

EHC New Technology Workshop

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>Miguel Crato, EHC President</i>	
	08.45-09.05	Icebreaker (<i>Lead by Fiona Brennan, EHC</i>)
	09.05-09.20	An update on novel therapies: A conversation with the New Product Review Committee
	09.20-09.30	Q&A
09.30-11.10 hrs	Session on replacement therapies <i>Chair: Ilmar Kruis</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>Sophie Susen - TBC</i>
	09.55-10.15	Replacement therapy for VWD - Pros and Cons of a vWF only concentrate Real-world experience <i>Sophie Susen - TBC</i>
	10.15-10.35	How do we treat platelet function defects? <i>Roseline d'Oiron – 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: Cristina Catarino	
	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>
	12.00-12.50	Monoclonal Antibodies- <ul style="list-style-type: none"> ● Women - <i>Cedric Hermans</i> ● VWD - <i>Caterina Casari</i> ● Moderate use – <i>Johannes Oldenburg - TBC</i> ● Monoclonal antibodies 2.0 - <i>Pratima Chowdary</i>
	12.50-13.10	Haemostasis balance and safety of novel non-replacement therapies <i>Panel discussion</i>
	13.10-13.15	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: Ana Boban	
	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>Glenn Pierce (virtual)</i> - <i>LMIC innovations</i> <i>Alok Srivastava - TBC</i>
	15.40-16.00	AAV safety <i>Federico Mingozzi (virtual)</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: Brian O'Mahony	
	16.35-16.55	Surgical procedures in patients post-GT Wolfgang Miesbach - TBC
	16.55-17.15	What is the role of liver biopsy in GT post-licensing? <i>Paul Batty</i>
	17.15-17.25	Novel approaches for viral vector delivery – CRISPR-cas9 David Gutstein
	17.25-17.55	Panel discussion on Gene Therapy: Focus on safety and shared-decision-making Chair BOM Panellists Shagi Galili, Pratima Chowdary, Johannes Oldenburg (TBC) & Miguel Crato (TBC)
	17.55-18.00	Q&A and group discussions to close the day
19.30-21.30 hrs	Dinner	
Sunday 5 November 2023		
09.00-10.15	Session on non-replacements - Part II Chair: Mariëtte Driessens	
	09.00-09.15	Update on Bispecific nanobody KB-V13A12 and SynthoPlates <i>Caterina Casari</i>
	09.15-09.30	Update on Star Therapeutics Ab - <i>Suthesh Sivapalaratnam</i>
	09.30-09.45	HMB-001 <i>Catherine Rea (virtual)</i>
	09.45-10.00	Collecting RWE and creating feedback into research and clinical practice <i>Mike Makris</i>
	10.00-10.15	Q&A
10.15-10.45	Coffee Break	
10.45-13.00	Session on considerations on treatment options Chair: Maria Elisa Mancuso	
	10.45-11.05	How do you incorporate shared-decision making into the evaluation and reimbursement process? <i>Brian O'Mahony</i>

	11.05-11.25	Mental health in Gene Therapy <i>Simon Fletcher</i>
	11.25-11.55	Ethics in the era of GT Lieke Baas
	11.55-12.25	Panel discussion on treatment choice Brian O'Mahony, Olivia Romero Lux & <i>Jan Blatny</i>
	12.25-12.40	Q&A and group discussion for closure
12.40-13.00		Closing discussions Miguel Crato, EHC President + NPR Committee members + MAG members
13.00-14.00		Lunch and departures

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