

The background features a light blue and white color scheme. On the left, there are faint, overlapping fingerprint patterns in a light blue color. On the right, there is a faint, light blue silhouette of a human heart. The text is centered in the middle of the image.

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# **ACCREDITED PANEL DISCUSSION**

**Normalising Haemostasis in Haemophilia**



**A New Standard of Care**

**September 2023**

# Acknowledgement and Disclosures

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## Expert Disclosures:



**Assoc. Prof. Jan Blatný** has received grants, research support, lecture honoraria, consultation fees from: CSL Behring, Novonordisk, Roche, Sobi, Takeda, Roche



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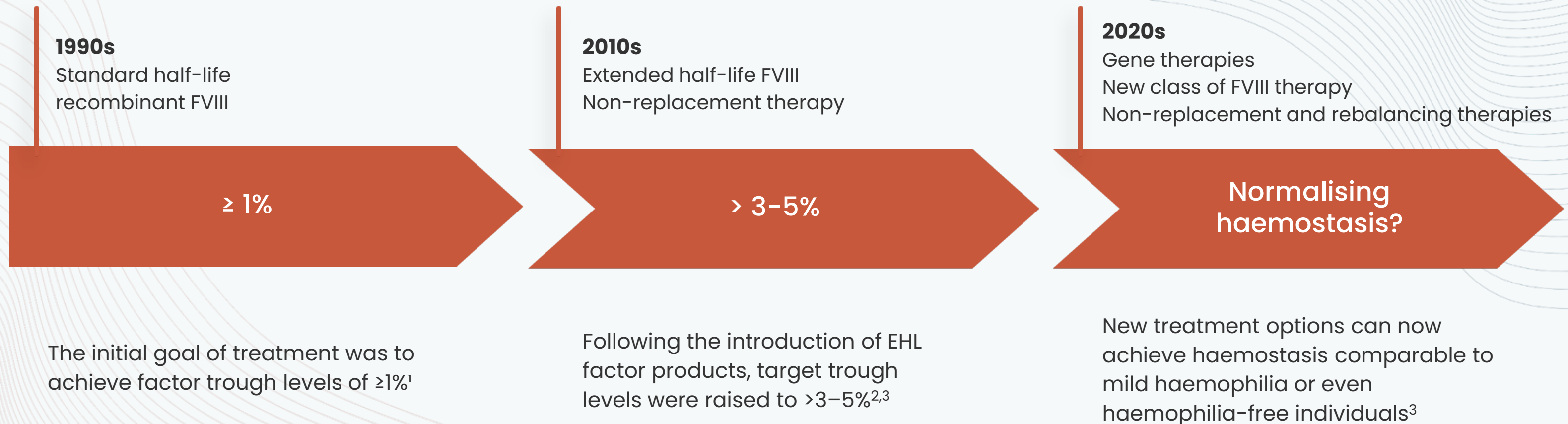
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# The Therapeutic Landscape for Haemophilia A is Evolving



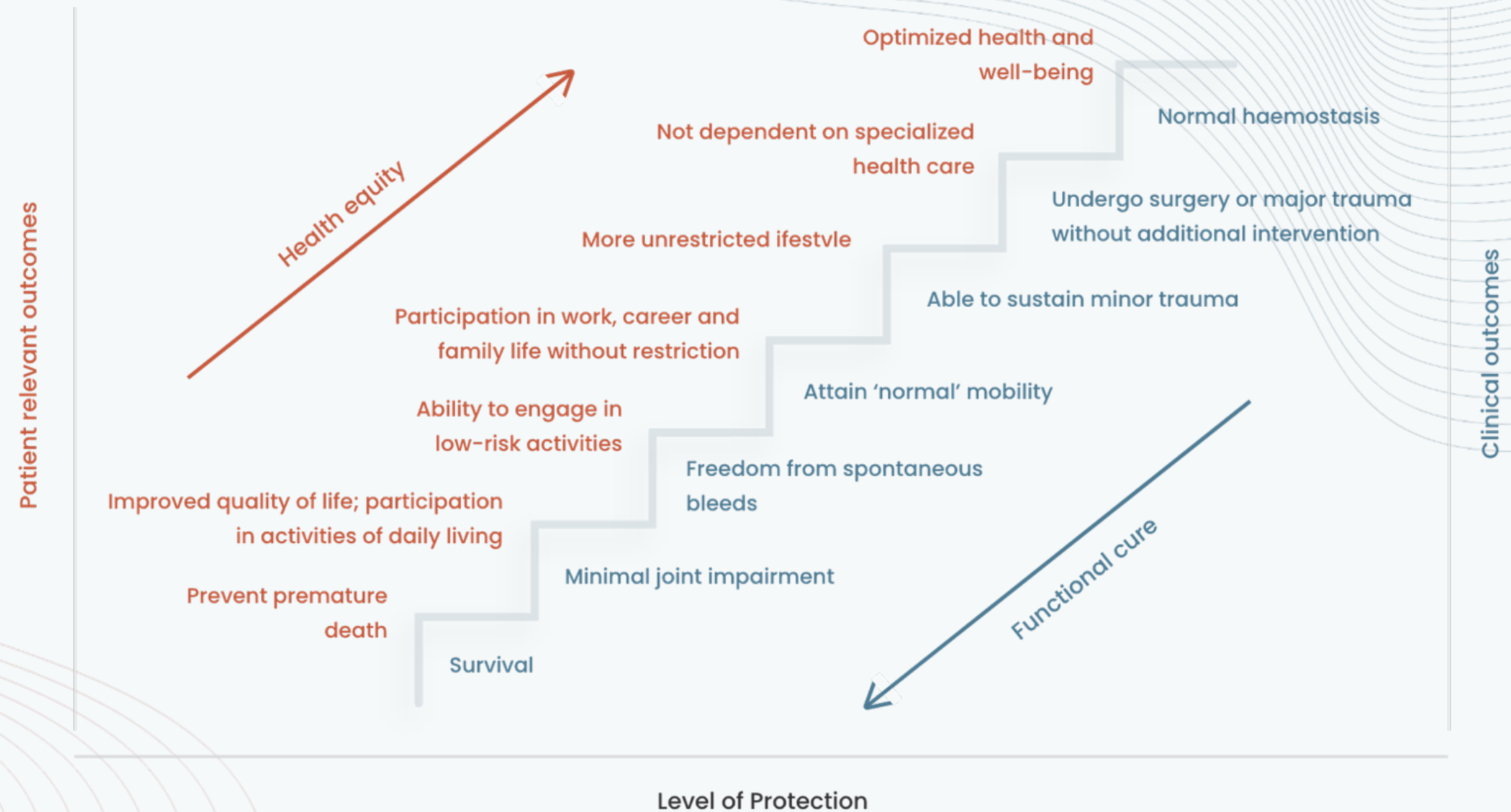
EHL: extended-half life. FVII: Factor VIII  
Figure adapted from Mannucci et al.<sup>3</sup>

1. Skinner, M.W. et al. Haemophilia 2020; 26:17-24. 2. Srivastava, A. et al. Haemophilia 2020; Suppl 6:1-158. 3. Mannucci, P.M., Haematologica 2020; 105:545-553

# Path Towards Improved Standards of Care and Health-Related Quality of Life



Alignment of clinical target and patient-relevant outcomes is a necessary step towards achieving health equity



# Why is Normalisation Important to People with Haemophilia?

## An aspirational and visionary standard of care

### Normalisation of haemostasis<sup>1</sup>

By normalising haemostasis, additional treatment for high-risk situations may no longer be needed

Normalising haemostasis prevents subclinical bleeding, halting any deterioration in joint health

### Normalisation of life<sup>1</sup>

Preservation of joint health should stop the development (or progression) of chronic pain

Patients can confidently take part in all physical and social activities, leading to better joint health and improved HRQoL

Patients will spend less time thinking about their illness and more time living their lives

# How Can We Achieve Normalisation?



The innovation of novel treatments, including new class factor replacement therapy, non-replacement and rebalancing treatments and gene therapy have resulted in improved bleed and joint protection for patients with haemophilia A



These strategies offer different mechanisms of actions, characteristics, and clinical implications while offering improved protection from bleeding

Harnessing these strategies should close the gap between currently accepted activity targets and elevate the standard of care



A therapy option with a simple dosing schedule may result in simpler clinical decision making



Increased adherence will empower patients to proactively manage their haemophilia and HCPs and MDTs will instead focus on the management of comorbidities



Normalisation requires a holistic approach to treatment and care provided by integrated and effective MDTs, regardless of the modality of treatment



# How Can We Elevate the Standard of Care in Haemophilia A?

Aim for **excellent protection** from bleeds with reduced treatment burden, using advanced therapeutic options where available

Take the person's **stage of life** into account; treatment needs can change as the patient grows older and different stages of life are associated with different needs

Use a **personalised approach** and empathically listen to patient feedback, in order to fully understand their experience of the disease, preferences, goals and aspirations

**Monitoring outcome measures** by regular follow - up, such as ABR, HJHS, imaging (ultrasound, MRI), HRQoL and pharmacokinetic measurements

When taking treatment decisions, take any **haemophilia - related complications** (such as inhibitor status, joint health, mobility restrictions and chronic pain) and **co - morbidities** (including depression, anxiety) into account

# Elevating the Standard of Care in Haemophilia A – What to Consider:



No perfect outcome score exists

Actively listen to your patients and use their aspirations as the framework for treatment goals, as well as outcome measures used in clinical trials



How can you demonstrate a higher standard of care

Longitudinal follow up of people with haemophilia receiving treatment to show we achieved a higher standard

–

Focus on small steps to improve your standard of care in haemophilia A

# Normalisation – A Visionary Concept

Normalisation of haemostasis is applicable for all people with haemophilia

Normalisation of life will need to be tailored to each patient, taking into account:

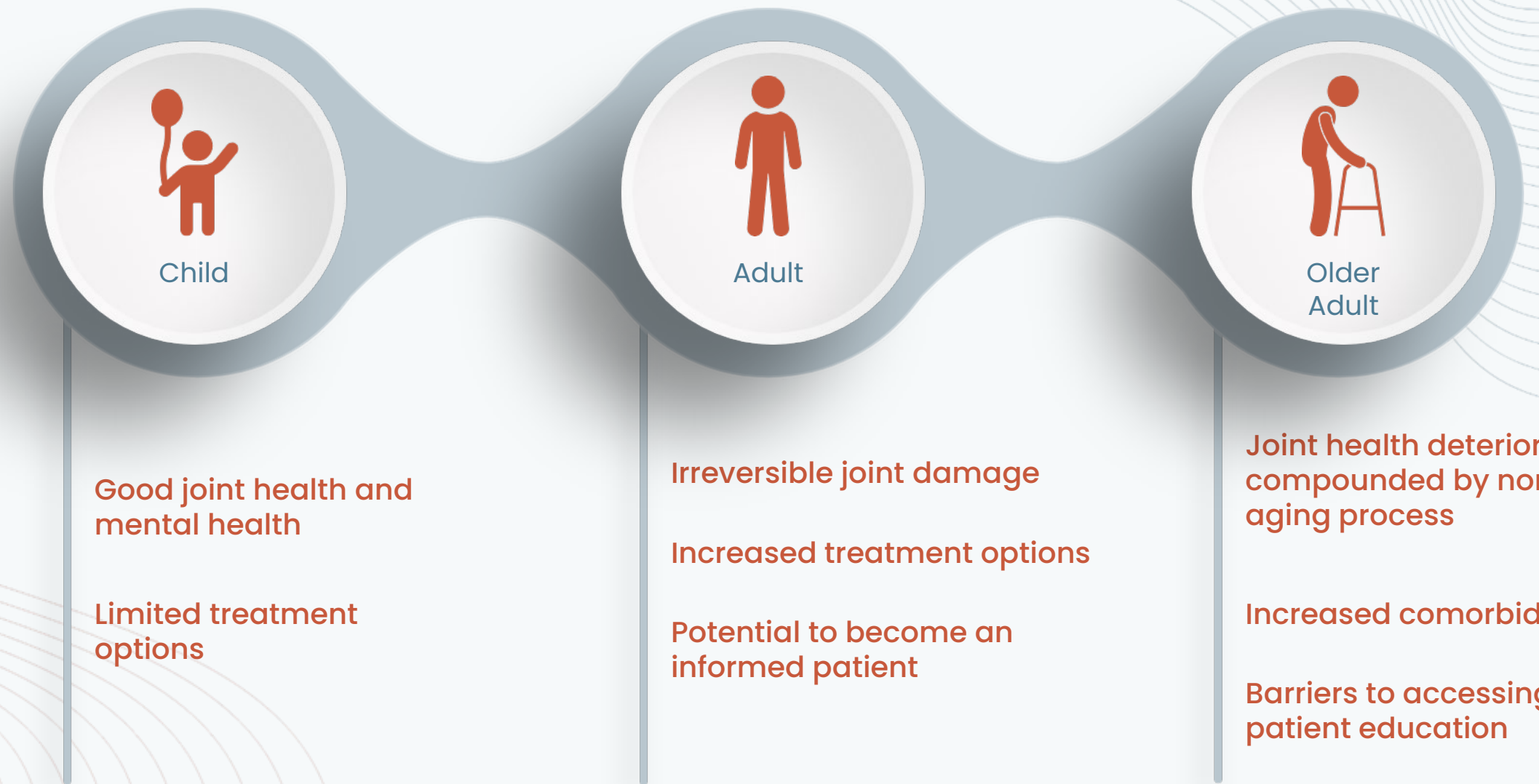
Phase of life

Pathophysiology

Treatment options

Comorbidities

The patient's personal aspirations



# Clinical Takeaways



Normalising haemostasis is a realistic and appropriate aspiration for patients, using available novel treatment options



Normalising haemostasis is more than just zero bleeds



Normalising haemostasis as a treatment goal is adaptable for all stages of life

# References

Holme, P.A. et al., 2023 unpublished manuscript

Mannucci, P.M., Haematologica 2020; 105:545-553

Skinner, M.W. et al. Haemophilia 2020; 26:17-24

Srivastava, A. et al. Haemophilia 2020; Suppl 6:1-158

A light blue world map is centered in the background. Numerous dark blue location pins are scattered across the map, with a high concentration in North America and Europe. The text 'COR2ED' is overlaid on the map. The letters 'C', 'O', 'R', and '2' are in a dark blue color, while 'E' and 'D' are in a reddish-orange color.

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