

Women and girls with bleeding disorders

Suzanne O'Callaghan

There were several sessions on women and girls at the WFH 2024 World Congress, all with the strong message that it's time for action on female-specific research, treatment and care.

Women and girls with bleeding disorders

Co-chairs: Paula James, Canada;
Immaculada Soto-Ortega, Spain

New haemophilia carrier classification - how do we adapt our care pathways?

~ Jill M. Johnsen, USA

Impact of therapeutic innovations on women and girls with hemophilia

~ Cedric Hermans, Belgium

HMB - from menarche to menopause

~ Nicola Curry, UK

One session that stood out for me was a medical session on women and girls with bleeding disorders where the speakers were discussing the nuts and bolts of how to make meaningful changes to the management of women and girls with bleeding disorders.

Dr Jill Johnsen, a physician scientist from the US, began the session by raising the concern at how few females predicted to need care have touched a Haemophilia Treatment Centre (HTC) – only 18% in the US, which is well-resourced and could expect to have a higher number of patients.

She proposed that there should be:

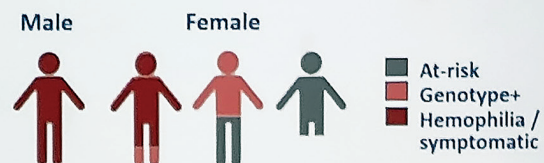
- Early diagnosis and care over the lifespan
- Testing for genotype at birth, confirming factor level as an infant
- Checking for bleeding risk and heavy menstrual bleeding.

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Many females are systemically missing in clinics, registries (and research)

For every male with hemophilia¹:

- ~ 2.77 females are at-risk
- ~ 1.56 are genotype positive
- ~ 0.8 have hemophilia / bleeding



Sources (hemophilia A & B)	Males	Females	% of females predicted to need care
U.S. HTCs (2012-2020) ¹	23,728	2,504	18%
WFH World Bleeding Disorders Registry (2022) ²	10,899	94	1%

¹Reviewed in Hermans C, Johnsen JM, Curry N. Haemophilia. Early view. <https://doi.org/10.1111/hae.14983>

²WBDR 2022 Data Report. <https://wfh.org/research-and-data-collection/world-bleeding-disorders-registry/>

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Speaking about haemophilia treatment, Dr Cedric Hermans, President of EAHAD (European Association of Haemophilia and Allied Disorders), commented that the *'therapeutic revolution has so far been primarily male-centric, benefiting mainly boys and males with haemophilia.'* He pointed out that mostly females have mild or moderate haemophilia but there is very little treatment experience data. He proposed that:

- The treatment goals for females should be *'conversion into a mild or normal phenotype'*.
- Researchers need to identify agents that can lead to normal haemostasis in females - nanobodies could be very useful.
- Females need different endpoints to males in clinical trials – and there needs to be clinical trials specific to females.

Dr Nicola Curry, a haematologist from Oxford in the UK, followed him with a discussion of heavy menstrual bleeding (HMB) and the severity of its impact on women and girls with bleeding disorders. Iron deficiency, for example, is a major problem and has a negative effect on quality of life.

In the UK HTC's have developed a Menarche Plan, where they discuss what is normal and what might occur at menarche (first menstrual period). However, the challenge for women with bleeding disorders is that periods may become heavier over their lifetime. She underlined that there needs to be an option for patient-initiated follow-up and women invited to contact the HTC when they have problems.

The speakers and audience debated how best to record and assess menstrual bleeding. Nicola Curry noted that tools to document menstrual bleeding such as Pictorial Blood Assessment Charts (PBACs) are important, but they need to reflect new forms of sanitary protection and be

available electronically, eg as an app. A member of the audience asked about having one standard Bleeding Assessment Tool (BAT) for diagnosis internationally. Cedric Hermans responded that there still needs to be work on this but a good first step would be to use existing BATs. Australia's Dr Janis Chamberlain also pointed out the quality of life evaluation tools that have been developed by her team in Newcastle.

The session finished with a discussion about research and treatments for females with bleeding disorders – that is a need for:

- More research into effective treatment for females with VWD.
- A different approach to physiological bleeding such as heavy menstrual bleeding
- Challenging the exclusion of females from clinical trials due to hormonal treatment for heavy menstrual bleeding.
- *'We need to do better.'*

READ MORE

This session was published as a State-of-the-Art review article in *Haemophilia* journal –

Hermans C, Johnsen JM, Curry N. **Women and girls with inherited bleeding disorders: Focus on haemophilia carriers and heavy menstrual bleeding.** *Haemophilia.* 2024;30(Suppl. 3):45–51. <https://doi.org/10.1111/hae.14983>

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