

EHC New Technologies Workshop

11-13 November 2022

Marseilles, France

Draft programme

| Friday 11 November | | |
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| 16.00-18.00 hrs | Registrations | |
| 19.30-21.30 hrs | Welcome dinner | |
| Saturday 12 November Focus on the clinical Update | | |
| 09.00-09.20 | Workshop introduction Chair: TBC | |
| | 09.00-09.05 | Welcome and introduction <i>Chair TBC</i> |
| | 09.05-09.20 | An overview of the treatment landscape for haemophilia and other rare bleeding disorders and its challenges <i>Mike Makris</i> |
| 09.30-10.50 | Session on replacement therapies Chair TBC | |
| | 09.30-09.35 | Welcome and introduction <i>Chair TBC</i> |
| | 09.35-09.55 | Do we need to redefine FVIII EHL in an era of BIVV001? <i>Robert Klamroth (virtual or pre-recorded TBC)</i> |
| | 09.55-10.15 | An update on replacement therapies in VWD and RBDs <i>Frank Leebeek (virtual)</i> |
| | 10.15-10.35 | FIX in the extravascular space: How to compare FIX products? <i>Cedric Hermans</i> |
| | 10.35-10.50 | Q&A and session closing |
| 10.50-11.20 | Coffee break | |

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| 11.20-12.40 | Session on non-replacement therapies Chair TBC | |
| | 11.20-11.25 | Welcome and introduction Chair TBC |
| | 11.25-12.00 | An update on Hemlibra and other bispecific antibodies (MIM8) <i>Roberta Gualtierotti (TBC)</i> |
| | 12.00-12.30 | An update on novel non-replacement therapies <ul style="list-style-type: none"> • An update on fitusiran <i>Savita Rangarajan (virtual)</i> • An update on concizumab <i>Allison Wheeler (virtual)</i> • An update on marstacimab <i>Johnny Mahlangu (Virtual)</i> |
| | 12.30-12.45 | Can we measure the correction of the coagulopathy in haemophilia for different products? <i>Annette Bowyer</i> |
| | 12.45-13.00 | Q&A and closing |
| 13.00-13.30 | Interview/Panel/Recap on RTs - PEGs, Immunogenicities and assay discrepancies NRTs - Thrombotic risks, biomarker assessment and immunogenicity Chair TBC Panellists TBC | |
| 13.30-14.40 | Lunch | |
| 14.40-16.20 | Session on gene therapy Chair TBC | |
| | 14.40-14.45 | Welcome and introduction Chair TBC |
| | 14.45-15.15 | An update on gene therapy in haemophilia A and B <i>Michiel Coppens</i> |
| | 15.15-15.35 | Gene therapy integration and long-term safety <i>Paul Batty</i> |
| | 15.35-15.45 | Q&A |
| 15.45-16.00 | Break | |

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| | 16.00-16.30 | Where will gene therapy (AAV and non-viral) be in 2030? <i>Glenn Pierce</i> |
| | 16.30-16.50 | Collecting qualitative data for GT <i>Barbara Konkle</i> |
| | 16.50-17.00 | Q&A |
| 17.00-17.15 | Break | |
| 17.15-17.45 | Interview/Panel/Recap on Is GT ready for the market? How do we deal with known and unknown unknowns? GT - variability, durability, immune response, liver toxicity and integration | |
| Sunday 13 November Focus on the rare bleeding disorders, organisation of care, financial aspects and SDM | | |
| 09.00-10.15 | Session on rare bleeding disorders Chair TBC | |
| | 09.00-09.05 | Welcome and introduction |
| | 09.05-09.25 | Thinking of novel clinical trials designs for rare bleeding disorders <i>Mariëtte Driessens</i> |
| | 09.25-09.45 | Use of bi-specific antibodies in the treatment of Glanzmann Thrombasthenia <i>Roger Schutgens</i> |
| | 09.45-10.00 | How can aptamers and other antibodies work for other rare bleeding disorders? <i>Radek Kaczmarek</i> |
| | 10.00-10.10 | Q&A |
| 10.10-10.25 | Break | |
| 10.25-11.40 | Session on organisation of care and shared decision-making Chair TBC | |
| | 10.25-10.30 | Welcome and intro Chair TBC |
| | 10.30-10.50 | Hub and Spoke: What does it mean for the gene therapy patient? <i>Flora Peyvandi</i> |

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| | 10.50-11.10 | Hub and Spoke: Beyond gene therapy <i>Speaker (TBC)</i> |
| | 11.10-11.30 | Is the Hub & Spoke model a risk to long-term care for patients with a bleeding disorder? <i>The motion for yes: Roger Schutgens</i> <i>The motion for no: Mike Makris</i> |
| | 11.30-11.40 | Q&A |
| 11.40-12.00 | Break | |
| 12.00-13.05 | Session on financial considerations Chair TBC | |
| | 12.00-12.05 | Welcome and intro Chair TBC |
| | 12.05-12.25 | Paying for uncertainty <i>Simone Boselli (TBC)</i> |
| | 12.25-12.45 | Using value frameworks <i>Brian O'Mahony</i> |
| | 12.45-13.05 | How different procurement approaches impact cost-effectiveness? <i>Renske ten Ham (virtual or on-site TBC)</i> |
| 13.05-13.25 | Panel discussions - lessons learned from the weekend and action points for the EHC Chair and panellists TBC | |
| 13.25-13.30 | Closure and take-aways | |