



HAL
open science

Management of von Willebrand disease with a factor VIII-poor von Willebrand factor concentrate: Results from a prospective observational post-marketing study

Nathalie Itzhar-baikian, Annie Borel-derlon, Jenny Goudemand, Françoise Bridey, Segolene Claeysens, Nathalie Itzhar-Baikian, Annie Harroche, Dominique Desprez, Claude Negrier, Pierre Chamouni, et al.

► To cite this version:

Nathalie Itzhar-baikian, Annie Borel-derlon, Jenny Goudemand, Françoise Bridey, Segolene Claeysens, et al.. Management of von Willebrand disease with a factor VIII-poor von Willebrand factor concentrate: Results from a prospective observational post-marketing study. *Journal of Thrombosis and Haemostasis*, 2020, 18 (8), pp.1922-1933. 10.1111/jth.14928 . hal-03143538

HAL Id: hal-03143538

<https://hal.inrae.fr/hal-03143538v1>

Submitted on 16 Feb 2021

HAL is a multi-disciplinary open access archive for the deposit and dissemination of scientific research documents, whether they are published or not. The documents may come from teaching and research institutions in France or abroad, or from public or private research centers.

L'archive ouverte pluridisciplinaire **HAL**, est destinée au dépôt et à la diffusion de documents scientifiques de niveau recherche, publiés ou non, émanant des établissements d'enseignement et de recherche français ou étrangers, des laboratoires publics ou privés.



Distributed under a Creative Commons Attribution 4.0 International License

menu close

```
* info *INFO navigate_next <#info-tab-pane>
* list *OUTLINE navigate_next <#readium-toc-body>
* perm_media *MATERIALS navigate_next <#files-tab-pane>
* image *FIGURES navigate_next <#figures-tab-pane>
* perm_media *SUPPLEMENTS navigate_next <#supplementary-tab-pane>
* *LINKS navigate_next <#links-tab-pane>
* *REFERENCES navigate_next <#references-tab-pane>
* format_quote *CITED BY navigate_next <#citations-tab-pane>
* more_horiz *RECOMMENDED navigate_next <#recommended-tab-pane>
```

navigate_before BACK *FIGURES
Cover Image

ARTICLE

Management of von Willebrand disease with a factor VIII-poor von Willebrand factor concentrate: Results from a prospective observational post-marketing study

Jenny Goudemand, Françoise Bridey, Ségolène Claeysens, Nathalie Itzhar-Baikian, Annie Harroche, Dominique Desprez, Claude Négrier, Pierre Chamouni, Hervé Chambost, Céline Henriet, Sophie Susen, Annie Borel-Derlon

format_quote CITE

<<https://onlinelibrary.wiley.com/action/showCitFormats?doi=10.1111%2Fjth.14928&mobileUi=0>>

© 2020 The Authors. /Journal of Thrombosis and Haemostasis/ published by Wiley Periodicals LLC on behalf of International Society on Thrombosis and Haemostasis

<https://doi.org/10.1111/jth.14928> <<https://doi.org/10.1111/jth.14928>>

Published in Journal of Thrombosis and Haemostasis

ISSN1538-7933

eISSN1538-7836

Received 2 December 2019

Accepted 14 May 2020

Published 4 August 2020

Volume 18

Issue 8

Pages 1922 - 1933

Abstract

Background

A triple-secured plasma-derived von Willebrand factor (pdVWF) almost devoid of factor VIII (FVIII):WILFACTIN[®], was approved in France in 2003, and then in other countries for the treatment of patients with von Willebrand disease (VWD).

Objective

To investigate long-term safety and efficacy of the product in real-life over the first 5 post-approval years.

Patients/Methods

This prospective, observational, national post-marketing study (PMS) enrolled patients of all ages and VWD types. Patients were observed for up to 3 years and treated for one or more occasions. Efficacy was assessed for each major event. Breakthrough bleeding rate 3 days post-infusion and annualized bleeding rate (ABR) were also evaluated for long-term prophylaxis.

Results

Overall, 155 of 174 patients enrolled from 31 centers were eligible for efficacy assessment. Most patients (76.8%) were severely affected (VWF:RCo \leq 15 IU/dL). They were treated for 743 bleeds and 140 surgeries including childbirth. Efficacy outcomes were excellent/good for 98.2% of 56 major surgeries and 94.0% of 67 major bleeds. Approximately 75% of 49 major mucosal bleeds were effectively managed without FVIII co-administration. In 32 patients receiving prophylaxis, breakthrough bleeding occurred in 1.5% of infusions and median ABR was 1.0 for 20 patients treated \geq 12 months. Excellent tolerability was confirmed with no safety concerns. No thrombotic events were observed.

Conclusions

Results from this PMS increase the clinical experience of a FVIII-poor pdVWF in patients of all ages and VWD types including those with thrombotic risk factors and emphasize that giving FVIII is not always mandatory to effectively treat patients with severe VWD.

sentiment_very_dissatisfied

*This publication does not have an outline.

* *FIGURES navigate_next <#figures-tab-pane>

* *SUPPLEMENTS navigate_next <#supplementary-tab-pane>

navigate_before BACK *FIGURES

remove_from_queue

*Annotations are not currently enabled.

* *REFERENCES navigate_next <#references-tab-pane>

* *CITED BY navigate_next <#citations-tab-pane>

* *RECOMMENDED navigate_next <#recommended-tab-pane>

navigate_before BACK *FIGURES

open_in_new Visit Publisher's site

<<https://onlinelibrary.wiley.com/doi/10.1111/jth.14928>>

Page1/12

* skip_next Next

* skip_previous Previous

* Go to start

* Go to the end

*

GO TO PAGE

*

info_outlined

Nodes are locations in the document that facilitate reading from beginning to end. You can navigate node by node or select one to jump to.

format_size

Alignment
format_align_left

format_align_justify

Font Size
remove

add

Hyphenation

/close

/ Return to default styles

Reading Flow
swap_vert

swap_horiz

Columns Pages
view_headline

vertical_split chrome_reader_mode

fullscreen

fullscreen_exit
remove_circle_outline

add_circle_outline

search

*SEARCH search

close
group_add

Copy Access
more_horiz

* print*Print document
* *Offline reading
*
stop offline_pin
*Save for offline reading

*Saving for offline reading
*Saved for offline reading *Remove
*Removing from offline documents
* library_books *View Offline documents
<https://onlinelibrary.wiley.com/reader/library>
* Login

<https://onlinelibrary.wiley.com/action/showLogin?redirectUri=%2Fdoi%2Fepdf%2F10.1111%2Fjth.14928>
/ Register

<https://onlinelibrary.wiley.com/action/registration?redirectUri=%2Fdoi%2Fepdf%2F10.1111%2Fjth.14928>

get_app
<https://onlinelibrary.wiley.com/doi/pdfdirect/10.1111/jth.14928?download=true>

menu

play_circle_filled

play_circle_filled

arrow_back BACK

chapter title here

figure title here

get_app
All Materials (0)

SHOW
All materials 0

Figures 0

Tables 0

Videos 0

Audio files 0

Code snippets 0

Supplements 0

fullscreen

fullscreen_exit
remove_circle_outline

add_circle_outline

play_circle_filled

play_circle_filled

arrow_back BACK

EXIT

chevron_right chevron_right

figure title here

publication title here

* sort DETAILS navigate_next <#caption-tab-pane>

* get_app DOWNLOAD

navigate_before BACK DETAILS

All Materials

publication title here