Von Willebrand disease

Suzanne O'Callaghan

Old Montreal *Photo: Shauna Adams*

Plenary (Arosenius Lecture) - Towards novel treatment options in von Willebrand disease

Speaker ~ Peter Lenting, Director of Research, French National Institute of Health and Medical Research - INSERM, Paris, France

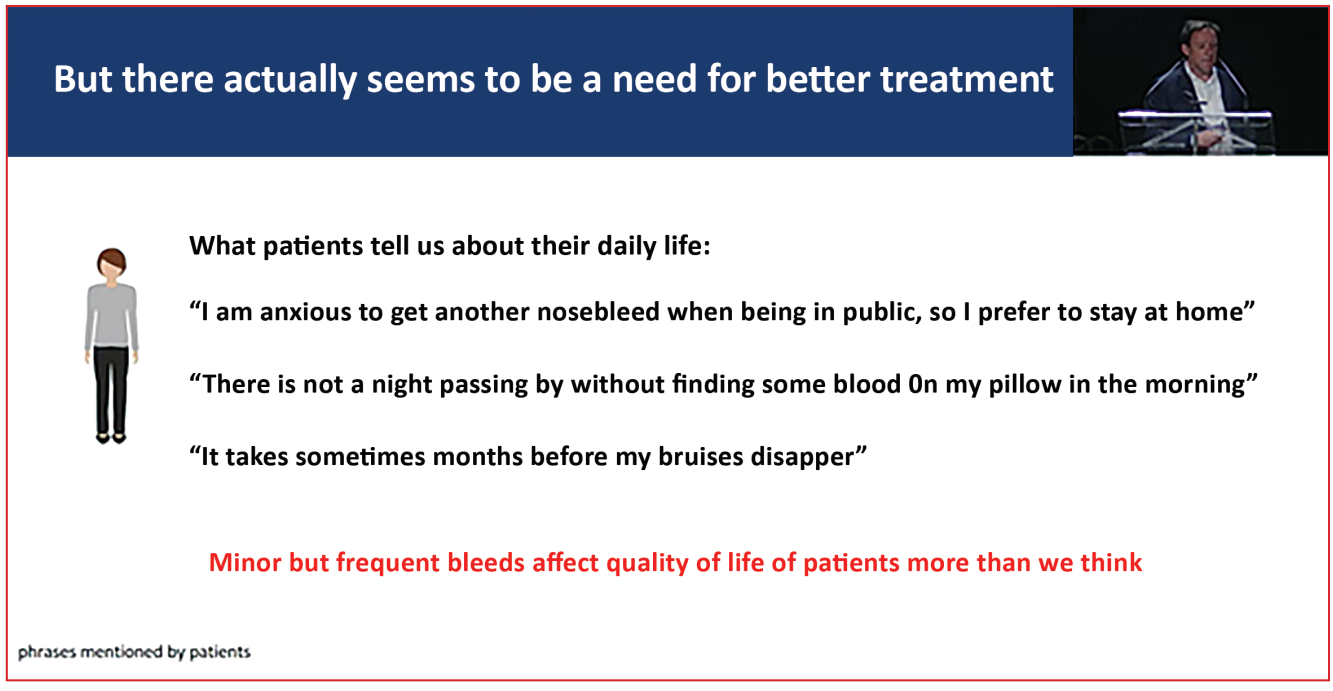
In the Arosenius Lecture medical plenary Peter Lenting explored some of the treatment issues for people with von Willebrand disease (VWD) and outlined some innovative treatments that are currently in the pipeline.

Although his VWD research is highly scientific, Peter Lenting consults his patients with VWD regularly to understand the impact of VWD and what outcomes they are seeking from treatment.

Clinicians would usually expect the biggest impact on quality of life to occur in severe forms of VWD. However, he has found there are also many issues for people with mild forms who experience frequent minor bleeds, especially women. Most patients with VWD have Type 1 (5-30% von Willebrand factor/VWF) and many are in the category now called VWF ^{low} (30-50% VWF). They have frequent minor bleeds which do bother them and affect their quality of life.

As a result, he proposed the goals of VWD treatment should be:

- To obtain a better efficacy
- To improve quality of life
- Ultimately, to cure the disease.



For his patients, this included:

- Reducing the volume that needs to be infused
- Subcutaneous (under the skin rather than into a vein) and less frequent infusions
- Gene therapy as a cure.

He outlined some current clinical trials and experimental studies and how they are contributing to these goals:

Emicizumab in Type 3 VWD

- subcutaneous, fewer infusions
- improves the half-life of factor VIII (8); might improve haemostasis (blood clotting)
- shows some promising results in early studies
- will still need to correct the VWF deficiency in some patients.

Nanobody therapy –

currently experimental mice studies

- increases factor VIII and VWF levels for at least 7 days
- corrects bleeding in VWD Type 1
- could this be used as prophylaxis in humans - a subcutaneous infusion once weekly or every 2 weeks?
- or could the nanobody molecule be used as gene therapy?

Suzanne O'Callaghan is HFA Policy Research and Education Manager.

Robyn Shoemark

Changing treatment, changes diagnosis

Chair ~ Kate Khair, Director of Research, Haemnet, London, UK

Non-factor therapy in persons with acquired hemophilia A and von Willebrand disease

~ Ming Lim, Assoc Prof, Division of Hematology and Hematologic Malignancies, University of Utah Health, Salt Lake City, USA

For patients with Type 3 VWD, standard treatment is factor VIII and von Willebrand factor (VWF). For patients who have developed an inhibitor to VWF or continue to bleed despite prophylaxis, standard treatment is plasma-derived FVIII/VWF or recombinant factor VIIa and there are few options for new treatments.

Ming Lim reported on a review of off-label use of emicizumab in 8 patients with Type 3 VWD, 3 with an inhibitor and 5 without an inhibitor with frequent bleeds. Results so far looked promising, providing haemostasis for these patients. Further studies need to be conducted to check and confirm the results.

Late-breaking Clinical Research

Chair: ~ Glenn Pierce, Vice President Medical, World Federation of Hemophilia, USA

A phase 1 sequential pharmacokinetic (PK) evaluation of octocog alfa, rurioctocog alfa pegol, and efanesoctocog alfa in severe hemophilia A

~ Annemieke Willemze, Senior Clinical Research Director, Sanofi Genzyme, Amsterdam, Netherlands

Annemieke Willemze discussed the BIVV001 (efanesoctocog alfa) trial results in the Late Breaking Clinical Research session. In question time, there was a discussion about the use of BIVV001 for patients with Type 2N and Type 3 VWD. Results have been similar to people with haemophilia A, with a weekly treatment injection and an increase in the half-life of the treatment to an average of around 43 hours. This is good news for those patients with little to look forward to on the horizon of new treatment options.

Robyn Shoemark is Clinical Nurse Consultant Haemophilia/Haematology at the Kids Factor Zone, The Children's Hospital at Westmead, Sydney, NSW.