

EHC New Technologies Workshop in Rare Bleeding Disorders

Draft Programme

3-5 November 2023 – Cascais, Portugal

Friday 3 November 2023		
16.00-18.00 hrs	Registrations	
19.00-21.00 hrs	Welcome dinner	
Saturday 4 November 2023		
08.45-09.30 hrs	Workshop opening <i>Chair TBC</i>	
	08.45-09.05	Icebreaker
	09.05-09.20	An update on novel therapies: A conversation with the NPR Committee
	09.20-09.30	Q&A
09.30-11.10 hrs	Session on replacement therapies <i>Chair TBC</i>	
	09.30-09.35	Welcome to the session
	09.35-09.55	An overview of the next generation of replacement therapies and how to evaluate them <i>TBC</i>
	09.55-10.15	Replacement therapy for VWD - Pros and Cons of a vWF only concentrate Real-world experience <i>TBC</i>
	10.15-10.35	How do we treat platelet function defects? <i>TBC</i>
	10.35-10.55	Prophylactic regimens in women with bleeding disorder? <i>Priyanka Raheja</i>
	10.55 - 11.10	Q&A
11.10-11.30 hrs	Coffee break	

11.30-13.15 hrs	Session on non-replacement therapies -Part 1 Chair TBC	
	11.30-12.00	Products updates: <ul style="list-style-type: none"> - An update on SerpinPC <i>Trevor Baglin (pre-recorded)</i> - An update on fitusiran <i>Alok Srivastava</i> - An update on concizumab <i>Pratima Chowdary</i>
	12.00-12.40	Monoclonal Antibodies- <ul style="list-style-type: none"> ● Women - <i>Cedric Hermans</i> ● VWD - <i>Caterina Casari</i> ● Moderate use – <i>Johannes Oldenburg</i> ● MAb 2.0 – <i>TBC</i>
	12.40-13.00	Haemostasis balance and safety of novel non-replacement therapies <i>Panel discussion</i>
	13.00-13.15	Q&A
13.15-14.15 hrs	Lunch	
14.15-14.30	Group photo	
14.30-16.15	Session on gene and cell therapy Chair TBC	
	14.30-15.00	An update on gene therapy licensing and trials <i>Ilmar Kruis</i>
	15.00-15.40	An update on gene therapy - AAV gene therapies experience: <ul style="list-style-type: none"> - <i>Real World Experience Germany</i> <i>Robert Klamorth (virtual)</i> - <i>Monitoring and dealing with adverse events</i> <i>Wolfgang Miesbach</i> - <i>Non-Viral Vectors</i> <i>TBC</i> - <i>LMIC innovations</i> <i>Alok Srivastava</i>
	15.40-16.00	AAV safety <i>Federico Mingozzi</i>
	16.00-16.15	Q&A
16.15-16.35 hrs	Break	
16.35-18.00	Session on gene therapy – Real World Evidence	

	Chair TBC	
	16.35-16.55	Surgical procedures in patients post-GT <i>Niamh O'Connell</i>
	16.55-17.15	What is the role of liver biopsy in GT post-licensing? <i>Paul Batty</i>
	17.15-17.45	Panel discussion on Gene Therapy: Focus on safety and shared-decision-making <i>Chair TBC</i> <i>Panellists</i> Shagi Galili, Pratima Chowdary
	17.45-18.00	Q&A and group discussions to close the day
19.30-21.30 hrs	Dinner	
Sunday 5 November 2023		
09.00-10.45	Session on non-replacements - Part II Chair TBC	
	09.00-09.15	Update on Bispecific nanobody KB-V13A12 and SynthoPlates <i>Peter Lenting</i>
	09.15-09.30	Update on Star Therapeutics Ab - <i>Suthesh Sivapalaratnam</i>
	09.30-09.45	HMB-001 <i>Catherine Rea</i>
	10.00-10.15	Collecting RWE and creating feedback into research and clinical practice <i>Mike Makris</i>
	10.15-10.45	Q&A
10.45-11.15	Coffee Break	
11.15-13.00	Session on considerations on treatment options Chair TBC	
	11.15-11.35	How do you incorporate shared-decision making into the evaluation and reimbursement process? <i>Brian O'Mahony</i>
	11.35-11.55	Mental health in Gene Therapy <i>TBC</i>

	11.55-12.15	Ethics in the era of GT Lieke Baas
	12.15-12.45	Panel discussion on treatment choice <i>Panelists TBC</i>
	12.45-13.00	Q&A and group discussion for closure
13.00-14.00	Lunch and departures	

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