



WORKSHOP

New Technologies in Rare Bleeding Disorders

1-3 November 2024
Helsinki, Finland

PROGRAMME

Friday 1 November 2024		
16:00-18:00	Registrations	
19:00-21:00	Welcome dinner	
Saturday 2 November 2024		
08:45-09:50	Workshop opening <i>Miguel Crato, EHC President</i>	
	08:45-09:05	Icebreaker
	09:05-09:35	An update on novel therapies: a conversation with the EHC Medical Advisory Group (MAG) <ul style="list-style-type: none"> • What is the most interesting scientific advancement in your opinion, since our latests Workshop? • What do you envision will be the most significant advancement during the next 12 months? <i>Maria Elisa Mancuso, Ana Boban, Jan Blatny and Johannes Oldenburg</i>
	09:35-09:50	Q&A

09:50-11:10	Session on replacement therapies <i>Chairs: Riitta Lassila, Olivia Romero Lux</i>	
	09:50-09:55	Welcome to the session
	09:55-10:15	Efanesoctocog in adults: data on prophylaxis and surgery <i>Johannes Oldenburg</i>
	10:15-10:35	Efanesoctocog in children: data on prophylaxis and surgery <i>Manuela Albisetti</i>
	10:35-10:55	Is there still a reason to use SHL and plasma-derived products? <i>Jan Blatny</i>
	10:55-11:10	Q&A
11:10-11:30	Coffee break	
11:30-13:15	Session on non-replacement therapies <i>Chairs: Victor Jimenez-Yuste, Lorenzo Ghirardi</i>	
	11:30-12:00	Emicizumab: "round-to-vial" strategy, no loading dose, low-dose regimen <i>Maria Elisa Mancuso</i>
	12:00-12:30	Concizumab: a new opportunity for haemophilia B with inhibitors? <i>Manuel Carcao</i>
	12:30-13:10	Update on other NFRT <i>Johannes Oldenburg</i>
	13:10-13:15	Q&A
13:15-14:15	Lunch	
14:15-14:30	Group picture	

14:30-16:15	Session on gene and cell therapy <i>Chairs: Radek Kaczmarek, Cedric Hermans</i>	
	14:30-15:00	Update on AAV gene therapy for haemophilia <i>Radek Kaczmarek</i>
	15:00-15:40	Other gene therapies in development <i>Alok Srivastava</i>
	15:40-16:00	Gene editing – Next generation gene therapy <i>Wolfgang Miesbach</i>
	16:00-16:15	Q&A
16:15-16:35	Break	
16:35-18:00	Session on replacement therapy – Real World Evidence <i>Chairs: Cristina Catarino, Panagiotis Christoforou</i>	
	16:35-16:55	RWE on efanesoctocog from the US <i>Steven Pipe</i>
	16:55-17:15	Haemophilia B: refixia for children, what is the current situation? <i>Manuel Carcao</i>
	17:15-17:50	Panel discussion: What is the practice on PUPs and what is the current situation on patients with inhibitors care? <i>Steven Pipe (Chair), Panagiotis Christoforou, Manuela Albisetti, Jan Blatny</i>
	17:50-18:00	Q&A and group discussion
19:30-21:30	Dinner	

Sunday 3 November 2024

09:00-10:15	Von Willebrand Disease <i>Chairs: Augusto Federici, Susan Halimeh</i>	
	09:00-09:20	Diagnosis of VWD <i>Augusto Federici</i>
	09:20-09:40	Prophylaxis in VWD with rVWF and pdVWF <i>Ana Boban</i>
	09:40-10:00	New treatment products for VWD: emicizumab, rondoraptivon <i>Giancarlo Castaman (Presenting online)</i>
	10:00-10:15	Q&A
10:15-10:45	Coffee Break	
10:45-13:00	Other rare bleeding disorders <i>Chairs: Jan Blatny, Admir Mehić</i>	
	10:45-11:05	Emicizumab: off label use for acquired haemophilia and VWD type 3 <i>Cédric Hermans</i>
	11:05-11:35	The therapeutic landscape for RBDs <i>Giancarlo Castaman (Presenting online)</i>
	11:35-12:00	Therapeutic landscape of inherited platelet disorders <i>Carlo Zaninetti</i>
	12:00-12:25	Bleeding disorders of unknown cause (BDUC) <i>Dino Mehic</i>
	12:25-12:40	Q&A and group discussion
12:40-13:00	Closing discussions <i>Miguel Crato, EHC President, Maria Elisa Mancuso, MAG Chair</i>	
13:00-14:00	Lunch and departures	