



NOVEMBER 17

8:30-8:40	Welcome	
8:40-9:00	Gene therapy update	- Gene therapy update - <i>Radoslaw Kaczmarek</i>
9:00-10:30	Science by press release	- How science is being communicated - <i>Timothy Caulfield</i> - Science by press release for hemophilia - <i>Mike Makris</i> - Editor perspective - importance of the peer review process, balanced data, and evidence - <i>Cedric Hermans</i>
10:30-10:45	Break	
10:45-12:00	Safety	- Factor 8 - <i>Johnny Mahlangu</i> - Factor 9 - <i>Wolfgang Miesbach</i> - Liver issues - <i>Sylvia Fong</i>
12:00-13:00	Lunch	
13:00-14:15	Variability & durability	- Factor 8 - <i>Margareth Castro Ozelo</i> - Factor 9 - <i>Pratima Chowdary</i> - WFH Gene Therapy Registry - <i>Barbara Konkle</i>
14:15-14:30	Break	
14:30-16:00	Regulatory perspective	- REMS request in the USA - <i>Len Valentino</i> - FDA perspective - <i>Peter Marks</i> - EMA perspective - <i>Andrea Laslop</i>
16:00-17:15	Informed consent & shared decision making	- Issues to consider for informed consent - HCP perspective - <i>Lindsey George</i> - Issues to consider for informed consent - PWH perspective - <i>Declan Noone</i> - WFH shared decision making tool - <i>Courtney Thornburg</i> - How regulators look at decisions making for gene therapy - <i>Martina Schuessler-Lenz</i>
19:00-21:30	Dinner	

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8:00-9:30	Expanding eligibility	- Eligibility for gene therapy - current limitations - <i>Alok Srivastava</i> - Gene therapy in China: efficacy, safety data, and future plans - <i>Lei Zhang</i> - Approaches to overcoming immune challenges - <i>Gallia Levy</i> - Clinical trials of inhibitors (of history of) - <i>Tara Robinson</i>
9:30-9:45	Break	
9:45-11:00	Alternatives to AAV	- Lentivirus and ET3 - <i>Chris Doering</i> - Gene editing - <i>Leah Sabin</i> - Non-viral gene therapy - <i>Jack Rychak</i>
11:00-12:00	Pricing gene therapy	- Dealing with uncertainty - <i>Anja Schiel</i> - Impact of different payment methods on price/health care system - <i>Lesley Tilson</i> - Outcomes and monitoring in the context of payment - <i>Kollet Koulianos</i>
12:00-12:45	Lunch	
12:45-13:50	Plans for commercialized gene therapy in HMIC	- Perspective from Argentina - <i>Daniela Neme</i> - Perspective from Brazil - <i>Margareth Castro Ozelo</i> - Perspective from South Africa - <i>Johnny Mahlangu</i> - Perspective from Turkey - <i>Kaan Kavakli</i> - Differences between LMIC & HMIC - <i>Bishesh Poudyal</i>
13:50-14:00	Closing remarks	

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