EHC Workshop on New Technologies in Haemophilia Care

22-24 November 2019

Athens, Greece

DRAFT PROGRAMME

22 Nov	23 Nov	24 Nov
	09.00 – 09.05 **Welcome and introduction to the workshop**	**09.00 – 09.05** **Welcome and introduction**
	Licensed therapies	**Gene Therapy**
	09.05 – 09.35 **Hemlibra: Overview of real-world evidence in inhibitor patients – *What have we achieved and what have we lost?***	**09.05 – 09.35** **Current state of play with clinical trials in gene therapy**
	09.35 – 10.05 **Hemlibra: Overview of real-world evidence in non-inhibitor patients**	**09.35 – 10.05** **Re-treatment with vector for gene therapy**
	10.05 – 10.25 **Q&A**	**10.05 – 10.25** **Q&A**
	10.25 – 10.45 *Coffee break*	*10.25 – 10.40* *Coffee break*
	10.45 – 11.15 **Extended half-life: Real-world evidence for efficacy (trough)**	**10.40 – 11.10** **Unanswered questions with adeno-associated virus**
	11.15 – 11.45 **Extended half-life: Safety of PEG**	**11.10 – 11.50** **Regulatory & Patient Perspectives: Future ethical considerations of enrolling children in gene therapy**
	11.45 – 12.00 **Q&A**	**11.50 – 12.20** **How will we pay for gene therapy in Europe**
	12.00 – 12.20 *Coffee break (coffee only)*	*12.20 – 12.35* *Coffee Break (coffee only)*
	12.20 – 12.50 **Update on Vonvendi: Indications for use how use rVWF in surgery**	**Panel discussions**
	12.50 – 13.00 **Q&A**	**12.35 – 13.20** **Which patients should be prioritised for gene therapy – Views from clinicians, payers and patients**
		13.20 – 13.30 **Conclusions and evaluations**
	13.00-14.00 *Lunch*	*13.30 - 14.30* *Lunch and departures*

This workshop was made possible thanks to the sponsorship of BioMarin

16.00-18.00 – Registration	**14.00 – 14.30 Novel therapies: Access and affordability in Europe** **14.30 – 15.00 Novel therapies: Safety and ongoing post-marketing trials** **15.00 – 15.20 Q&A** 15.20 – 15.40 Coffee Break (coffee only) **Therapies in Clinical Trials** **15.40 – 16.10 Update on SerpinSC** **16.10 – 16.40 Update on anti-TFPI** *16.40 – 17.00 Q&A* 17.00 – 17.20 Coffee Break **Panel discussion** **17.20 – 18.00 With standard half-life, extended half-life, Hemlibra and gene therapy available or on the way: How will we chose which therapies to choose for which patients and how will we compare affordability?**	
19.00-21.00 – Dinner	*19.00 – 20.00 Social activity* *20.00-22.00 – Workshop Dinner*	

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