

FOURTH INTERNATIONAL HOMOCYSTINURIAS PATIENT EXPERT VIRTUAL MEETING PROGRAM

SESSION 1	PRESENTER	AEDT	CET	EST
Opening	Tara Morrison	Tues 30 Nov 5.00pm	Tues 30 Nov 7.00am	Tues 30 Nov 1.00am
Classical Homocystinuria				
Tips to stay on diet for classical homocystinuria	Brooke Pinsent	Tues 30 Nov 5.05pm	Tues 30 Nov 7.05am	Tues 30 Nov 1.05am
Nutricia Metabolics continued support of the HCU community	Amira Ramadan Renee Wells	Tues 30 Nov 5.35pm	Tues 30 Nov 7.35am	Tues 30 Nov 1.35am
Observations from newborn screening for homocysteine in NSW	Kaustuv Bhattacharya	Tues 30 Nov 6.00pm	Tues 30 Nov 8.00am	Tues 30 Nov 2.00am
Homocystinuria and bones	Michel Tchan	Tues 30 Nov 6.30pm	Tues 30 Nov 8.30am	Tues 30 Nov 2.30am
Break & Networking		Tues 30 Nov 7.00pm	Tues 30 Nov 9.00am	Tues 30 Nov 3.00am
Research Updates				
The EHOD project: updates on guidelines and research	Martina Huemer	Tues 30 Nov 7.30pm	Tues 30 Nov 9.30am	Tues 30 Nov 3.30am
MTHFR deficiency: influence of early initiation of therapy on long-term outcome	Mathilde Yverneau	Tues 30 Nov 8.00pm	Tues 30 Nov 10.00am	Tues 30 Nov 4.00am
Homocystinuria patient and caregiver survey: experiences of diagnosis and patient satisfaction	Andrew Morris	Tues 30 Nov 8.30pm	Tues 30 Nov 10.30am	Tues 30 Nov 4.30am
B12 dose escalation in cblC deficiency: experience from a US natural history study	Irini Manoli	Tues 30 Nov 9.00pm	Tues 30 Nov 11.00am	Tues 30 Nov 5.00am
Clinical presentation and treatment practice in patients with CBS deficiency: update on E-HOD registry analysis	Viktor Kozich	Tues 30 Nov 9.30pm	Tues 30 Nov 11.30am	Tues 30 Nov 5.30am
Close		Tues 30 Nov 10.00pm	Tues 30 Nov 12.00pm	Tues 30 Nov 6.00am

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SESSION 2	PRESENTER	AEDT	CET	EST
Panel Discussions				
Panel Q & A CblC and Severe MTHFR Deficiency Moderator: Kaustuv Bhattacharya	Matthias Baumgartner Martina Huemer Irina Manoli David Rosenblatt	Wed 1 Dec 7.00am	Tues 30 Nov 9.00pm	Tues 30 Nov 3.00pm
Panel Q & A Classical homocystinuria Moderator: Kaustuv Bhattacharya	Andrew Morris Viktor Kozich Harvey Levy	Wed 1 Dec 7.45am	Tues 30 Nov 9.45pm	Tues 30 Nov 3.45pm
Research announcement	Karolina Stepien	Wed 1 Dec 8.30am	Tues 30 Nov 10.30pm	Tues 30 Nov 4.30pm
Break & Networking		Wed 1 Dec 8.40am	Tues 30 Nov 10.40pm	Tues 30 Nov 4.40pm
Novel Treatments Progress				
Clinical Trial Recruitment: COVID , challenges & where we are today	Mark Bechter	Wed 1 Dec 9.00am	Tues 30 Nov 11.00pm	Tues 30 Nov 5.00pm
Discovery of CDX-6512, a GI-stable methionine-gamma-lyase as a potential orally-administered enzyme therapy for homocystinuria	Kristen Skvorak	Wed 1 Dec 9.30am	Tues 30 Nov 11.30pm	Tues 30 Nov 5.30pm
Natural history studies and why they are needed in rare disease drug development	Jalé Güner Sagar A. Vaidya	Wed 1 Dec 10.00am	Tues 30 Nov 12.00am	Tues 30 Nov 6.00pm
Strategies to correct CBS deficiency: studies in mice	Warren Kruger	Wed 1 Dec 10.30am	Tues 30 Nov 12.30am	Tues 30 Nov 6.30pm
SYNB1353: An Investigational Methionine-consuming Synthetic Biotic Medicine for the Treatment of Homocystinuria	Mylène Perreault	Wed 1 Dec 11.00am	Tues 30 Nov 1.00am	Tues 30 Nov 7.00pm
Break & Networking		Wed 1 Dec 11.10am	Tues 30 Nov 1.10am	Tues 30 Nov 7.10pm
HCU Network America & HCU Network Australia Research Grant Awardees				
Evaluation of benefits of thiol-based reductants in classical homocystinuria	Tomas Majtan	Wed 1 Dec 11.30am	Tues 30 Nov 1.30am	Tues 30 Nov 7.30pm
Altered folate metabolism in HCU: opportunities for improved therapy	Ken Maclean	Wed 1 Dec 12.00pm	Tues 30 Nov 2.00am	Tues 30 Nov 8.00pm
Patient Organisation and Collaboration Updates				
RARE-X: Homocystinuria patient's data powering progress	Vanessa Vogel-Farley	Wed 1 Dec 12.30pm	Tues 30 Nov 2.30am	Tues 30 Nov 8.30pm
HCU Network America: Advocating for improved NBS	Margie McGlynn	Wed 1 Dec 1.00pm	Tues 30 Nov 3.00am	Tues 30 Nov 9.00pm
Acknowledgements & Close	Tara Morrison	Wed 1 Dec 1.30pm	Tues 30 Nov 3.30am	Tues 30 Nov 9.30pm