adherent to prescribed prophylactic treatment regimens. The model also revealed that, compared to respondents whose mother had at least a Bachelor's degree, respondents whose mother did not were

3.8 (95%CI: 1.0, 14.3; $p=.05$) times more likely to be non-adherent to prophylaxis. The final parsimonious model also included disease severity, race, and ethnicity because they increased the overall precision of the model.

Discussion

Previous studies suggest that as PWH age out of childhood and into adolescence and young adulthood, AYAs often begin taking primary responsibility for their bleeding disorder treatment [13, 25]. Subsequently, adherence to prophylaxis regimens frequently worsens [17, 26] as the need for prophylaxis is often deprioritized or perceived as unimportant [13]. Further, as children transition into young adulthood, their activity level often intensifies, which could heighten the risk of bleeding.

Results from our study support this theory, but provide even more granularity for this picture—showing that the period of transition from adolescence into young adulthood is a critical time period regarding adherence to prophylaxis. Specifically, although only 17% of participants aged 13-17 years (adolescents) were non-adherent to their prescribed treatment regimens, that proportion nearly tripled to 47% among those aged 18-25 years (young adults). This difference persisted even after controlling for clinical and sociodemographic variables with logistic regression analysis showing that compared to adolescents, young adults were >6 times more likely to be non-adherent to prescribed prophylactic treatment regimens. Reasons behind this are likely complex. Transitioning from adolescence to young adulthood represents a tectonic shift as choices and challenges evolve to include decisions about education or career training, entering the workforce, leaving the family home, managing finances, and (sometimes) entering into marriage and parenthood. All of these competing demands—compounded by the fact that, during young adulthood, primary responsibility for bleeding disorder care shifts from the parents to the patient—likely explain much of the difference in adherence to recommended prophylactic treatment regimens between adolescent PWH and young adult PWH that we observed. Indeed, previous research suggests that as many as two-thirds of young adults will experiment with stopping or reducing prophylactic dosing [27]. Subsequently, the case for personalized prophylactic treatment plans for young (and older) adults is gaining momentum [28], and this study underscores the fact that among PWH, targeting the transition period from adolescence to young adulthood is important for maintaining adequate haemophilia treatment across the lifecourse. Finally, it may also be that case that transitioning into young adulthood means leaving the health insurance coverage once provided by one's parents and having to obtain health insurance coverage independently. While health insurance status/type did not ultimately predict adherence in our study after, young adults (vs adolescents) (34% vs 61%, $p=.03$) were much less likely to rely exclusively on private/commercial health insurance for

coverage, and were more likely to rely upon public/government health insurance (e.g., Medicaid) or be uninsured. Future studies should continue to evaluate the impact of health insurance status among young adults who are managing a bleeding disorder.

A second finding from this study was that prophylactic adherence was worse for AYAs whose mothers have less formal education. Specifically, compared to respondents whose mother had at least a Bachelor's degree, respondents whose mother did not were nearly 4 times more likely to be non-adherent to prescribed prophylactic treatment regimens. This finding is novel and supports previous research that suggests better knowledge about one's bleeding disorder and the importance of treatment is related to improved adherence [12, 13, 16, 18]. This finding also suggests that health care providers should dedicate adequate time and resources to family adherence education, and that, especially for AYAs, adherence efforts go beyond the patient and extend to family members and other social support members.

Previous work about increasing adherence among AYA PWH has suggested that effective strategies might also include designing individualized prophylactic treatment regimens around "risk periods" (e.g., sporting events and other physical activity or time away from home), focusing on "wellness" instead of "adherence" per se, and recognizing health as a "state of doing" and not a "state of being" thereby integrating the bleeding disorder into everyday life instead of allowing the disease to become a burden [29]. Other research has suggested that it is important for PWH to alter the treatment paradigm from a victimized state where the infusion is something you do *to* yourself, to an empowerment state where the infusion is something you do *for* yourself [30]. These approaches, which stemmed from research in AYA PWH, are similar to earlier research in other (non-haemophilia) AYA chronic disease states that suggest motivation, a sense of normality, experience of results, and parental support and encouragement all lead to increased compliance [31].

This study has limitations. Primarily, data are cross-sectional, thus causal inference cannot be made. For example, although study results support that AYA PWH whose mothers have less formal education tend to have worse adherence, the directionality of this relationship cannot be confirmed. That is, it is also possible that AYAs who maintain adherence to prophylaxis need less medical attention, thereby providing a better climate for their parents to pursue college education. This cannot be teased out in a cross-sectional study and should be examined in the future with prospective research. A second limitation is that all data are self-reported. As such, information about blood disorder type and severity, health insurance coverage, and other demographic, clinical, and behavioral information are not confirmed by medical record review or administrative claims data. However, by obtaining data through self-report, this study was able to collect important, reliable, and valid patient-

report outcomes (PRO) data about adherence. Finally, AYA PWH were primarily recruited from large national or regional haemophilia meetings. Thus, our convenience sample of AYA PWH may not adequately represent the broader AYA PWH population who do not typically attend these meetings. Despite these limitations, this is the first study to our knowledge to analyze predictors of adherence to prescribed prophylactic clotting-factor treatment regimens among AYAs using a standardized and validated patient-reported measure of adherence.

Conclusion

Previous research suggests that among AYAs with a bleeding disorder, better adherence to clotting-factor treatment regimens is associated with less chronic pain and the preservation of joint health and quality of life [3, 8-11]. Results from this study suggest that adherence efforts should be especially targeted to young adults as they transition from adolescence (i.e., parental supervision) and assume primary responsibility for their bleeding disorder care. Finally, healthcare providers should be especially mindful of AYAs whose mothers have less formal education and ensure that adequate time and resources are dedicated to family adherence education.

Acknowledgements

This study was sponsored and funded by Pfizer Inc.

Disclosures

Drs. McLaughlin, Anderson, and Tortella are employees and shareholders of Pfizer Inc. Mr. Munn is a member of Nurse Advisory Board for Baxter, Biogen-Idec, CSL Behring, NovoNordisk and Pfizer. He received a consulting fee from Bayer and was a speaker for Novo Nordisk. Dr. Witkop and Mrs. Lambing stated that they had no interests which might be perceived as posing a conflict or bias at the time the study was conducted.

References

1. Coppola A, Franchini M, Tagliaferri A. Prophylaxis in people with haemophilia. *Thromb Haemost* 2009; **101**: 674-81.
2. Coppola A, Tagliaferri A, Di Capua M, Franchini M. Prophylaxis in children with hemophilia: evidence-based achievements, old and new challenges. *Seminars in thrombosis and hemostasis* 2012; **38**: 79-94.

3. McLaughlin JM, Witkop ML, Lambing A, Anderson TL, Munn J, Tortella B. Better adherence to prescribed treatment regimen is related to less chronic pain among adolescents and young adults with moderate or severe haemophilia. *Haemophilia* 2014.
4. Plug I, van der Bom JG, Peters M, Mauser-Bunschoten EP, de Goede-Bolder A, Heijnen L, *et al.* Thirty years of hemophilia treatment in the Netherlands, 1972-2001. *Blood* 2004; **104**: 3494-500.
5. Iorio A, Marchesini E, Marcucci M, Stobart K, Chan AK. Clotting factor concentrates given to prevent bleeding and bleeding-related complications in people with hemophilia A or B. *The Cochrane database of systematic reviews* 2011: CD003429.
6. Valentino LA. Controversies regarding the prophylactic management of adults with severe haemophilia A. *Haemophilia* 2009; **15 Suppl 2**: 5-18, quiz 9-22.
7. Fischer K. Prophylaxis for adults with haemophilia: one size does not fit all. *Blood Transfus* 2012; **10**: 169-73.
8. Makris M. Prophylaxis in haemophilia should be life-long. *Blood Transfus* 2012; **10**: 165-8.
9. Collins P, Faradji A, Morfini M, Enriquez MM, Schwartz L. Efficacy and safety of secondary prophylactic vs. on-demand sucrose-formulated recombinant factor VIII treatment in adults with severe hemophilia A: results from a 13-month crossover study. *J Thromb Haemost* 2010; **8**: 83-9.
10. Valentino LA. Secondary prophylaxis therapy: what are the benefits, limitations and unknowns? *Haemophilia* 2004; **10**: 147-57.
11. Aledort LM, Dimichele DM. Inhibitors occur more frequently in African-American and Latino haemophiliacs. *Haemophilia* 1998; **4**: 68.
12. Hacker MR, Geraghty S, Manco-Johnson M. Barriers to compliance with prophylaxis therapy in haemophilia. *Haemophilia* 2001; **7**: 392-6.
13. Lindvall K, Colstrup L, Wollter IM, Klemenz G, Loogna K, Gronhaug S, *et al.* Compliance with treatment and understanding of own disease in patients with severe and moderate haemophilia. *Haemophilia* 2006; **12**: 47-51.

14. Ljung R, Auerswald G, Benson G, Jetter A, Jimenez-Yuste V, Lambert T, *et al.* Novel coagulation factor concentrates: issues relating to their clinical implementation and pharmacokinetic assessment for optimal prophylaxis in haemophilia patients. *Haemophilia* 2013; **19**: 481-6.
15. Ragni MV, Fogarty PJ, Josephson NC, Neff AT, Raffini LJ, Kessler CM. Survey of current prophylaxis practices and bleeding characteristics of children with severe haemophilia A in US haemophilia treatment centres. *Haemophilia* 2012; **18**: 63-8.
16. Lindvall K, Colstrup L, Loogna K, Wollter I, Gronhaug S. Knowledge of disease and adherence in adult patients with haemophilia. *Haemophilia* 2010; **16**: 592-6.
17. du Treil S, Rice J, Leissing CA. Quantifying adherence to treatment and its relationship to quality of life in a well-characterized haemophilia population. *Haemophilia* 2007; **13**: 493-501.
18. Remor E. Predictors of treatment difficulties and satisfaction with haemophilia therapy in adult patients. *Haemophilia* 2011; **17**: e901-5.
19. De Moerloose P, Urbancik W, Van Den Berg HM, Richards M. A survey of adherence to haemophilia therapy in six European countries: results and recommendations. *Haemophilia* 2008; **14**: 931-8.
20. Gater A, Thomson TA, Strandberg-Larsen M. Haemophilia B: impact on patients and economic burden of disease. *Thromb Haemost* 2011; **106**: 398-404.
21. Thornburg CD. Physicians' perceptions of adherence to prophylactic clotting factor infusions. *Haemophilia* 2008; **14**: 25-9.
22. Duncan NA, Kronenberger WG, Roberson CP, Shapiro AD. VERITAS-PRN: a new measure of adherence to episodic treatment regimens in haemophilia. *Haemophilia* 2010; **16**: 47-53.
23. Duncan N, Kronenberger W, Roberson C, Shapiro A. VERITAS-Pro: a new measure of adherence to prophylactic regimens in haemophilia. *Haemophilia* 2010; **16**: 247-55.
24. Mickey RM, Greenland S. The impact of confounder selection criteria on effect estimation. *Am J Epidemiol* 1989; **129**: 125-37.

25. Schrijvers LH, Uitslager N, Schuurmans MJ, Fischer K. Barriers and motivators of adherence to prophylactic treatment in haemophilia: a systematic review. *Haemophilia* 2013; **19**: 355-61.
26. Geraghty S, Dunkley T, Harrington C, Lindvall K, Maahs J, Sek J. Practice patterns in haemophilia A therapy -- global progress towards optimal care. *Haemophilia* 2006; **12**: 75-81.
27. Fischer K, Valentino L, Ljung R, Blanchette V. Prophylaxis for severe haemophilia: clinical challenges in the absence as well as in the presence of inhibitors. *Haemophilia* 2008; **14 Suppl 3**: 196-201.
28. Franchini M, Mannucci PM. Prophylaxis for adults with haemophilia: towards a personalised approach? *Blood Transfus* 2012; **10**: 123-4.
29. Khair K, Gibson F, Meerabeau L. The benefits of prophylaxis: views of adolescents with severe haemophilia. *Haemophilia* 2012; **18**: e286-9.
30. Saunders C, Caon C, Smrka J, Shoemaker J. Factors that influence adherence and strategies to maintain adherence to injected therapies for patients with multiple sclerosis. *The Journal of neuroscience nursing : journal of the American Association of Neuroscience Nurses* 2010; **42**: S10-8.
31. Kyngas H. Compliance of adolescents with chronic disease. *Journal of clinical nursing* 2000; **9**: 549-56.

Table 1 Respondent Characteristics by Level of Adherence as Measured by the VERITAS-Pro, 2012 (n=73)^a

| Characteristic | Adherent (n=51) | Non-Adherent (n=22) | Total (n=73) | Fisher's p-value |
|----------------|--------------------|------------------------|-----------------|---------------------|
| Age | | <i>n (%)</i> | | .01 |
| 13-17 | 34 (67) | 7 (32) | 41 (56) | |
| 18-25 | 17 (33) | 15 (68) | 32 (44) | |
| Gender | | | | .30 |
| Male | 51 (100) | 21 (95) | 72 (99) | |

| | | | | |
|---------------------------------------|---------|---------|---------|-----|
| Female | 0 (0) | 1 (5) | 1 (1) | |
| Race | | | | .36 |
| White (only) | 38 (75) | 19 (86) | 57 (78) | |
| Non-White ^b | 13 (25) | 3 (14) | 16 (22) | |
| Ethnicity | | | | .26 |
| Hispanic | 8 (16) | 1 (5) | 9 (12) | |
| Non-Hispanic | 43 (84) | 21 (95) | 64 (88) | |
| Health Insurance^c | | | | .30 |
| Private / Commercial only | 28 (55) | 8 (36) | 36 (49) | |
| Public / Government only ^d | 12 (24) | 6 (27) | 18 (25) | |
| Both Public and Private | 2 (4) | 4 (18) | 6 (8) | |
| Insured – type unknown | 5 (10) | 3 (14) | 8 (11) | |
| Uninsured | 2 (4) | 1 (5) | 3 (4) | |
| Mother's Education | | | | .19 |
| Bachelor's or higher | 20 (39) | 5 (23) | 25 (34) | |
| Less than Bachelor's | 31 (61) | 17 (77) | 48 (66) | |
| Father's Education | | | | .99 |
| Bachelor's or higher | 18 (35) | 8 (36) | 26 (36) | |
| Less than Bachelor's | 33 (65) | 14 (64) | 47 (64) | |
| Bleeding Disorder | | | | .53 |
| Haemophilia A | 46 (90) | 18 (82) | 64 (88) | |
| Haemophilia B | 3 (6) | 3 (14) | 6 (8) | |
| von Willebrand | 2 (4) | 1 (5) | 3 (4) | |
| Severity | | | | .55 |
| Mild | 1 (2) | 0 (0) | 1 (1) | |
| Moderate | 3 (6) | 3 (14) | 6 (9) | |
| Severe | 45 (92) | 18 (86) | 63 (90) | |
| Inhibitor Development | | | | .61 |
| Ever | 23 (45) | 8 (36) | 31 (42) | |
| Never | 28 (55) | 14 (64) | 42 (58) | |

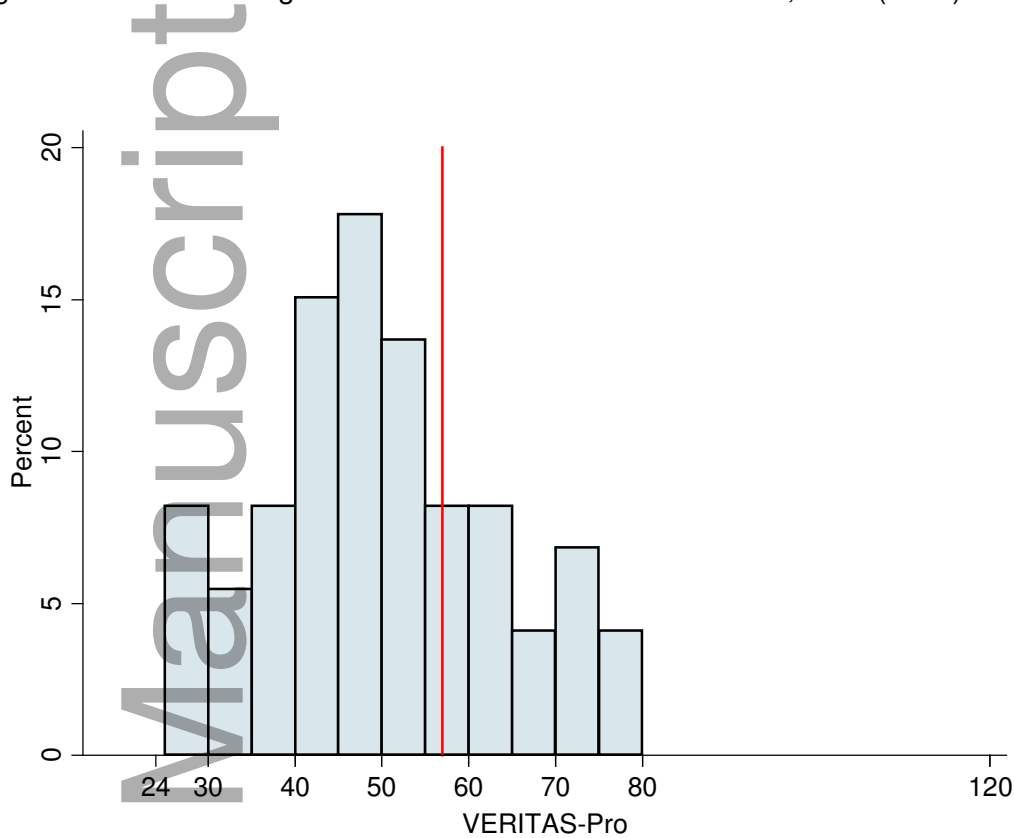
^aAdherence was assessed using the Validated Haemophilia Regimen Treatment Adherence Scale for prophylactic participants (VERITAS-Pro). The cutoff for non-adherent prophylactic patients was a total VERITAS-Pro score ≥ 57 as established in Duncan N, Kronenberger W, Roberson C, Shapiro A. VERITAS-Pro: a new measure of adherence to prophylactic regimens in haemophilia. *Haemophilia*. Mar 2010;16(2):247-255.

^bMost (13/16, 81%) of non-white respondents were black or African American, 2/16 (13%) were Asian, and 1/16 (6%) was mixed race.

^cn=71, two respondents answered 'Don't Know' to whether or not they had health insurance and were not included.

^dOnly two respondents had VA only insurance, the others had Medicaid only.

Figure 1 Percentage Distribution of VERITAS-Pro Scores, 2012 (n=73)



The red line represents the cutoff for non-adherent prophylactic patients (VERITAS-Pro ≥ 57) as established by Duncan et al. [23]. Median VERITAS-Pro score was 48 (range=25–78) and 22 (30%) patients were non-adherent (VERITAS-Pro ≥ 57 ; to the right of the red line).

Table 2 VERITAS-Pro Scores by Subscale and Age Group (n=73)

| VERITAS-Pro Section | Median | IQR | Mean | Range | SD |
|---------------------|--------|-----|------|-------|----|
|---------------------|--------|-----|------|-------|----|

All ages (13-25) (n=73)

| | | | | | |
|------------------------------------|------|-----------|------|-------|------|
| Total scale | 48 | 41–58 | 49.5 | 25–78 | 13.4 |
| Time | 8 | 6–11 | 8.9 | 4–20 | 3.9 |
| Dose | 4 | 4–8 | 6.1 | 4–15 | 2.8 |
| Plan | 6 | 4–9 | 6.8 | 4–16 | 3.0 |
| Remember | 9 | 6–12 | 9.1 | 4–18 | 3.7 |
| Skip | 7 | 5–10 | 7.7 | 4–16 | 3.4 |
| Communicate | 10 | 7–14 | 10.8 | 4–20 | 4.4 |
| <i>Adolescents (13-17) (n=41)</i> | | | | | |
| Total scale | 45 | 38–54 | 45.4 | 25–76 | 12.7 |
| Time | 8 | 5–10 | 8.0 | 4–20 | 4.0 |
| Dose | 4 | 4–7 | 5.7 | 4–12 | 2.4 |
| Plan | 5 | 4–8 | 6.3 | 4–16 | 2.9 |
| Remember | 8 | 5–10 | 8.1 | 4–16 | 3.5 |
| Skip | 7 | 4–9 | 7.0 | 4–16 | 3.1 |
| Communicate | 10 | 7–14 | 10.2 | 4–20 | 4.1 |
| <i>Young Adults (18-25) (n=32)</i> | | | | | |
| Total scale | 54.5 | 44.5–64.5 | 54.6 | 35–78 | 12.7 |
| Time | 10 | 8–12 | 10.1 | 4–20 | 3.5 |
| Dose | 5.5 | 4–9 | 6.8 | 4–15 | 3.2 |
| Plan | 7.5 | 4.5–9 | 7.5 | 4–16 | 3.1 |
| Remember | 11 | 8–12 | 10.3 | 4–18 | 3.5 |
| Skip | 8 | 5.5–11 | 8.5 | 4–16 | 3.6 |
| Communicate | 11.5 | 7.5–16 | 11.5 | 4–19 | 4.7 |

Possible subscale scores ranged from four points (most adherent) to 20 (least adherent), and possible total scores ranged from 24 (most adherent) to 120 (least adherent).

Table 3 Odds Ratios (OR) and 95% Confidence Intervals (CI) of Being Non-Adherent as Measured by the VERITAS-Pro, 2012 (n=73)^{a,b}

| Characteristic | OR (95%CI) | p-value |
|----------------|------------|---------|
|----------------|------------|---------|

| | | |
|---------------------------|-------------------|------|
| Age | | |
| 18-25 | 6.16 (1.81, 21.0) | <.01 |
| 13-17 | 1.00 | |
| Mother's Education | | |
| Less than Bachelor's | 3.77 (1.00, 14.3) | .05 |
| Bachelor's or higher | 1.00 | |

^aAdherence was assessed using the Validated Haemophilia Regimen Treatment Adherence Scale for prophylactic participants (VERITAS-Pro). The cutoff for non-adherent prophylactic patients was a total VERITAS-Pro score ≥ 57 as established in Duncan N, Kronenberger W, Roberson C, Shapiro A. VERITAS-Pro: a new measure of adherence to prophylactic regimens in haemophilia. *Haemophilia*. Mar 2010;16(2):247-255.

^bThe model was also adjusted for disease severity and race/ethnicity.

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