

Basel, Switzerland 9-11 October 2025

#### WORD OF WELCOME

Dear Colleagues and Friends,

This year, the Severe Aplastic Anaemia Working Party Educational Course will be held in Basel, Switzerland, from 9-11 October 2025. It will be a great pleasure to welcome international physicians and experts with a special interest in aplastic anemia, on behalf of SAAWP EBMT.

The conference will feature comprehensive sessions on acquired aplastic anemia, inherited bone marrow failure and paroxysmal nocturnal hemoglobinuria.

We are aiming to share knowledge, foster collaboration and advance our understanding in the field of aplastic anemia, paving the way for the future studies to be conducted by our Working Party.

And this scientific discussion will also give us the opportunity to honor our mentors who have pioneered the field of aplastic anemia.

We look forward to welcoming you to Basel!

Antonio Risitano - Severe Aplastic Anaemia Working Party Chair
Austin Kulasekararaj - Severe Aplastic Anaemia Working Party Secretary
Jakob Passweg - Severe Aplastic Anaemia Working Party Local Organiser
Beatrice Drexler - Severe Aplastic Anaemia Working Party Local Organiser
Kelly Hennessy - Severe Aplastic Anaemia Working Party nurse



09:40 - 10:00

# Severe Aplastic Anaemia Working Party Educational Course

Basel, Switzerland 9-11 October 2025

Thursday, 9 October 2025

13:00 – 14:00	Session I: Pathogenesis of acquired AA	
	Chairs: Edward Gordon-Smith (UK) & Andrea Bacigalupo (IT)	
13:00 - 13:20	From a genetic point of view	Marcin Wlodarski (DE)
13:20 - 13:40	From an immunology point of view	Leo Hansmann (DE)
13:40 – 14:00	HLA and AA: Friends or Foe	Shinji Nakao (JP)
14:00 – 15:30	Session II: 30 years in AA – What have we learned	
	Chairs: Austin Kulasekararaj (UK) & Antonio Risitano (IT)	
14:00 - 14:20	Diagnosis	Alicia Rovó (CH)
14:20 - 14:40	Treatment I: Transplantation	Andrea Bacigalupo (IT)
14:40 - 15:00	Treatment II: IST	Jakob Passweg (CH)
15:00 - 15:20	Long-term outcome and late effects	André Tichelli (CH)
15:20 – 15:30	Discussion: What have learned in 30 years	
15:30 – 16:00	Coffee Break	
16:00 – 16:45	Industry Symposium kindly sponsored by Alexion:	
	Scaling New Heights in PNH: Navigating the Path of Dual Complement Inhibition	
	Chair: Austin Kulasekararaj (UK)	
16:00 - 16:03	Introduction, welcome, faculty introduction and agenda overview	Talha Munir (UK)
16:03 – 16:18	Terminal Complement Inhibition in PNH: A Foundation for Disease Control	Talha Munir (UK)
16:18 – 16:32	The Next Step in Complement Control: Addressing Extravascular Haemolysis in PNH	Austin Kulasekararaj (UK)
16:32 - 16:40	Panel Discussion/Q&A	All
16:40 – 16:45	Key Takeaways and Closing Remarks	Austin Kulasekararaj (UK)
16:45 – 17:30	Session III: Inherited BMFs: HCT Versus gene therapy	
10.45 - 17.50	Chairs: Carlo Dufour (IT) & Constantijn Halkes (NL)	
16:45 – 17:00	Role of HCT in inherited BMFs	Éliane Gluckman (FR)
17:00 – 17:15	Genetic counselling of families (patients and donor!) with inherited BMFs	Fabian Beier (DE)
17:15 – 17:13 17:15 – 17:30	Gene therapy approaches in bone marrow failure syndromes	Senthil Bhoopalan (US)
17.15 - 17.30	Gene therapy approaches in bone marrow failure syndromes	зепініі впоораіан (оз)
20:00 - 22:00	Scientific Business Meeting	
		Friday, 10 October 2025
09:00 - 10:00	Session IV: Complement	
	Chairs: Camilla Frieri (IT) & Jaroslaw Maciejewski (US)	
09:00 - 09:20	Complement and disease	Daniel Ricklin (CH)
09:20 - 09:40	Other complement – associated diseases / Benefits of complement	Patricia Hirt-Minkowski (CH)
	inhibitors in other indications	

Discussion: complement and transplant/more



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10:00 - 11:00 10:00 - 10:20 10:20 - 10:40 10:40 - 11:00	Session V: Daily hurdles in AA Chairs: Olaia Naveiras (CH) & Austin Kulasekararaj (UK) Tapering EPAG – How, when and when to re-start again Work up and treatment of MAA How to distinguish "clonal haematopoiesis" from "transformation" in AA	Beatrice Drexler (CH) Britta Höchsmann (DE) Daria Babushok (US)
11:00 – 11:30	Coffee Break	
11:30 – 12:45	Session VI: Initiative session Chairs: Jakob Passweb (CH) & Andrea Bacigalupo (IT)	
11:30 - 11:55	ICARIIS initiative	Régis P. de la Tour (FR)
11:55 - 12:20	AA treatment decisions/algorithm	Rodrigo Calado (BR)
12:20 – 12:45	Status of art: HCT versus IST (SAAWP initiatives)	Austin Kulasekararaj (UK) Antonio Risitano (IT)
12:45 – 13:30	Lunch Break	
13:30 – 14:15	Industry Symposium kindly sponsored by Novartis:	

This symposium is organized and funded by Novartis and is intended for an audience of non-US HCPs within the context of the EBMT Severe Aplastic Anaemia Working Party Educational Course 2025 on 9-11 October 2025, in Basel, Switzerland. This presentation may include data/information on investigational uses of compounds/drugs that have not yet been approved by regulatory authorities.

Bone Marrow Failure and Complement Dysregulation: Managing Overlapping AA and PNH

**Chair:** Beatrice Drexler (CH)

13:30 - 13:42	Overview of AA and PNH: From Pathophysiology to Practice	Beatrice Drexler (CH)
13:42 - 14:07	Best Practice in Action: Expert Perspectives on AA and PNH Care	
	Interactive case-based discussion	Shinji Nakao (JP)
		Morag Griffin (UK)
14:07 – 14:15	Audience Q&A	All
14:15 – 15:30	Session VII: PNH	
	Chairs: Rodrigo Calado (BR) & Austin Kulasekararaj (UK)	
14:15 – 14:35	Therapeutic algorithm in 2025 for PNH	Morag Griffin (UK)
14:35 - 14:55	HSCT in PNH	Camilla Frieri (IT)
14:55 – 15:15	Future directions in PNH	Régis P. de la Tour (FR)
15:15 – 15:30	Discussion	
15:30 – 16:00	Coffee Break	
16:00 – 17:15	Session VIII: The patients, the nurses and caregivers perspective	
	Chairs: Britta Höchsmann (DE) & Jens Panse (DE)	
16:00 - 16:25	Infectious risk in AA patients	Flore S. de Fontbrune (FR)
16:25 - 16:50	Deciphering fatigue in AA/PNH for future care	Pascale Burmester (DE)
16:50 - 17:15	Transition of AA patients	Kelly Hennessy (UK)



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Saturday, 11 October 2025

09:00 - 10:10 09:00 - 09:20	Session IX: Deciphering acquired versus inherited BMFs in 2025  Chairs: Maurizio Miano (IT) & Alicia Rovó (CH)	Carlo Dufour (IT)
	From the paediatric perspective	
09:20 - 09:40	From the adults perspective	Simone Feurstein (DE)
09:40 - 10:10	New germline kids on the block 2025 and monitoring of iBMFs	Marcin Wlodarski (DE)
10:10 - 10:40	Coffee Break	
10:40 - 11:30	Session X: Long-term outcome	
	Chairs: André Tichelli (CH) & Simone Cesaro (IT)	
10:40 - 10:50	Cutting edge message from the chair	Antonio Risitano (IT)
10:50 - 11:10	Long term outcome in IST versus HCT	Beatrice Drexler (CH)
11:10 – 11:30	How to follow up on QoL in AA?	Jens Panse (DE)
11:30 – 12:00	Session XI: Future Directions	
	Chairs: Beatrice Drexler (CH) & Anna Sureda (ES)	
11:30 - 11:55	New kids on the block (Ruxo, Tregs,)	Shreyans Gandhi (UK)
11:55 - 12:00	Closing remarks	



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