ISTH 2024 Congress: novel therapies

Ashley Fletcher

Ashley Fletcher attending the Congress

At the **ISTH 2024 Congress** in Bangkok, Thailand, some exciting new treatments for haemophilia were highlighted. The treatments use a range of innovative new technologies that target different proteins in the body to prevent bleeding in haemophilia.

One important new treatment is called **Inno8**, a novel VHH-based FVIIIa-mimetic molecule, a new type of medicine for people with severe haemophilia A. It works very well, lasts a long time in the body, and can be taken as a pill once a day. This means people wouldn't need to get injections anymore, making it easier for them to take their medicine. However, it is currently at a very early stage and being tested in Beagle dogs rather than humans, so it may be quite a way off from being available.

But what about treatments that are almost ready? There are several that have finished phase 3 trials, which means they are very close to being available to be evaluated for government funding.

These include:

- **Mim8**: Works like emicizumab but lasts longer and could be available in 2026.
- **Fitusiran:** An RNA interference (RNAi) therapy targeting antithrombin, expected to be ready in 2026.
- **Concizumab**: A monoclonal antibody designed to inhibit tissue factor pathway inhibitor (TFPI), improving clot formation, expected in 2025.
- **Efanesoctocog alpha**: A new recombinant factor VIII with an extended half-life, also expected in 2025.

These treatments are showing promise and could soon help people with haemophilia manage their condition better.

Some other treatments are still being tested in phase 2 or 3 trials, meaning they are not yet ready but look promising for the future. These include:

- **NXT007**: Could be available by 2030. A bispecific antibody that mimics the cofactor function of activated FVIII.
- **SerpinPC**: Might be ready by 2028 and has shown promise in reducing bleeding episodes as it is a protein C inhibitor.
- **Marstacimab**: Another promising treatment, an antibody that blocks a protein called tissue factor pathway inhibitor (TFPI), could be available in 2027.

There was also talk about **SS315**, a novel FVIIIa-mimetic bispecific antibody, as another new medicine that might be available in the future.

All these new treatments show that haemophilia care is getting better, with more options that might have fewer side effects and make life easier for patients.

The Congress highlighted the need to choose the right treatment based on how safe it is, how often it needs to be taken, and how much it costs. The new oral treatments presented at ISTH 2024 give hope for a better future for people with haemophilia, aiming to make their lives easier and healthier.

Ashley Fletcher is the AHCDO Australian Bleeding Disorders Registry (ABDR) Research Fellow.