

On outcomes for hemophilia

Balen, E.C. van

Citation

Balen, E. C. van. (2022, November 30). *On outcomes for hemophilia*. Retrieved from https://hdl.handle.net/1887/3492202

Version:	Publisher's Version
License:	<u>Licence agreement concerning inclusion of doctoral thesis in the</u> <u>Institutional Repository of the University of Leiden</u>
Downloaded from:	https://hdl.handle.net/1887/3492202

Note: To cite this publication please use the final published version (if applicable).

On outcomes for hemophilia

Erna C. van Balen

On outcomes for hemophilia ISBN: 978-94-6458-552-0

Provided by thesis specialist Ridderprint, ridderprint.nl Printing: Ridderprint Design cover and chapter pages: Okki Poortvliet, www.okkipoortvliet.com Layout and design: Erwin Timmerman, persoonlijkproefschrift.nl

Copyright © 2022. E.C. van Balen, Leiden, the Netherlands All rights reserved. No part of this thesis may be reproduced, stored in a retrieval system, or transmitted in any form or by any means, electronically, mechanically, by photography, by recording, or otherwise, without prior written permission of the author.

On outcomes for hemophilia

Proefschrift

ter verkrijging van de graad van doctor aan de Universiteit Leiden, op gezag van rector magnificus prof.dr.ir. H. Bijl, volgens besluit van het college voor promoties te verdedigen op woensdag 30 november 2022 klokke 13:45 uur

door

Erna C. van Balen – van Leeuwen

geboren te Groningen in 1980

Promotores:

Prof. dr. J.G. van der Bom Prof. dr. F.R. Rosendaal

Co-promotor:

Dr. S.C. Gouw

Leden promotiecommissie:

Prof. dr. F.W. Dekker Prof. dr. H.C.J. Eikenboom Prof. dr. M.C. de Vries Prof. dr. H.F. Lingsma (Erasmus MC) Prof. dr. C.B. Terwee (Amsterdam UMC)

The work described in this thesis was performed at the department of Clinical Epidemiology of Leiden University Medical Center Financial support for printing of this thesis was kindly provided by Chipsoft and CSL Behring

Waar een wil is, is een weg. En waar een Erna is, is een wil.

Voor iedereen met hemofilie

Contents

Chapter 1	General introduction, aims and outline, and study populations	9	
PART I: TREATMENT DECISIONS			
Chapter 2	Patient-centered care in hemophilia: Patient perspectives on visualization and participation in decision-making	27	
Chapter 3	Patient perspectives on novel treatments in hemophilia: a qualitative study	45	
PART II: DEFINING, MEASURING AND QUANTIFYING OUTCOMES			
Chapter 4	Defining patient value in hemophilia care	67	
Chapter 5	Patient-relevant health outcomes for hemophilia care: development of an international standard outcomes set	75	
Chapter 6	Validation of PROMIS Profile-29 in adults with hemophilia in the Netherlands	141	
Chapter 7	Socio-economic participation of persons with hemophilia: results from the sixth Hemophilia in the Netherlands study	171	
PART III: CONCLUSIONS			
Chapter 8:	Summary and general discussion	197	
APPENDICES	Nederlandse samenvatting Factsheet Hemofilie in Nederland Portfolio List of publications	218 226 226 215	
	Dankwoord	231	
	Curriculum Vitae	233	