

Forum PBC Meeting

Monday, April 7, 2025
Delta Hotels Edinburgh
111 Glasgow Rd
Edinburgh EH12 8NF, United Kingdom

AGENDA

11:00 AM	Registration and Light Lunch	
12:00 PM	Session I: Ethics and Equipoise in PBC Clinical Trials	
12:00 PM	Moderators: Veronica Miller, Forum & Robert-Mitchell Thain, PBC Foundation	
12:00 PM	Introductory Remarks	<i>Veronica Miller, Forum Robert-Mitchell Thain, PBC Foundation</i>
12:10 PM	Evidence, Equipoise and Methodological Absolutism	<i>Søren Holm, Manchester University</i>
12:20 PM	Ethics and Equipoise in PBC Clinical Trials	<i>Keith Berelowitz, patientRx</i>
12:30 PM	Evidence and Reasons in Clinical Trial Design and Regulatory Approval	<i>Mark Sheehan, Oxford University</i>
12:40 PM	Panel Discussion	<i>Marco Carbone, University of Milano-Bicocca Mo Christie, PBC Foundation</i>
1:10 PM	Session II: Scientific Validity and Statistical Challenges	
1:10 PM	Moderators: Benjamin Miller, Ipsen & Veronica Miller, Forum	
1:10 PM	When the Going Gets Tough: Emulating a Control Arm in PBC When a Randomized Trial is No Longer Feasible	<i>Bettina Hansen, Erasmus University</i>
1:25 PM	Achieving Robust Evidence while Minimising Expected Control Group Size - An Bayesian Adaptive Trials Perspective	<i>Sofia Villar, MRC Biostatistics Unit at University of Cambridge</i>
1:40 PM	Efficient Bayesian Model-based Approaches to Reduce Placebo Burden in Rare Disease Trials	<i>Satrajit Roychoudhury, Pfizer*</i>
1:55 PM	Panel Discussion	<i>Ingo van Thiel, Deutsche Leberhilfe eV Bettina Hansen, Erasmus University Sofia Villar, MRC Biostatistics Unit at University of Cambridge Alejandra Villamil, Hospital Italiano de Buenos Aires Satrajit Roychoudhury, Pfizer*</i>
2:25 PM	Break	
2:55 PM	Session III: Risk Stratification in PBC Clinical Trials	
2:55 PM	Moderators: Veronica Miller, Forum & Alejandra Villamil, Hospital Italiano de Buenos Aires	
2:55 PM	Biliary Cirrhosis in PBC is Irreversible: Mandate for Early Stage Detection and Treatment	<i>John Vierling, Baylor College of Medicine</i>
3:10 PM	What is Risk to the Patient?	<i>Carol Roberts, PBCers*</i>
3:25 PM	Risk Stratification - Industry Panel	<i>Steve Gao, Gilead Barry Crittenden, Gilead* Benjamin Miller, Ipsen Paul Nitschmann, Intercept Pharmaceuticals Pam Vig, Mirum</i>
3:45 PM	Discussion	<i>Danielle Alstat, Life with PBC: Primary Biliary Cholangitis Support Group David Jones, Newcastle University</i>

4:15 PM	Session IV: 21st Century Solutions to 21st Century Problems	
4:15 PM	Moderators: Veronica Miller, Forum & Pam Vig, Mirum	
4:15 PM	Changing the Future: Moving Towards Solutions	<i>Robert-Mitchell Thain, PBC Foundation</i>
4:30 PM	EASL and the PBC journey	<i>Ana Lleo, Humanitas University</i>
4:45 PM	Closing Discussion	<i>All Participants</i>
5:15 PM	Adjourn & Networking Reception	
	*indicates remote participation	