Shared Doctor-Patient Decision Making

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Institute for Policy Advancement Ltd
12 November 2021

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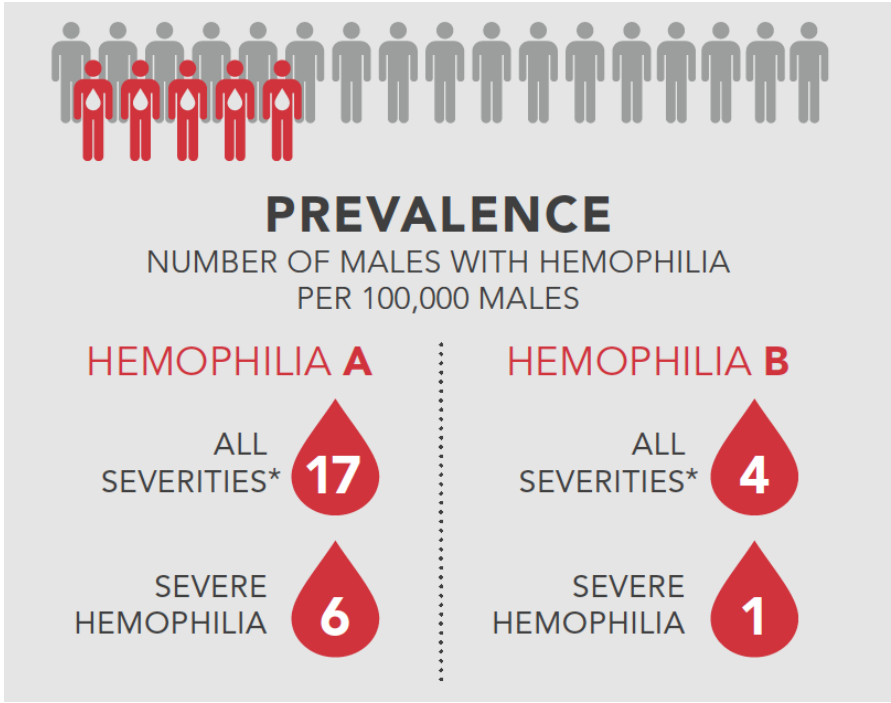
What is Hemophilia?

- Deficiency of clotting factor protein
  - Blood does not clot properly
- X-linked recessive
  - Does not discriminate by race or region of the world
  - Predominantly male; but women have hemophilia too
  - One-third no known family history
- Well characterized genetically and clinically
  - FVIII (Hemophilia A) ~80%
  - FIX (Hemophilia B) ~20%
  - Prevalence / Prevalence at Birth[1]:
    - Per 100,000 Males, 21 will have hemophilia
    - Per 100,000 Male Births, 30 will have hemophilia
    - People with hemophilia still have a life expectancy disadvantage.



[1]Iorio et al. Establishing the male prevalence and prevalence at birth of hemophilia. Ann Intern Med. (2019) doi:10.7326/M19-1208

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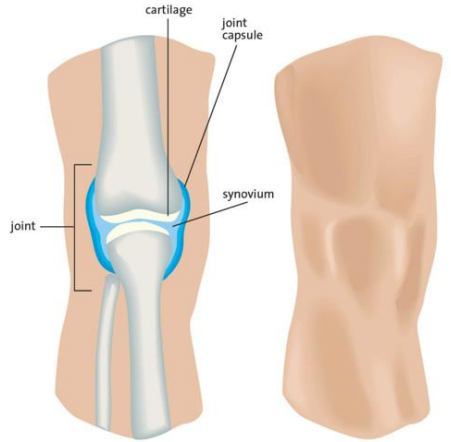
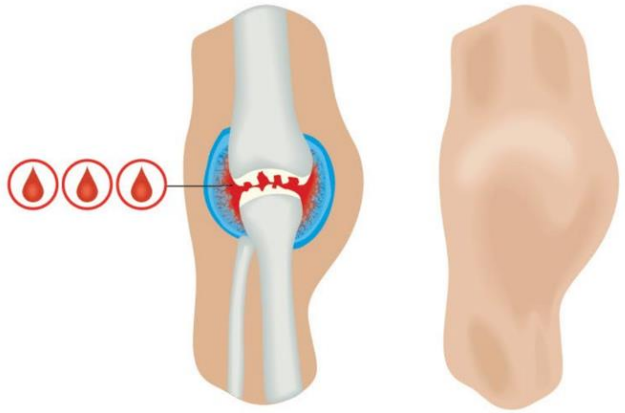
What is Hemophilia?

- Clinical manifestations
  - Internal bleeding into joints, soft tissues
  - Significant morbidity and mortality
  - ~20-30% people with severe hemophilia A develop neutralizing antibodies
- State of the Art Treatment
  - Prophylaxis (factor replacement, substitution therapies)
  - Integrated disease management
- There is no cure, at least yet





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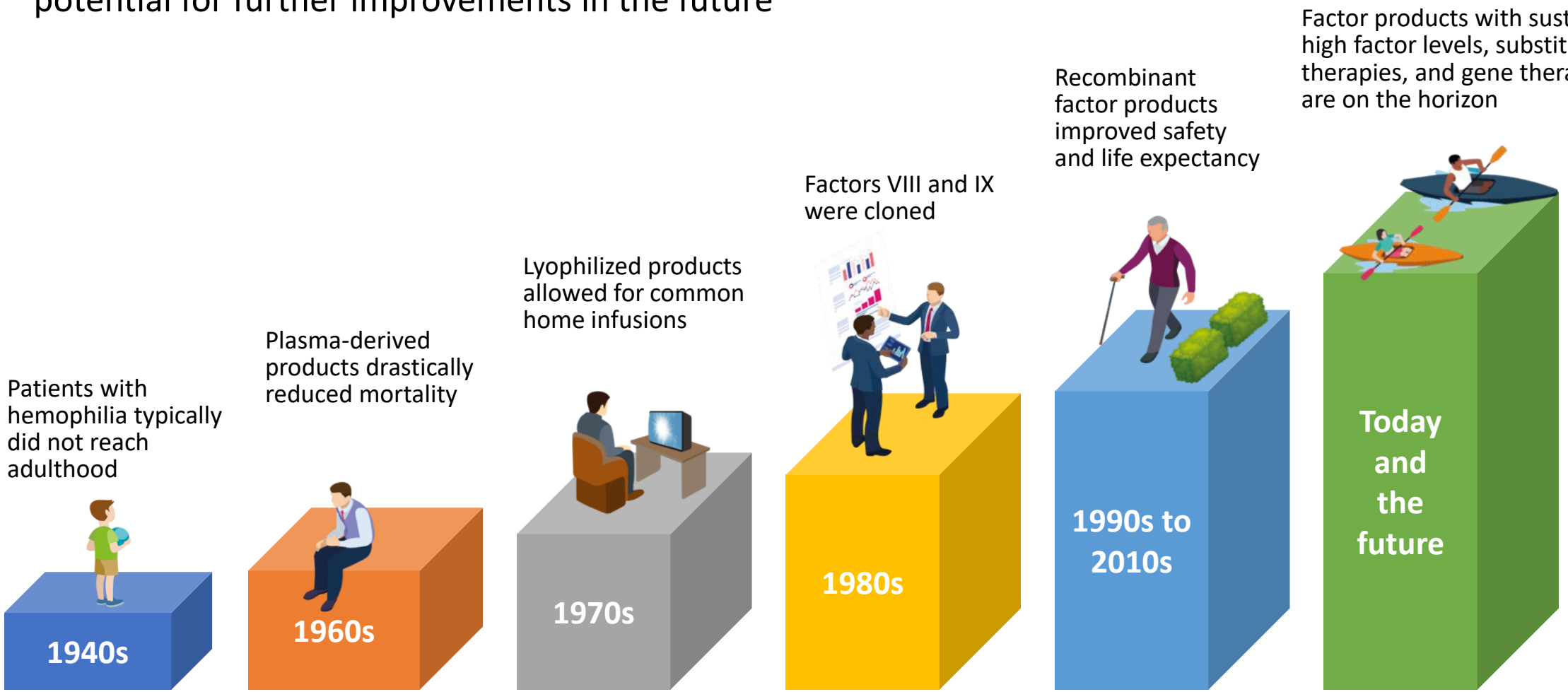
With each treatment advance, treatment goals have evolved…

…from reducing early death to decreasing spontaneous bleeding and associated morbidity, with the potential for further improvements in the future



Lusher JM. In: Kaushansky K, Berliner , eds. 50 Years in Hematology: Research That Revolutionized Patient Care. Washington, DC: ASH; 2008:25-27; Skinner MW, et al. Haemophilia. 2020;26(1):17-24.

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Today, a Wide Range of Treatment Options

	No inhibitor			Inhibitor		
	On demand	Prophylaxis	Curative	On demand	Prophylaxis	Immune tolerance induction
Standard half-life (SHL) recombinant clotting factor	x	x				x
SHL, plasma derived (PD)	x	x				x
Extended half-life recombinant clotting factor	x	x				x
Recombinant bypassing clotting factor				x	x	
PD-bypassing clotting factor				x	x	
Nonfactor replacement		x*			x**	
Gene therapy			x*			

*Available only *via* clinical trial for patients with hemophilia A or B without inhibitor. **Commercially available or available *via* clinical trial for patients with hemophilia A and inhibitor; available only *via* clinical trial for patients with hemophilia A or B and inhibitor.

Nossair F et al., Therapeutic Advances in Hematology. August 2018:239-249. doi:10.1177/2040620718784830

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Dimensions of Patient-Centered Health Care

Safe	• Avoid harm to patients
Effective	• Provide services based on sound scientific knowledge to patients who could benefit from such services and refrain from providing services to patients that may not benefit them
Patient-centered	• Care that is respectful to the patient's values, needs, concerns
Timely	• Reduce delays in patient care that may be harmful to the patient's overall well-being
Efficient	• Avoid waste of services and resources
Equitable	• Provide care to all patients that is of equal quality that does not vary based on an individual's race, ethnicity or other personal characteristics

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Patients as Partners

Health reforms around the world are altering the relationship between healthcare providers and the health systems they serve.

- Increasing patient engagement
- Introducing structural reforms to integrate care around the patient
- Increasing the importance of the patient voice in determining value
- Increasing the need for more effective, patient-directed management of chronic diseases

Adapted from PharmaFutures: Pathway to Value, Meteos 2013

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Health Equity

> “Health inequities are avoidable inequalities in health between groups of people within countries and between countries[1]”

Health inequity for people with hemophilia[2,3]

- Treatment access and availability vary
- People with hemophilia can currently expect a range of treatment options that improve life expectancy, but some lives are lived with chronic pain and disability

1. World Health Organization. https://www.who.int/social_determinants/thecommission/finalreport/key_concepts/en/. Accessed October 2, 2020.
2. Skinner MW. Haemophilia. 2012;18(suppl 4):1-12. 3. Skinner MW, et al. Haemophilia. 2020;26(1):17-24

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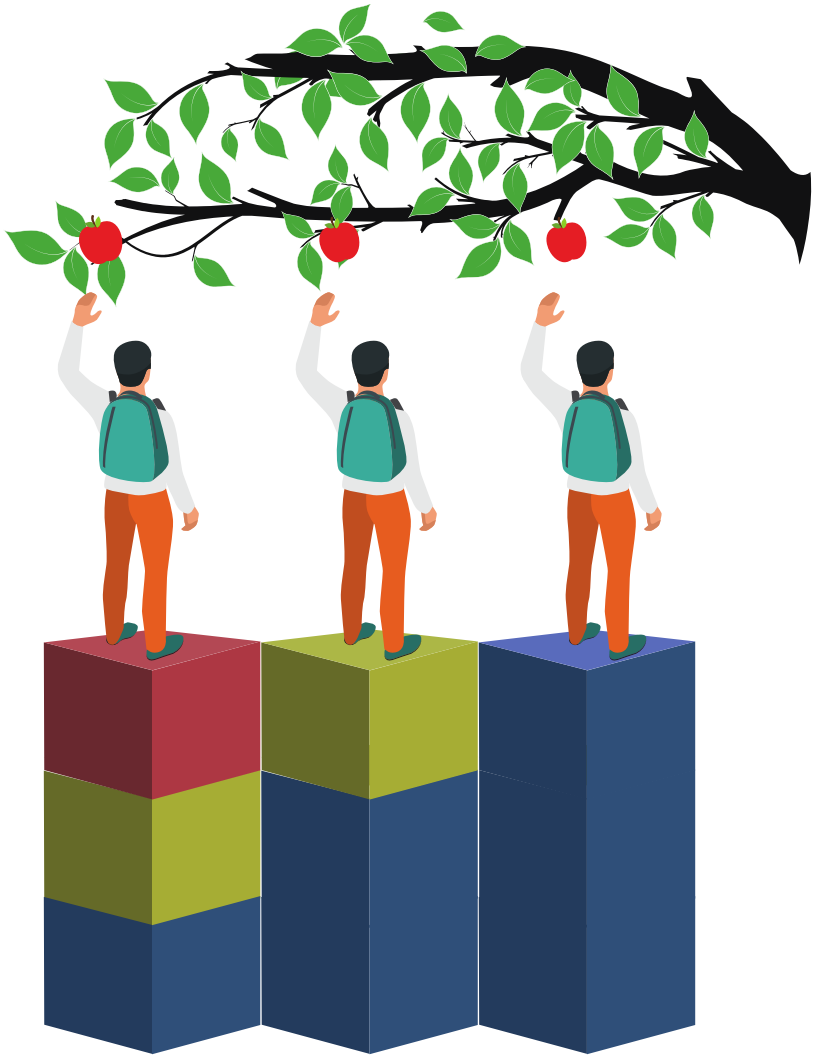
Achieving health equity for people with hemophilia

Advances in hemophilia therapies bring new opportunities

Possibility to attain a lifestyle undeterred by disease complications

Achieving health equity requires aligning aspirations of patients and health care providers



Skinner MW, et al. *Haemophilia*. 2020;26(1):17-24.

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Patients have a unique perspective and will consider issues differently than regulators, manufacturers, scientists, clinicians and payers.[1]

[1]FDA Patient Focused Drug Development Initiative

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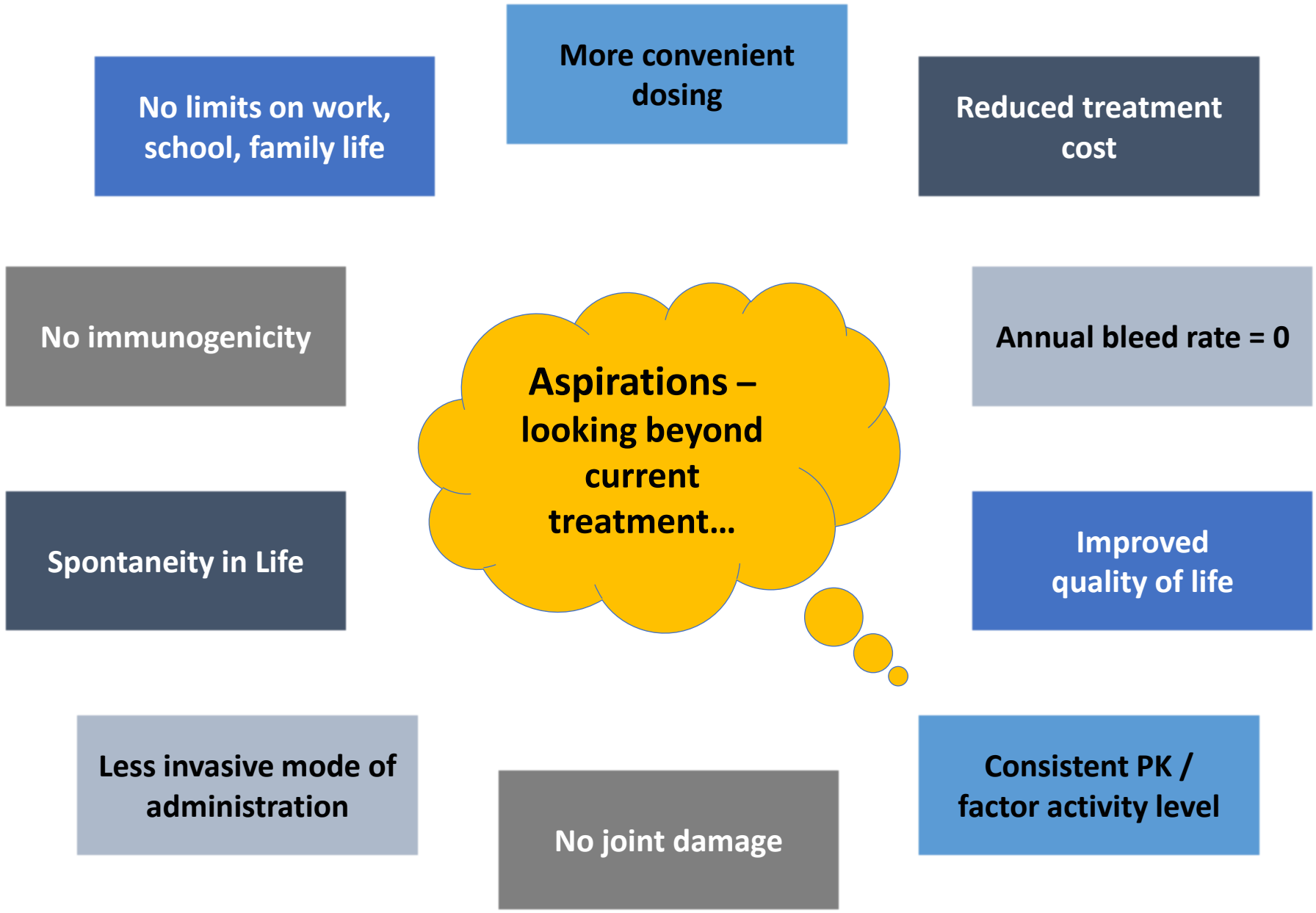

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Treatment and Life Goals Will Be Different

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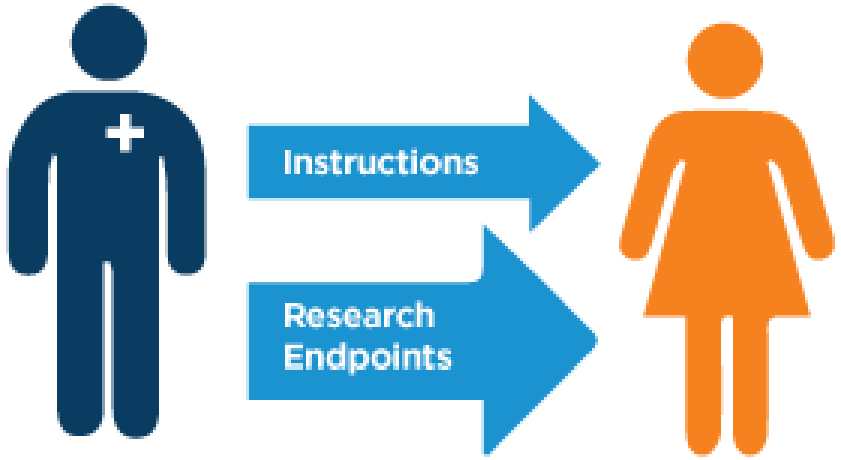
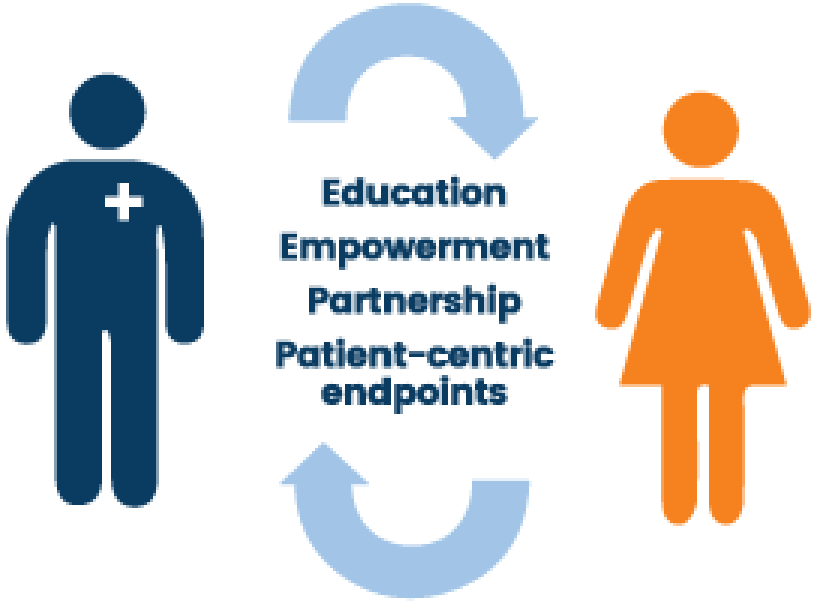
Traditional uni-directional research-centered view



New bi-directional patient-centered view



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What is Shared Decision Making?

- It is more than informed consent
- It is a process wherein:
  - a health care provider shares with a patient all of the relevant information and best scientific evidence available on the pros and cons of all potential treatment options
  - a patient shares with the provider all of their relevant values, preferences and goals
  - with this mutual understanding the patient and provider decide the best course of action

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Key Benefits of Shared Decision Making

- Advances medicine beyond the traditional “one-size fits all” regimens typical of the customary, paternalistic clinical model
- Moves health care decision making from a transactional interaction to a true and equitable relationship between the patient and health care team
- Promotes a more symmetrical and equitable partnership between the health care provider and patient

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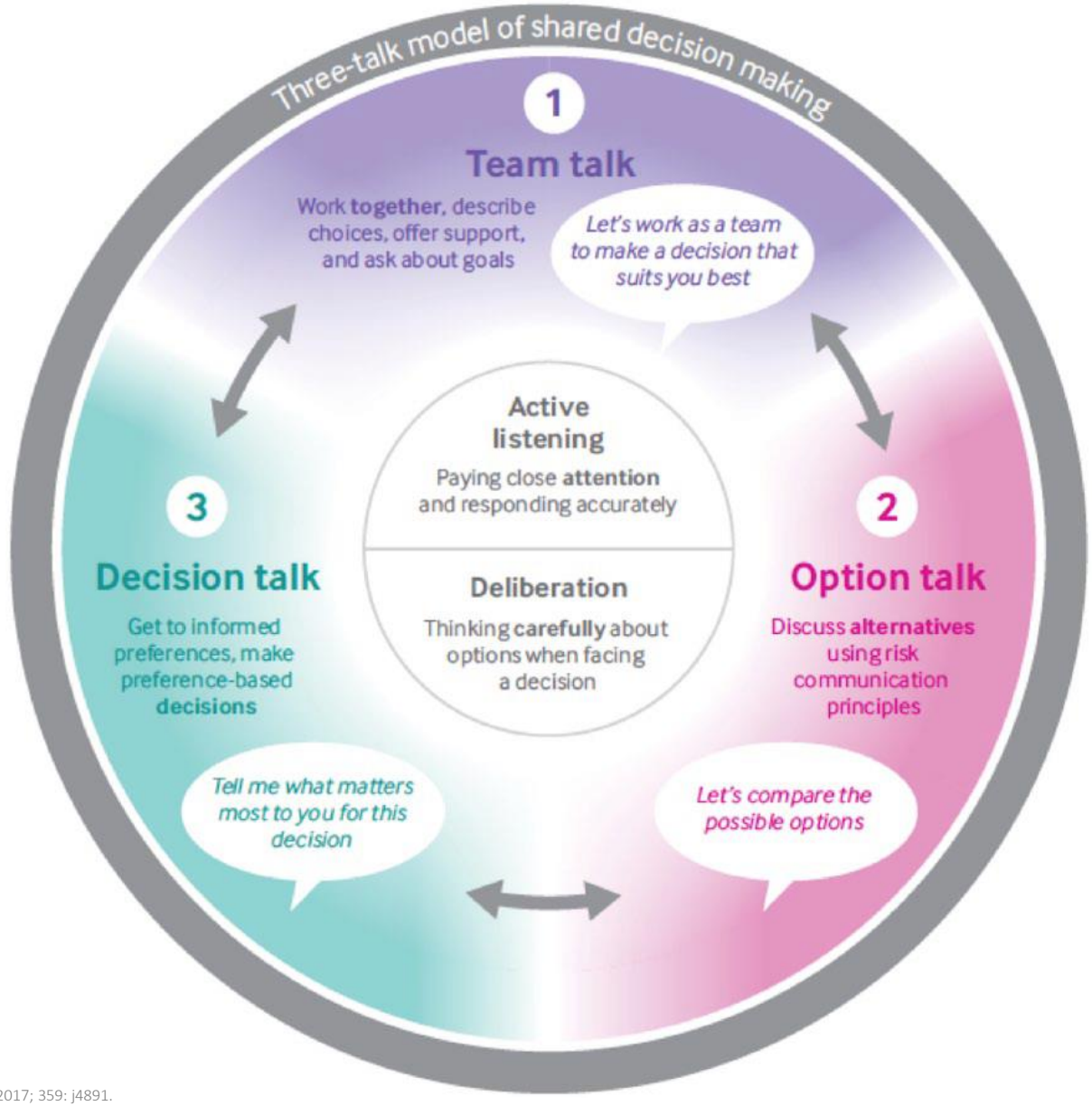
Three Talk Model of Shared Decision Making

The overall goal of this model is making a decision based on informed preferences.



Elwyn G, Durand MA, Song J, et al. A three-talk model for shared decision making: multistage consultation process. BMJ (Clinical Research Ed) 2017; 359: j4891.

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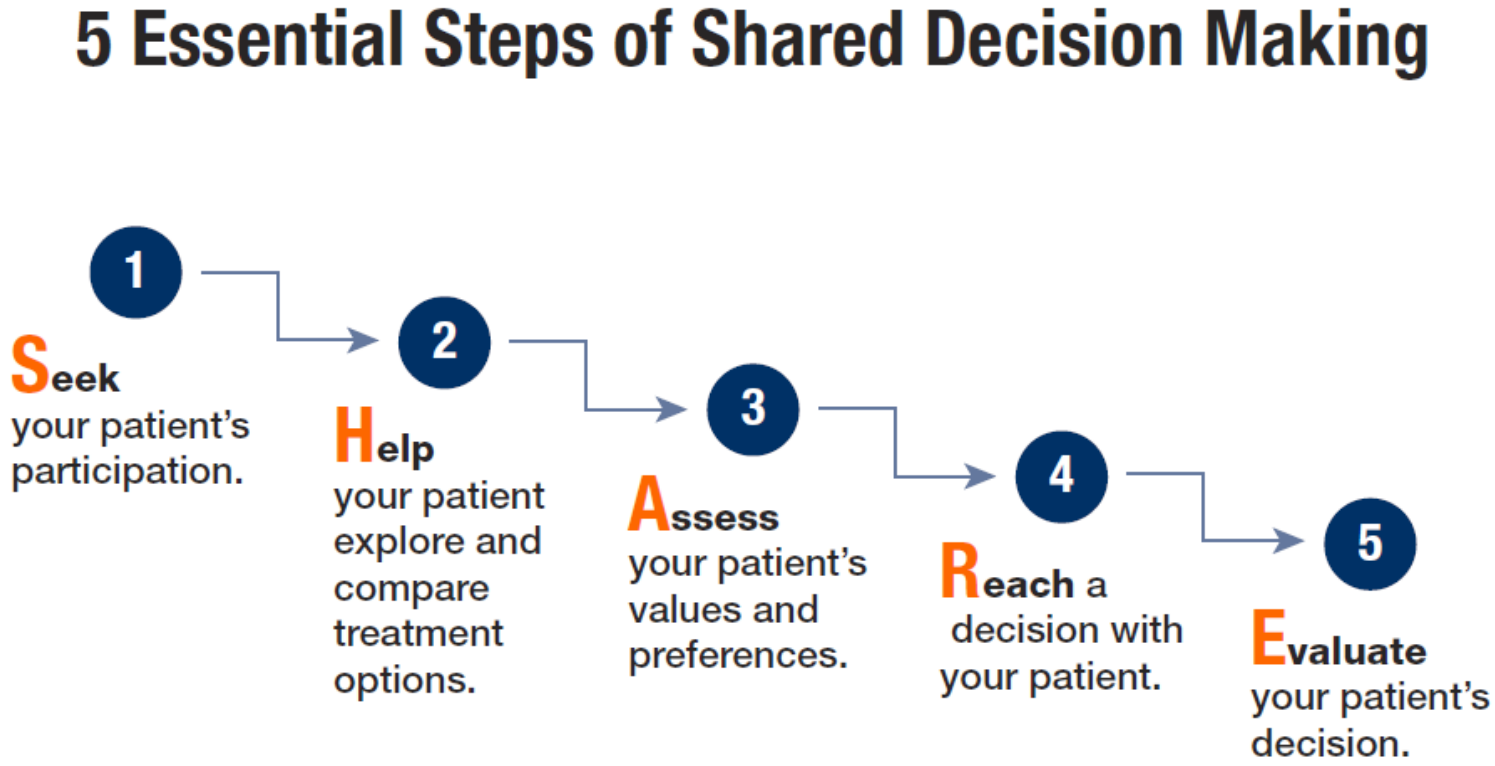
The 5-step SHARE approach

Healthcare decisions are based on informed preferences of both the patient and practitioner.



Agency for Healthcare Research and Quality. The SHARE Approach.

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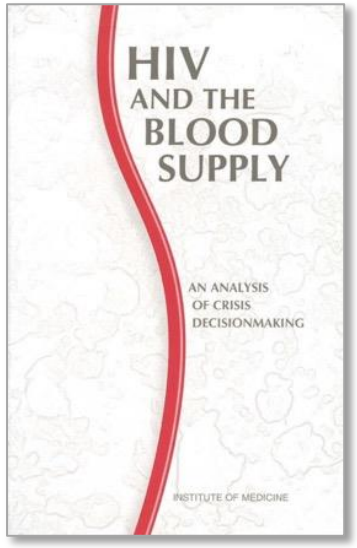
SDM Adopted in Hemophilia in 1980s



Blood safety is a shared responsibility of many diverse organizations. They include … manufactures, groups like the NHF, and others.[1]

How is medical decision-making shared? The case of haemophilia patients and doctors: the aftermath of the infected blood affair in France

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2 July 2003

Keywords: AIDS, clinical relationship, decision-making, haemophilia, prosecution, sociology

Abstract

Objective This article looks at how users and doctors in France have rethought the question of shared decision-making in the clinical field of haemophilia following a major crisis – that of the infected blood affair.

Design We did a qualitative survey based on semi-structured interviews in three regions of France.

Setting and participants The interviews covered 31 clinical doctors of haemophilia and 31 users: 21 adult males with severe haemophilia (21/31), infected (14/21) or not (7/21) with HIV, the infected wife of one of the latter (1/31) and nine parents of young patients with severe haemophilia (9/31), either HIV positive (6/9) or negative (3/9).

[1]Institute of Medicine (US) Committee to Study HIV Transmission Through Blood and Blood Products. HIV And The Blood Supply: An Analysis Of Crisis Decisionmaking. Leveton LB, Sox HC Jr, Stoto MA, editors. Washington (DC): National Academies Press (US); 1995. PMID: 25121199.; [2]Fillion E. How is medical decision-making shared? The case of haemophilia patients and doctors: the aftermath of the infected blood affair in France. Health Expect. 2003 Sep;6(3):228-41. doi: 10.1046/j.1369-6513.2003.00244.x. PMID: 12940796; PMCID: PMC5060181.

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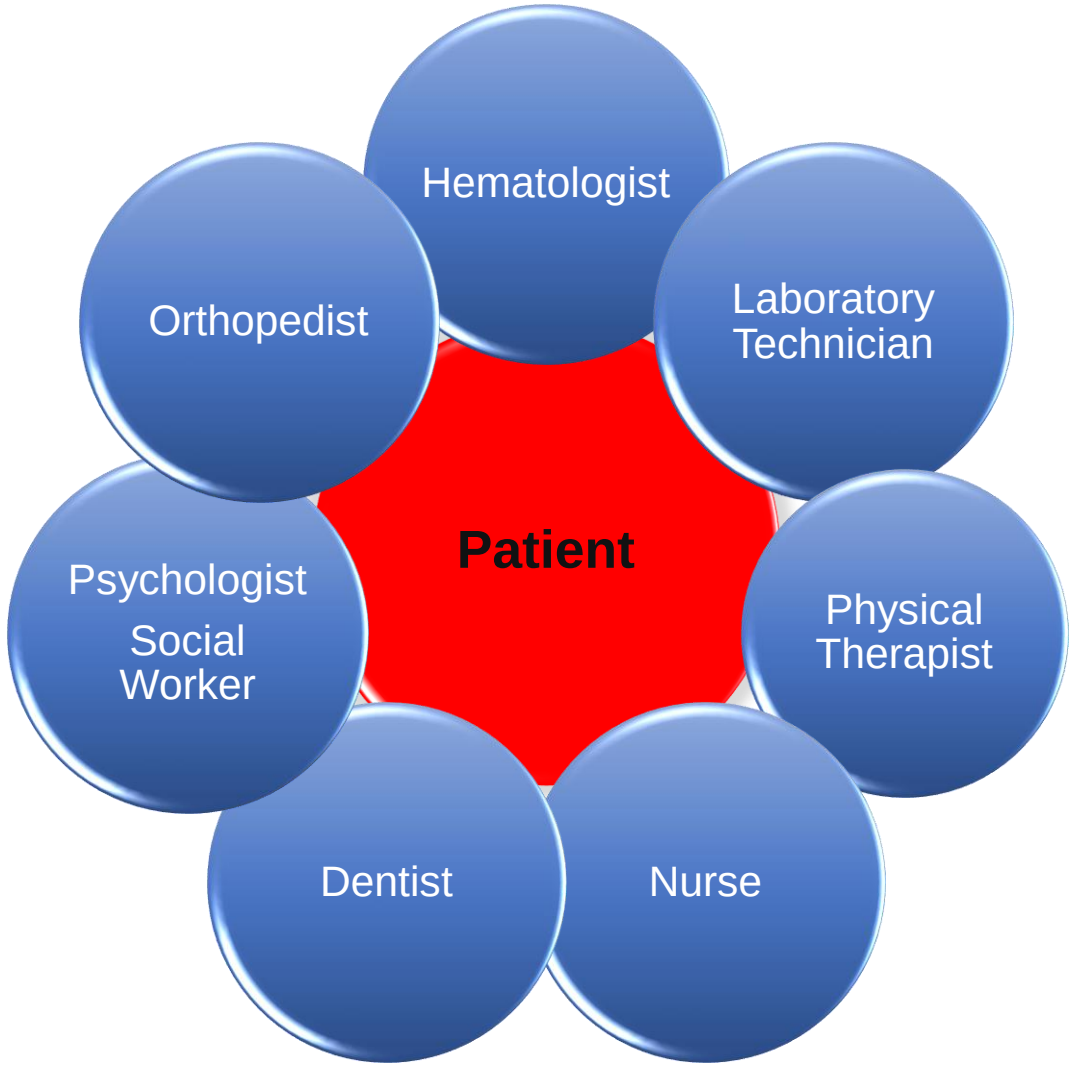
Integrated multidisciplinary care model



Patient is at the center and a core participant.

Essential to achieving optimal health outcomes.

Essential to reducing healthcare utilization.

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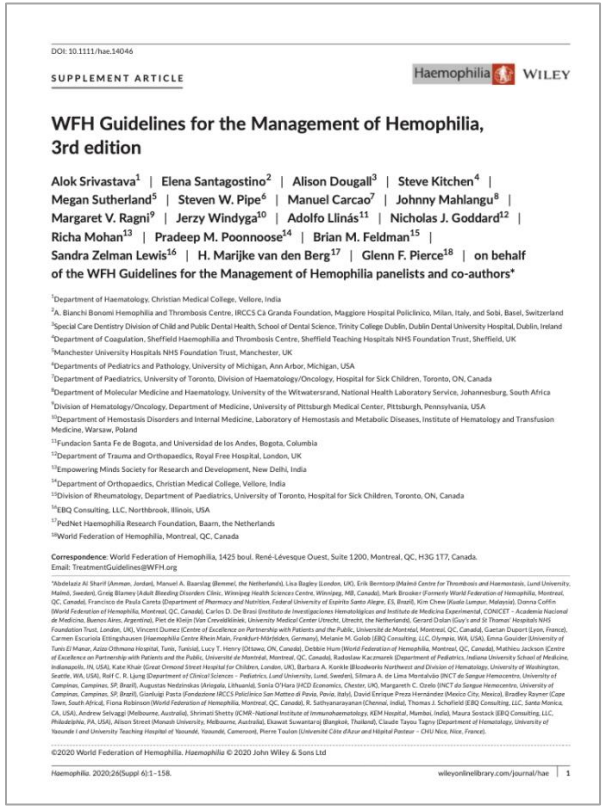
WFH Treatment Guideline Recommendations[1]

- *It is important to involve patients (and their parents/caregivers) in decision-making; incorporate their particular preferences, values, and personal experiences;[2] and obtain their concurrence with short- and long-term treatment and management plans.*
- *All parties should engage in truly shared decision-making through educated discussions about available healthcare options and anticipated outcomes, including evidence-informed guideline recommendations, benefits and risks of the various choices, and expressed concerns and values of the patient and caregivers.*[3]



DOI: 10.1111/hae.14046

SUPPLEMENT ARTICLE

Haemophilia WILEY

WFH Guidelines for the Management of Hemophilia, 3rd edition

Alok Srivastava[1] | Elena Santagostino[2] | Alison Dougall[3] | Steve Kitchen[4] | Megan Sutherland[5] | Steven W. Pipe[6] | Manuel Carcao[7] | Johnny Mahlangu[8] | Margaret V. Ragni[9] | Jerzy Windyga[10] | Adolfo Llinás[11] | Nicholas J. Goddard[12] | Richa Mohan[13] | Pradeep M. Poonnoose[14] | Brian M. Feldman[15] | Sandra Zelman Lewis[16] | H. Marijke van den Berg[17] | Glenn F. Pierce[18] | on behalf of the WFH Guidelines for the Management of Hemophilia panelists and co-authors*

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[1]Srivastava et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. *Haemophilia*. 2020: 26(Suppl 6): 1- 158. https://doi.org/10.1111/hae.14046
[2]Karazivan et al. The patient- as- partner approach in health care: a conceptual framework for a necessary transition Acad Med . 2015 ; 90 (4): 437 – 441;
[3]Fried. Shared decision making—finding the sweet spot . N Engl J Med . 2016 ; 374 (2): 104 - 106 .

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Shared Decision Making = Patient Empowerment

- The overall goal of SDM is to empower patients to understand their vital role in the process, and the consequences of their decisions.
  - Helps keep the patient engaged
  - Ensures a patient is adequately educated about the treatment options to confidently share in the treatment decision.
  - Leads to higher overall longitudinal healthcare quality[1]
  - Improved treatment adherence[2]
  - Leads to increased patient satisfaction[3]

[1]Cramm JM and Nieboer AP. A longitudinal study to identify the influence of quality of chronic care delivery on productive interactions between patients and (teams of) healthcare professionals within disease management programmes. BMJ Open 2014; 4: e005914.
[2]Zolnierek KB and Dimatteo MR. Physician communication and patient adherence to treatment: a meta-analysis. Medical Care 2009; 47: 826–834.
[3]Stewart M, Brown JB, Donner A, et al. The impact of patient-centered care on outcomes. J Fam Pract 2000; 49: 796–804.

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Shared Decision Making Success Factors

- Healthcare providers establish open communication and a trusting relationship with their patients, and present information in a neutral manner, free of overt and implicit or unconscious bias
- Knowledge alone is insufficient for patients to participate in SDM; the power to influence the decision-making process must also be assured
- Healthcare providers should work with patients to identify and track patient-centered outcomes of importance (e.g., decreased pain, increased physical activity, or decrease in missed work or school)

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Shared Decision Making Limitations

- Considering the shared nature of SDM, there is the potential for 'conflict decision' whereby a patient's choice may be challenged by the practitioner or vice versa
  - SDM May be more challenging if the practitioners themselves lack the information and experience required to properly advocate for these therapies.
- Healthcare systems may impose specific procedures or limitations counterproductive to SDM (e.g., incentivizing certain practice targets, limiting interaction time, limiting available treatments or refusing to reimburse treatments jointly selected)
- Caution must be exercised to aid patients who may be overwhelmed, disinterested and/or wish to take a passive role in SDM

Valentino LA, et a;.,Personalising haemophilia management with shared decision making. J Haem Pract 2021; 8(1): 69-79. https://doi.org/10.17225/jhp00178

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Developing Country Considerations

- Standard prophylaxis regimens are cost prohibitive in developing countries, which have limited resources for diagnosis and management.
- Although a desired treatment option may be unavailable or limited within a health system, or an individual may be ineligible, an SDM discussion including all treatments is still a useful process in empowering PWH with valuable information.
- Culturally adapted SDM may be implemented in developing countries to facilitate discussions about treatment, burden of a treatment and expected goals allowing patients to make appropriate decisions tailored to their resources and life situation.

Valentino LA, et a;.,Personalising haemophilia management with shared decision making. J Haem Pract 2021; 8(1): 69-79. https://doi.org/10.17225/jhp00178

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Correlation of Patient-Centered Outcomes and Patient-Reported Outcomes

- PCO and PRO are based on the shared principle that patients have unique perspectives capable of changing and improving the pursuit of clinical questions[1]
- PRO may enhance patient-centered care:[2]
  - Improving interactions between patients and clinicians
  - Identifying benefits and harms of interventions (clinical research)
  - Supplementing policy-making and population surveillance
- Integrating and collecting PRO data to support PCO requires linking it to clinical information from a range of data sources[2]

PCO PatientCentered Outcome, PRO Patient-Reported Outcome

[1]Frank L, Basch E, Selby JV, Patient-Centered Outcomes Research I. The PCORI perspective on patient-centered outcomes research. *JAMA.* Oct 15 2014;312(15):1513-1514; [2]Snyder CF, Jensen RE, Segal JB, Wu AW. Patient-reported outcomes (PROs): putting the patient perspective in patient-centered outcomes research. *Med Care.* Aug 2013;51(8 Suppl 3):S73-79.

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The NEW ENGLAND JOURNAL of MEDICINE

Perspective

Standardizing Patient Outcomes Measurement

Michael E. Porter, Ph.D., M.B.A., Stefan Larsson, M.D., Ph.D., and Thomas H. Lee, M.D.

The arc of history is increasingly clear: health care is shifting focus from the volume of services delivered to the value created for patients, with “value” defined as the outcomes achieved relative to the costs.[1] But progress has been slow and halting, partly because measurement of outcomes that matter to patients, aside from survival, remains limited. And for many conditions, death is a rare outcome whose measurement fails to differentiate excellent from merely competent providers

viders to embrace accountability for results.

If we’re to unlock the potential of value-based health care for driving improvement, outcomes

example, only 139 (7%) are actual outcomes and only 32 (<2%) are patient-reported outcomes (see bar graph).[2] Defaulting to measurement of discrete processes is un-

Perspective

DECEMBER 23, 2010

What Is Value in Health Care?

Michael E. Porter, Ph.D.

In any field, improving performance and accountability depends on having a shared goal that unites the interests and activities of all stakeholders. In health care, however, stakeholders have

myriad, often conflicting goals,

Value — neither an abstract

value is a central challenge. Nor is value measured by the process of care used; process measurement and improvement are important tactics but are no substitutes for measuring outcomes and costs.

- What matters to patients are outcomes that encompass the whole cycle of care
- Historically, outcomes measurement has focused on clinical status and left out functional status

Value in Healthcare = Value Created for Patients

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Porter ME, et al. N Engl J Med 2010, 2016

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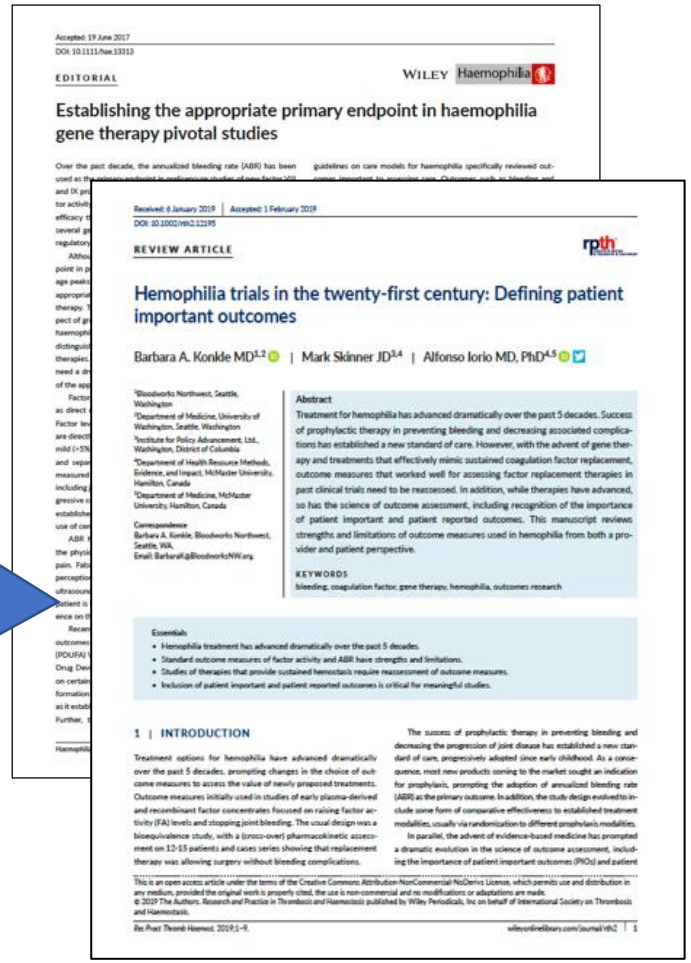
It is clear that although there have been great advances …, more needs to be done not only to develop new therapies …, but to address broader economic, social, and educational barriers that still remain.

Conclusion FDA Voice of the Patient Report May 2016

…, while therapies have advanced, so has the science of outcome assessment, including recognition of the importance of patient important and patient reported outcomes.



EDITORIAL

Establishing the appropriate primary endpoint in haemophilia gene therapy pivotal studies

REVIEW ARTICLE

Hemophilia trials in the twenty-first century: Defining patient important outcomes

Barbara A. Konkle MD[1,2] | Mark Skinner JD[3,4] | Alfonso Iorio MD, PhD[4,5]

FDA Patient-Focused Drug Development Initiative Voice of the Patient Series Report on Hemophilia A/B, VWD, and Other Heritable Bleeding Disorders. May 2016; Pierce, G. F., et al (2017), Establishing the appropriate primary endpoint in haemophilia gene therapy pivotal studies. Haemophilia, 23: 643-644. doi:10.1111/hae.13313; Konkle BA, Skinner M, Iorio A. Hemophilia trials in the twenty-first century: Defining patient important outcomes. Res Pract Thromb Haemost. 2019;00:1–9. https://doi.org/10.1002/rth2.12195

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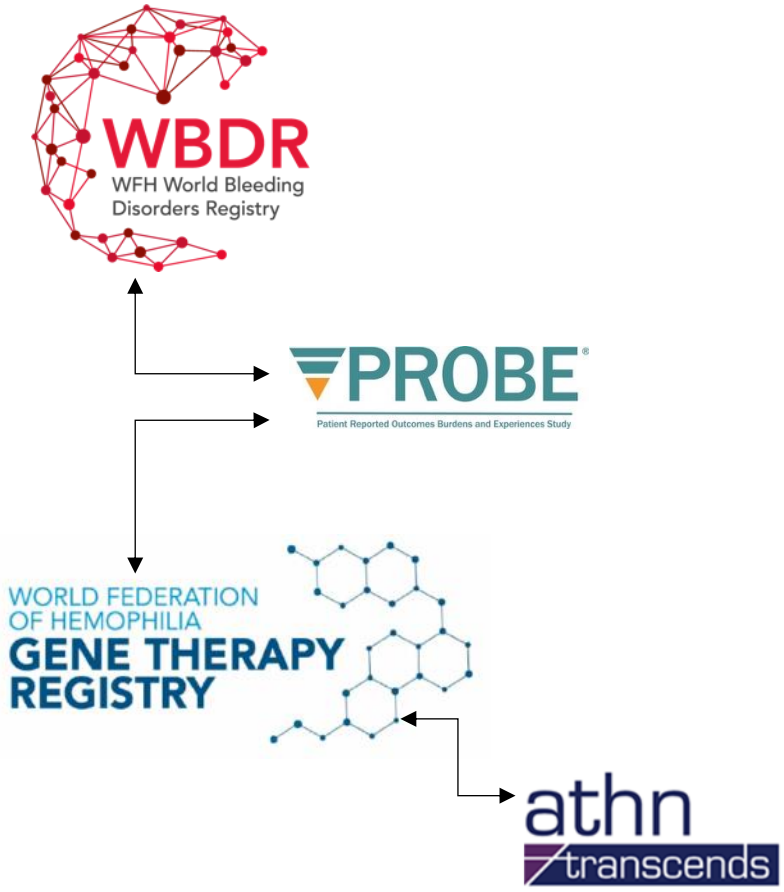
360° View Of Patient Experience
Treatment, Safety, Efficacy, Outcome Data



The only global registry collecting standardized clinical data on people with hemophilia from around the world[1]

Patient-centered data collection on outcomes important to people with hemophilia from around the world[2]

A single global registry for all patients with hemophilia who receive gene therapy collecting long-term data, from clinical trials and post-marketing[3]

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[1]https://www.wfh.org/en/our-work-research-data/world-bleeding-disorders-registry [2] www.PROBEstudy.org [3]https://doi.org/10.1111/hae.14015

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PROBE®

Patient Reported Outcomes Burdens and Experiences Study

www.PROBEstudy.org

- Address healthcare payers' desire to better understand outcomes important to patients
- Illustrate patient knowledge, perspectives and experience can contribute to defining and measuring key health outcomes
- Move advocacy beyond emotion and anecdote to arguments grounded in evidence

[1]Skinner, M. W., et al., (2018). The Patient Reported Outcomes, Burdens and Experiences (PROBE) Project: development and evaluation of a questionnaire assessing patient reported outcomes in people with haemophilia. Pilot and Feasibility Studies, 2018 4:58. doi: 10.1186/s40814-018-0253-0. [2]Chai-Adisaksopha et al., Test-retest properties of the Patient Reported Outcomes, Burdens and Experiences (PROBE) questionnaire and its constituent domains. *Haemophilia*. 2019;25:7583. https://doi.org/10.1111/hae.13649 [3]Chai-Adisaksopha C, et al., Psychometric properties of the Patient Reported Outcomes, Burdens and Experiences (PROBE) questionnaire BMJ Open 2018;8:e021900. http://dx.doi.org/10.1136/bmjopen-2018-021900 [4]Chai-Adisaksopha, C, et al., Exploring regional variations in the cross-cultural, international implementation of the Patient Reported Outcomes Burdens and Experience (PROBE) study. *Haemophilia*. 2019; 25: 365 372. https://doi.org/10.1111/hae.13703/hae.14410

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Consistent collection of relevant well-specified Patient-Reported Outcomes

Aims	Collect and report relevant outcomes within clinical studies	Increase predictability and consistency of payer and Health Technology Assessment decisions	Longitudinal data collection on outcomes meaningful to the quality of life and functioning of patients
Uses	Market Authorization Product Registration	Advocacy Insurance Coverage Ministry of Health	**Shared Decision Making** **Clinical Applications** **Benchmarking Progress**

PROBE
Patient Reported Outcomes Burdens and Experiences Study

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A Cross-sectional Study in Colombia

- **Acute pain** most frequently reported symptom (80%)
- **Chronic pain** reported by 55%
- 88% reported use of **pain medication**
- 48 % reported **difficulty with activities of daily living**
- 32% reported **requiring use of mobility aids or assistive devices.**
- 25 moderate or severe PWH A completed the PROBE questionnaire during a Hemophilia Educational Bootcamp 29 Nov to 1 Dec 2019 in Medellin, Colombia
- Median age 20 (range 10-59 years)

Characteristic (N=25)		n(%)
Use a mobility aid or assistive device in the past 12mo		8 (32)
Use any medication for pain in the past 12mo		22 (88)
Acute pain in the past 12mo		20 (80)
Chronic pain in the past 12mo		11 (44)
Difficulty with ADLs		12 (48)
Clinically significant inhibitors		7 (28)
Current treatment regimen	Regular prophylaxis	18 (72)
	Intermittent, “periodic” prophylaxis	5 (20)
	Episodic (“on-demand”)	1 (4)
	Immune tolerance induction (ITI)	0
	No treatment available	0
	Other	1 (4)
Target joints		19 (76)
More than 2 spontaneous bleeds in a joint in the past 12 mo		13 (52)

PROBE
Patient Reported Outcomes Burdens and Experiences Study

Vicaya et al. EAHAD 2021 , ABS060, ABS109

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Impact of Fear & Anxiety on Physical Activity

Global Findings From The HemActive Study

- Experience or fear of joint damage, joint deterioration, bleeding, and pain can influence behavior and physical activity in persons with hemophilia (PWH).
- Anxiety and fear represent emotional factors that drive PWH to adjust their physical activities.
- PWH were equally or more likely to adjust activities due to fear of vs. experiencing an event.(Fig 1)
- PWH were more likely to stop participating in an activity after experiencing an event vs. fear of that event.(Fig 2)
- **Three-fourths of PWH adjusted their activities (average 2 days per week), while nearly half stopped activities due to their hemophilia.**

Figure 1: Reasons why PWH **adjust** activities

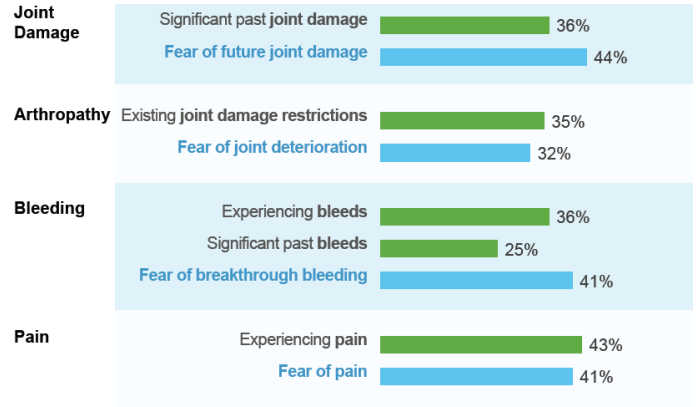
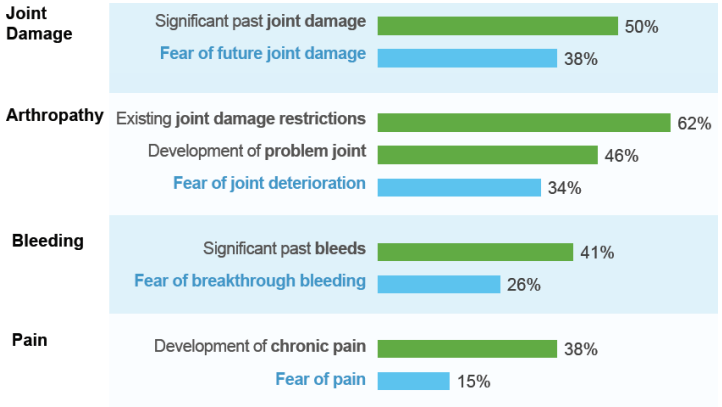


Figure 2: Reasons why PWH **stop** activities



Cross-sectional Study of 275 participants (194 PWH; 81 caregivers); 167 (61%) and 83 (39%) had severe or moderate hemophilia; 67% on prophylaxis

Skinner et al, HemACTIVE Study EAHAD 2020 P304

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Traditional Clinical Outcomes

- Lifespan (survival)
- Factor levels (peaks & troughs)
- Bleeding frequency (annualized bleed rate, target joints)
- Function and mobility
- Joint outcomes (structure, range of motion)

Patient Relevant Outcomes

- Educational pursuit
- Work / Career Opportunities
- Family / Social life engagement
- Activity / Sports goals
- Decreased burden of illness

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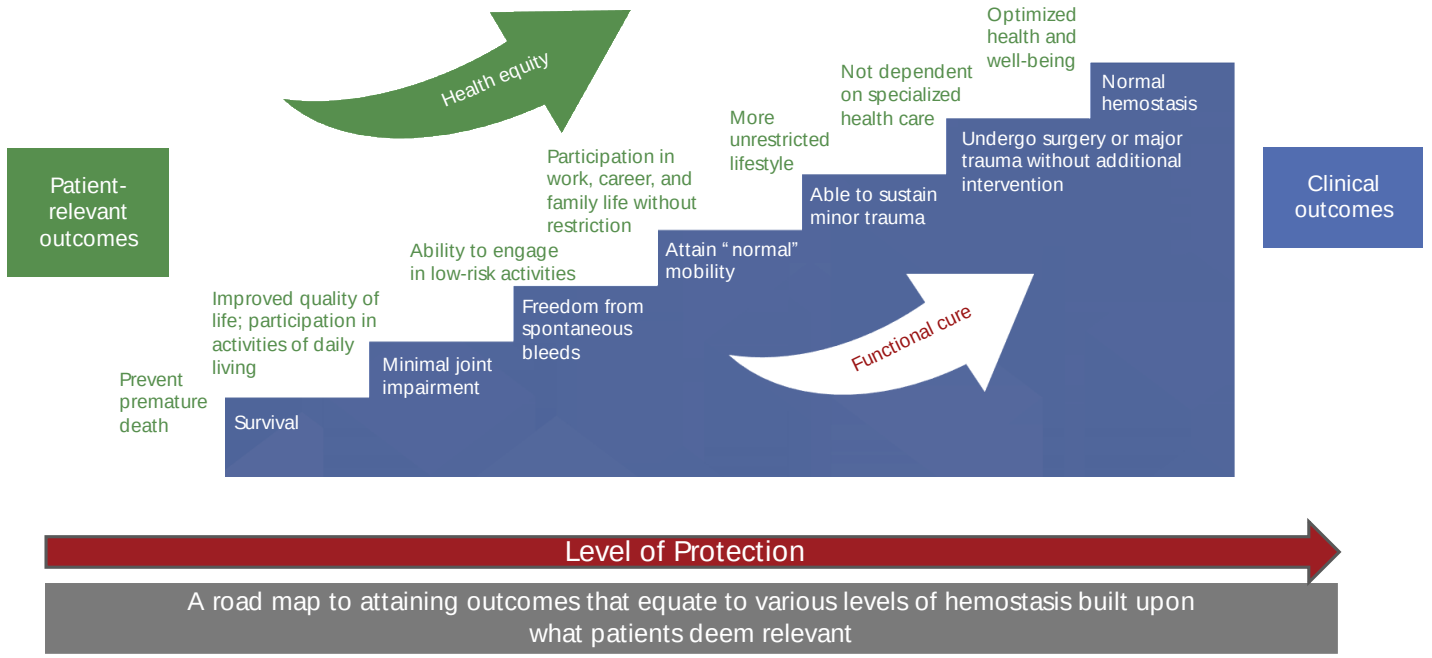
Achieving the Unimaginable: Health Equity

Redefining expectations

- **Freedom** from both lifestyle and medical restrictions caused by hemophilia
- **Tracking** clinical and patient-centric outcomes in parallel
- The **value** of a treatment is not limited to efficacy endpoints alone, but rather provides a stepwise approach towards a functional cure (haemophilia-free mindset) and health equity
- Turning **aspirations** into realistic achievements with novel treatments



Skinner, MW, Nugent, D, Wilton, P, et al. Achieving the unimaginable: Health equity in haemophilia. *Haemophilia*. 2020; 26: 17– 24.

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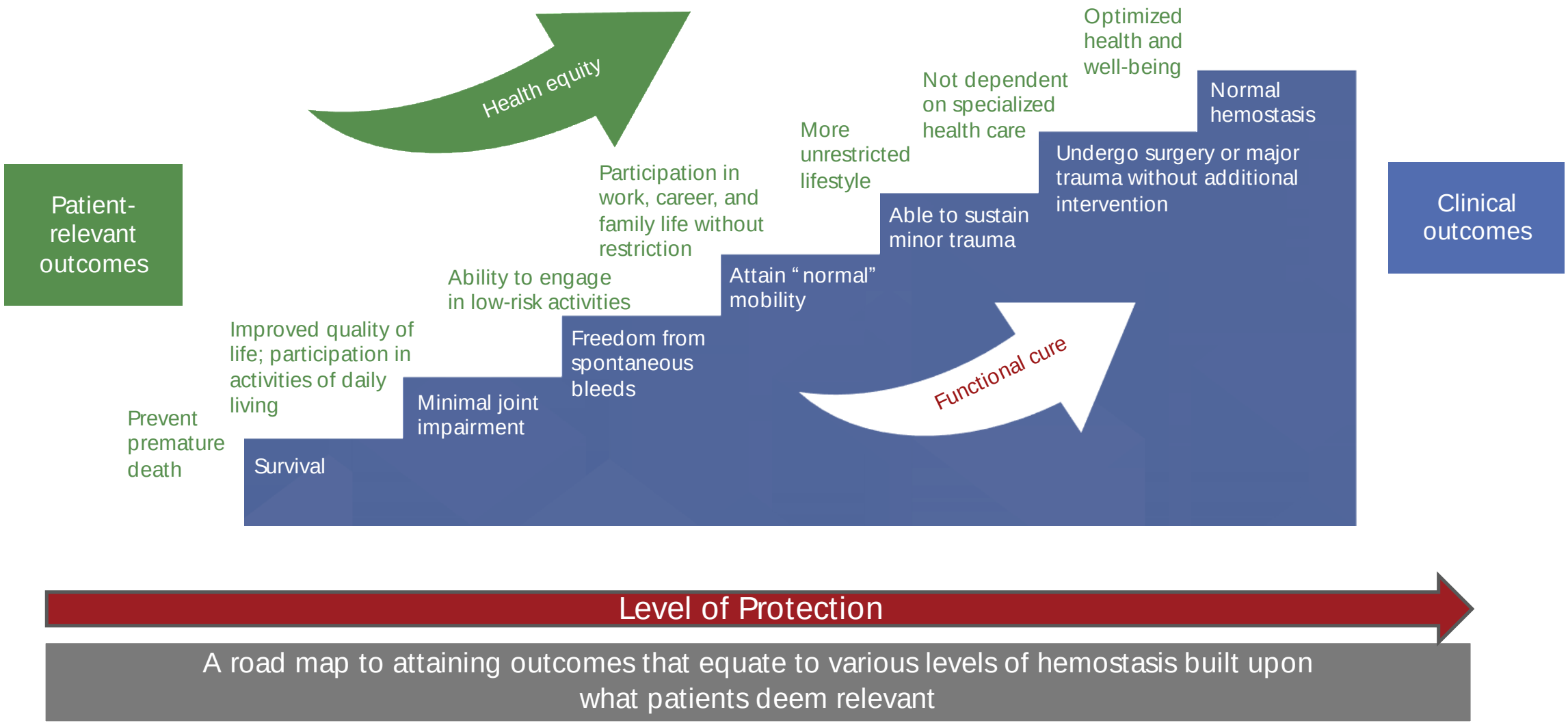

Skinner, MW, Nugent, D, Wilton, P, et al. Achieving the unimaginable: Health equity in haemophilia. *Haemophilia*. 2020; 26: 17– 24.

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Key Takeaway Messages

When patients are fully informed of all their available treatment options, they make choices that are more aligned with their preferences and values; therefore, leading to a higher quality of care.[1]

Evolving technologies to normalize hemostasis along with assessment of **patient-centric outcomes** open the possibility of attaining freedom from lifestyle restrictions caused by hemophilia, ameliorating inequities caused by the disease.[2]

The overall goal of SDM is to **empower** patients to understand their vital role in the process, and the consequences of their decisions.

[1]Foundation for Informed Medical Decision Making; [2]Skinner MW, et al. *Haemophilia*. 2020;26(1):17-24.

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Every man dies,
not every man really lives.

Attributed to William Wallace
Braveheart
Scottish revolutionary
1270-1305

The goal of treatment is not simply to add years to a person with hemophilia's life, but to add life to their years!

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Resources to Support SDM

NAME	DESCRIPTION	URL
Ottawa Personal Decision Guides	Designed to help people identify their decision making needs, plan the next steps, track their progress, and share their views about any health-related or social decisions	*https://decisionaid.ohri.ca/decguide.html*
Laval University and McMaster University	Prepares the clinician to discuss scientific evidence with the patient (or caregiver) so they can make an informed decision together	*https://www.boitedecision.ulaval.ca/fileadmin/documents/Boites_PDF/Prophylaxis/Dbox_prophylaxis_treatment_options_AN.pdf*
The Mayo Clinic Shared Decision Making National Resource Center	Advances patient-centred medical care by promoting shared decision making through the development, implementation, and assessment of patient decision aids and shared decision making techniques	*https://shareddecisions.mayoclinic.org/*
Dartmouth-Hitchcock and the Dartmouth Institute Center for Shared Decision Making	Provides patient decision aids, decision support counselling, and facilitation of advance care planning discussions	*https://www.dartmouth-hitchcock.org/shared-decision-making/resources*
The National Learning Consortium	SDM fact sheet with an overview of the process and links to other resources	*https://www.healthit.gov/sites/default/files/nlc_shared_decision_making_fact_sheet.pdf*

Valentino LA, et a;.,Personalising haemophilia management with shared decision making. J Haem Pract 2021; 8(1): 69-79. https://doi.org/10.17225/jhp00178

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Thank you

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