

# Progress in Haemostasis

## From individual patients to pathophysiological insights

Johanna A. Kremer Hovinga<sup>1,2</sup>; Rüdiger E. Scharf<sup>3,4</sup>

<sup>1</sup>Department of Hematology & Central Hematology Laboratory, Inselspital, Bern University Hospital, Bern, Switzerland;

<sup>2</sup>Department of Clinical Research, University of Bern, Bern, Switzerland; <sup>3</sup>Division of Hemostasis, Hemotherapy & Transfusion Medicine, and Hemophilia Comprehensive Care Center, Heinrich Heine University Medical Center, Düsseldorf, Germany; <sup>4</sup>Biological Medical Research Center, Heinrich Heine University, Düsseldorf, Germany

In January 2016, *Progress in Haemostasis* was introduced as a subtitle to *Hämostaseologie*. This subtitle was chosen for several programmatic reasons:

- to reflect and communicate recent advances made in our field,
- to promote and catalyze submissions by authors from non-German speaking countries,
- to raise the Journal's profile and reputation, and
- to increase the Journal's attractiveness to the readership inside and outside of the GTH (1).

The current edition of *Hämostaseologie – Progress in Haemostasis* documents that we have made great strides towards achieving these goals. When designing the scientific program of the 61<sup>st</sup> GTH Annual Meeting, a majority of invited speakers agreed to submit a lecture manuscript.

Overall, we received 15 manuscripts, covering four plenary sessions, five personal highlight talks and six state-of-art lectures. These papers are being published in two separate issues of *Hämostaseologie – Progress in Haemostasis*.

We are happy to provide you with this year's GTH congress edition, containing four plenary and two state-of-the-art lecture contributions, all of which are reporting on recent advances made in basic science or clinical research and thus reflecting specifically the progress in hemostasis.

### Correspondence to:

Prof. Dr. Johanna A. Kremer Hovinga  
E-Mail: Johanna.Kremer@insel.ch

**Fortschritte in der Hämostaseologie  
Von individuellen Patienten zu pathophysiologischen Einblicken**

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These communications are not only distributed among subscribers of the *Journal* but also among all attendees of the 61<sup>st</sup> GTH Annual Meeting and will thus remain a keepsake of the Basel Congress 2017.

Fifty-five years ago, on Valentine's day 1962, Ewald R. Weibel, at the time a post-doctoral fellow in the laboratory of George E. Palade at the Rockefeller Institute, New York, later professor of anatomy and dean of the University of Bern, Switzerland, was working late on the electron microscope studying lung sections, when he observed peculiar rod-shaped cell organelles in endothelial cells, „which consisted of a bundle of fine tubules, enveloped by a tightly fitted membrane“ (2).

These cell organelles are now known as Weibel-Palade bodies, their main component is von Willebrand factor. **Marjon Mourik** and **Jeroen Eikenboom** report on the lifecycle of Weibel-Palade bodies from biogenesis, through maturation to secretion and discuss recognized defects affecting or interacting with this lifecycle (3).

Next, **James Byrnes** and **Alisa Wolberg** review our understanding of venous thrombogenesis and put it into context with recent findings (4). Specifically, the authors are highlighting the contribution of an altered blood flow, of factor XIII, fibrinogen as well as of microvesicles and circulating blood cells, especially red blood cells, essential for the typical „red clot“ aspect of venous thrombi, and leukocytes, including formation of neutrophil extracellular traps in this process.

One goal of the Organizing Committee of the 61<sup>st</sup> GTH Annual Meeting was to have a strong pediatric program, covering continuous disorders from childhood to late adulthood including discussion of age-related differences. Autoimmune diseases involving coagulation factors and pla-

telets were identified as a second topic of interest. **Thomas Kühne**, chair of the Intercontinental Cooperative ITP Study group (ICIS), brings these two topics together in his comprehensive review on diagnosis and management of immune thrombocytopenia (ITP) in childhood comparing it to aspects seen in adult patients suffering from ITP (5).

Genome editing with engineered nucleases is a hot topic. **Toni Cathomen** and coworkers nicely review current possibilities of gene editing (6). The authors explain the mode of action of three principal available systems, including zinc-finger nucleases, talens (transcription activator like effector nucleases) and the most promising candidate, CRISPR/Cas9 (6). Moreover, Cathomen et al. also discuss individual advantages and disadvantages and first clinical applications, including (animal) studies in hemophilia.

The recent large cohort and surveillance studies have kindled an intense and still ongoing debate on the role of product class for inhibitor development in hemophilia A, with as yet many unanswered questions and uncertainties (7). **Erik Berntorp** from Lund University in Sweden, a country with the first plasma-derived factor concentrates available in the 1950/1960ies and a high recombinant factor concentrate usage today, provides a well-balanced review of the available data on inhibitor development in previously untreated patients in relation to plasma-derived or recombinant FVIII products (8).

The concluding manuscript of this issue is contributed by **Maria Brehm** who meticulously reviews in detail the current state-of-the-art of von Willebrand factor biosynthesis and its stepwise processing from single monomers to large multimers (9). This paper will help us to better under-

stand the inheritance patterns, the often variable clinical presentation and findings in von Willebrand disease.

Taken together, we trust that this issue of *Hämostaseologie – Progress in Haemostasis* fulfills the GTH core mission: to offer communication of transformative scientific discoveries and to stimulate discussions of their translation into clinical application and practices. As GTH Congress President 2017 or Editor-in-Chief, we are grateful to the authors, the members of the Editorial Board, and the referees for their work and input during peer review.

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