



# WORKSHOP

## New Technologies in Rare Bleeding Disorders

1-3 November 2024

Helsinki, Finland

### DRAFT 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	
	08:45-09:05	Icebreaker
	09:05-09:35	An update on novel therapies: A conversation with the EHC Medical Advisory Group
	09:35-09:50	Q&A
09:50-11:10	Session on replacement therapies	
	09:50-09:55	Welcome to the session

	<b>09:55-10:15</b>	<b>Efanesoctocog in adults: Data on prophylaxis and surgery</b>
	<b>10:15-10:35</b>	<b>Efanesoctocog in children: Data on prophylaxis and surgery</b>
	<b>10:35-10:55</b>	<b>Is there still a reason to use SHL and plasma-derived products?</b>
	<b>10:55-11:10</b>	<b>Q&amp;A</b>
<b>11:10-11:30</b>	<b>Coffee break</b>	
<b>11:30-13:15</b>	<b>Session on non-replacement therapies</b>	
	<b>11:30-12:00</b>	<b>Emicizumab: "Round-to-vial" strategy, no loading dose, low-dose regimen</b>
	<b>12:00-12:30</b>	<b>Concizumab: RWE from Canada</b>
	<b>12:30-13:10</b>	<b>Update on other NFRT</b>
	<b>13:10-13:15</b>	<b>Q&amp;A</b>
<b>13:15-14:15</b>	<b>Lunch</b>	
<b>14:15-14:30</b>	<b>Group picture</b>	
<b>14:30-16:15</b>	<b>Session on gene and cell therapy</b>	
	<b>14:30-15:00</b>	<b>Update on AAV gene therapy for haemophilia</b>
	<b>15:00-15:40</b>	<b>Other gene therapies in development</b>
	<b>15:40-16:00</b>	<b>Gene editing – Next generation gene therapy</b>
	<b>16:00-16:15</b>	<b>Q&amp;A</b>

<b>16:15-16:35</b>	<b>Break</b>	
<b>16:35-18:00</b>	<b>Session on replacement therapy – Real World Evidence</b>	
	<b>16:35-16:55</b>	<b>RWE on efanesoctocog from the US</b>
	<b>16:55-17:15</b>	<b>Haemophilia B: Refixia for children, what is the current situation?</b>
	<b>17:15-17:50</b>	<b>Panel discussion: What is the practice on PUPs and what is the current situation on patients with inhibitors care?</b>
	<b>17:50-18:00</b>	<b>Q&amp;A and group discussion</b>
<b>19:30-21:30</b>	<b>Dinner</b>	
<b>Sunday 3 November 2024</b>		
<b>09:00-10:15</b>	<b>Von Willebrand Disease</b>	
	<b>09:00-09:20</b>	<b>Diagnosis of VWD</b>
	<b>09:20-09:40</b>	<b>Prophylaxis in VWD with rVWF and pdVWF</b>
	<b>09:40-10:00</b>	<b>New treatment products for VWD: emicizumab, rondoraptivon</b>
	<b>10:00-10:15</b>	<b>Q&amp;A</b>
<b>10:15-10:45</b>	<b>Coffee Break</b>	
<b>10:45-13:00</b>	<b>Other rare bleeding disorders</b>	
	<b>10:45-11:05</b>	<b>Emicizumab: off label use for acquired haemophilia and VWD type 3</b>

	<b>11:05-11:35</b>	<b>The therapeutic landscape for RBDs</b>
	<b>11:35-12:00</b>	<b>Glanzmann Disease and other platelet disorders: Therapeutic landscape</b>
	<b>12:00-12:25</b>	<b>Bleeding disorders of unknown cause (BDUC)</b>
	<b>12:25-12:40</b>	<b>Q&amp;A and group discussion</b>
<b>12:40-13:00</b>	<b>Closing discussions</b>	
<b>13:00-14:00</b>	<b>Lunch and departures</b>	