



GARROD SYMPOSIUM

PRECISION MEDICINE IN IEMs



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Target Audience

Physicians, Laboratorians, Researchers, Registered Nurses, Genetic Counsellors, Registered Dietitians, Clinical Specialists, Laboratory Technicians, and Trainees.

Learning Objectives

By the end of this activity, participants will be able to:

- Discuss indigenous & immigrant patient experiences from an equity, diversity and inclusion perspective (allied health conference)
- Recognize current progress, new methods, and approaches to treating inherited metabolic diseases.
- Discuss the ethical and economic issues around advanced therapy medicinal products, particularly within the context of a publicly-funded healthcare system.
- Identify how the latest technologies are being applied to diagnosing and therapeutic monitoring of inherited metabolic diseases.
- Evaluate how advances in precision medicine are influencing the further expansion of newborn screening.
- Recognize the importance of translational research in advancing our understanding of, and our ability to improve the lives of patients with inherited metabolic diseases.

Please note: 25% of the scientific program will be interactive.

SHERATON SUITES CALGARY EAU CLAIRE

May 12–14, 2022

Thursday, May 12, 2022

Sub Meeting Agenda

ALLIED HEALTH CONFERENCE – LOOKING WITHIN (Wildrose Room Centre & South)

- 12:00–1:00 Registration Opens
(Main Foyer)
- 1:00–1:05 Welcome and Spiritual Message
Cree Elder Doreen Spence
- 1:00–1:10 Introduction of Panel

Session Chairs: Karen Sabo, Catherine Rombough

- 1:15–1:30 Indigenous Cultural Diversity in Health Care
Cree Elder Doreen Spence
- 1:30–1:45 Video Vignette – Patient Experiences
Immigrants Coping with IEM Diagnosis in
Community Practice
Thiru Govender
- 1:45–2:00 Video Vignette – Patient Experiences
Role of Social Worker in Inherited Metabolic
Diseases Clinic
Francine Abma-Vink
- 2:00–2:15 Pharmacogenetics – An Overview
Christy Gilkes
- 2:15–2:35 Questions / Panel Discussion
- 2:35–2:40 Wrap Up
- 2:40–3:00 Break
- 3:00–4:00 **Allied Health Breakout Sessions:**
- Dietitians Workshop
(Room: Wolf Willow)
- RN Workshop – Round Table Talk
(Room: Silver Willow)
- 3:00–4:00 Canadian MPS Society Meeting
(Calypso Room)

Thursday, May 12, 2022

Symposium Agenda

- 12:00–4:30 Registration
(Main Foyer)
- 4:30–4:45 Welcome to Garrod Symposium
(Wildrose Room Centre & South)
David Sinasac, PhD, FCCMG
Chair of Garrod Symposium
- Andreas Schulze, MD, PhD, FRCPC**
President of Garrod Association
- 4:45–5:30 Opening Talk
- Session Chair: Jagdeep Walia**
- Approaches to Gene Therapy in IEMs
Steven Gray, PhD
- Platform Presentations**
- Session Chair: Christy Gilkes**
- 5:30–5:45 Late-onset ornithine transcarbamylase
deficiency: biochemical, clinical, and
functional consequences of a rare
regulatory region variant
Samuel Tholl, Chitra Prasad
- 5:45–6:00 Long term follow-up and treatment outcome
of primary biotin deficiencies.
Vancouver experience
Gabriella Horvath
- 6:00–6:15 Efficacy and safety of the recommended
pegvaliase dosing regimen in adults with
phenylketonuria in the phase 3 PRISM
studies
**Kristin Lindstrom, Stephanie Sacharow,
Hope Northrup, Kaleigh Whitehall,
Richard Rowell, Barbara Burton,
Janet Thomas**
- 6:30–8:00 Welcome Reception
(Main Foyer)
- 7:30–9:30 Garrod Board of Directors
(Calypso Room)

Friday, May 13, 2022

Symposium Agenda

7:00–8:00 AM Registration & Breakfast ([Main Foyer](#))

Plenary Session I - Gene Therapy Trials - Current Approaches & Progress

Session Chair: Matthew Lines

- 8:00–8:45 Cisterna Magna delivery
Heather Leigh Gray-Edwards, DVM, PhD
- 8:45–9:15 Pre-clinical gene therapy trials?
Japdeep Walia, MBBS, FRCPC, FCCMG
- 9:15–9:45 Gene Therapy for GSD Ia
John Mitchell, MD
- 9:45–10:15 Refreshment Break and Exhibits ([Main Foyer](#))

Plenary Session II - Ethics & Economics of ATMP

Session Chair: David Sinasac

10:15–11:30 Panel Discussion

Moderator: Lorian Hardcastle, JD, LLM, SJD (doctorate)

Panelists: Cheryl Rockman-Greenberg, MD, CM, FRCPC, FCCMG, Bartha Maria Knoppers, PhD, Larry Lynd, PhD, BSP

Platform Presentations

Session Chair: Christy Gilkes

- 11:30–11:45 Elucidating the role sphingolipids in the pathogenesis of hepatocellular adenoma linked to GSD Ia
Jae Yeon Park, Dwight Koeberl, Farah ElTurk, John Mitchell
- 11:45–12:00 PM Effect of Creatine Supplementation on AGAT Expression and Metabolic Intermediates in GAMT-Deficient mice
Ilona Tkachyova, Madeleine Hall, Joshua Atienza, Haneen Ali, Jens Feugman, Nico Guischard, Andreas Schulze
- 12:00–12:15 Triheptanoin for the Treatment of Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): Final Results of an Open-Label, Long-Term Extension Study
Jerry Vockley, Barbara Burton, Gerard Berry, Nicola Longo, John Phillips, Amarilis Sanchez-Valle, Kimberly Chapman, Pranoot Tanpaiboon, Stephanie Grunewald, Elaine Murphy, Xiaoxiao Lu, Syeda Rahman, Kathryn Ray, Antonio Nino Ramirez
- 12:15–1:15 Lunch ([Main Foyer](#))
- 1:15–1:45 Dessert and Exhibits ([Main Foyer](#))

Plenary Session III - Novel Approaches to Therapy

Session Chair: Florin Sasarman

- 1:45–2:30 Genetic Approaches to treat mtDNA Diseases
Carlos T. Moraes, PhD
- 2:30–3:00 Lipid Nanoparticle – mRNA (LNP-mRNA) therapies for inborn errors of metabolism
Aneal Khan, MSc, MD, FRCPC, FCCMG
- 3:00–3:30 Epigenetic mechanisms in the cblC inborn error of vitamin B12 metabolism
David Rosenblatt, MD
- 3:30–4:00 In-utero enzyme replacement therapy for a fetus with Infantile Onset Pompe Disease (IOPD)
Pranesh Chakraborty, MD, FRCPC
- 4:00–4:30 Refreshment Break and Exhibits ([Main Foyer](#))
- 4:30–6:00 Poster Walk & Light Refreshments ([Wildrose Room North](#))

Friday Gala Dinner transportation

- 6:15 / 6:45 Bus Leaves for Bell Music Centre (or walk ~25 minutes)
[See below for details about the scheduled tours](#)
- 6:45 Doors Open @ Bell Music Centre
- 7:00 Tours begin @ Bell Music Centre
- There will be two half-hour tours, each consisting of two groups of 25 people, at 7:00 and 7:30. Check your tickets after receiving them from the registration desk to determine which tour time you have been assigned. Buses for each timed tour are indicated above. Hors d'oeuvres and a bar set up outside of the performance hall will also be available prior to the start of the dinner.
- 8:20 Gala Dinner Introductions - Dinner beings
- 8:40 Announcement of Garrod Grant, Presentation and Poster Awardees
- 9:30 Pioneer Award
- 10:00 Entertainment
- 11:00 PM Close - Bus back to hotel

Saturday, May 14, 2022

Symposium Agenda

7:00–8:00 Breakfast
(Main Foyer)

Plenary Session IV – New Approaches for Diagnosis & Treatment Monitoring Session Chair: Aneal Khan

8:00–8:45 Normal newborn screen?
Clinical untargeted metabolomics as a functional screen to improve genomic analysis
Sarah Elsea, PhD., FACMG

8:45–9:15 Development of a biobank and untargeted metabolomics pipeline aimed at improving diagnosis, informing treatment and probing pathophysiology of IMDs
Alicia DiBattista, PhD

9:15–9:45 Flux Analysis in DCMA fibroblasts
Steven Greenway, MSc, MD, FRCPC

9:45–10:15 Refreshment Break and Exhibits
(Main Foyer)

Plenary Session V – Precision Medicine: NBS Session Chair: Ross Ridsdale

10:15–10:45 Newborn Screening for MPS-1 Hurler
Matthew Henderson, PhD, FCACB, FCCMG

10:45–11:15 NBS for SMA
Kirstin Kernohan, PhD, FCCMG

11:15–12:00 Closing Talk

Session Chair: David Sinasac

Translational Research in Inborn Errors of Metabolism: From Bench to Bedside
Lindsey Burrage, MD, PhD

12:00–12:30 Meeting Update and Closing Remarks

12:30–1:30 Lunch (provided)
(Main Foyer)

1:00–3:00 Garrod Association Membership Meeting
All Members Welcome
(Wildrose Room Centre & South)



DOWNLOAD THE PRESENTATIONS

garrodsymposium.ca

Password: garrod22

*Presentations will be available if the speaker has provided consent.

DISCLOSURE OF CONFLICTS OF INTEREST

In keeping with accreditation requirements and the National Standard for Support, McMaster University, Continuing Health Sciences Education Program requires that all speakers, planning committee members, moderators, facilitators and authors participating in this activity must disclose all relationships with for-profit and not-for-profit organizations over the previous two years. Disclosures must be done in print, verbally, and in writing on a slide prior to the speaker's presentation.

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DISCLOSURES OF POTENTIAL CONFLICTS OF INTEREST

The following Planning Committee Members and Speakers DID NOT declare a relationship with a FOR-PROFIT and/or NOT-FOR-PROFIT organization over the previous two years:

Christy Gilkes
Steven Greenway
Lorian Hardcastle
Matthew Henderson
Kristin Kernohan

Bartha Maria Knoppers
Matthew Lines
Larry Lynd
Carlos Moraes
Catherine Rombough

Karen Sabo
Florin Sasarman
David Sinasac
Paula Waters

The following Planning Committee Members and Speakers HAVE OR HAD a relationship with a FOR-PROFIT and/or NOT-FOR-PROFIT organization over the previous two years:

Lindsay Burrage: Member of Scientific Advisory Boards for MSUD Family Support Group. Received research grants from National Institutes of Health, Burroughs Wellcome Fund.

Pranesh Chakraborty: Speaking Honoraria from Novartis. Participated in advisory boards with Sanofi, Ultragenyx and Waters. Received grants from Biomarin, Takeda, Biogen, Cambrooke, Vitaflor, Nutricia, NFDC, CIHR, CF Foundation.

Alicia DiBattista: Is CTO - Neurolytxs Inc.

Sarah Elsea: Consultant with Dicerna, Enzyvant. Chair, Professional Advisory Board with PRISMS, Inc. Clinical trial with Vanda Pharmaceuticals. Board member with ACMG Foundation, and ABMG. Research grants received from Smith-Magenis Syndrome Research Foundation, PTC Therapeutics, PRISMS, Rhythm Pharmaceuticals, U.S. NIH, Speragen, Jerome Lejeune Foundation.

Steven Gray: Consulting for Roche and Alektor. Scientific Advisory Board member with Codexis, Lysogene, Nanoscope, Opsin Therapeutics, Sarepta, and Vertex Pharmaceuticals, Charcot Marie Tooth Association. Received funded grants, research or clinical trials with Taysha Gene Therapies and Roche. Licensing royalty income from Asklepios Biopharma, Neurogene, Abeona Therapeutics, Taysha Gene Therapies.

Heather Leigh Gray-Edwards: SAB; consultant with AGCT, Biogen. Received grants from EnClever Therapies, ASC therapeutics, and AGTC. AAV gene therapy in phase I/II – AXO-GM1 and AXO-GM2.

Aneal Khan: Has received any combination of honoraria, travel grants or consulting fees from Alexion, Actelion, Amicus, AvroBio, Biomarin, Cytonet, Horizon, Idorsia, Sanofi, Takeda and Ultragenyx. Is a board member of the International Collaborative Group of Gaucher Disease, Sanofi Pompe Registry, Hypophosphatosis Advisory Board, Canadian Fabry Association, Canadian Pompe Association, Mito-Canada, Canadian Fabry Disease Initiative. Patent on cell free mitochondrial DNA analysis. All other investments or relationships that could be seen by a reasonable, well informed participant as having potential to influence the content of the educational activity: CEO, Discovery DNA, President, M.A.G.I.C. Clinic Ltd. (Metabolics and Genetics in Canada), President, Rare Disease Network of Alberta, Revenue Sharing Agreement on Fabry Gene Therapy with University Health Network.

Murray Potter: Advisory board / consultant with Ultragenyx Therapeutics and Advisory board member with Horizon Therapeutics.

Cheryl Rockman-Greenberg: Is a member of Scientific Advisory board with Alexion Pharmaceuticals, Sanofi-Genzyme. Has received research grant from Takeda and Alexion

David Rosenblatt: Is a salary Professor with McGill University. PI – CIHR and Collaborator with NIH.

Andreas Schulze: Consulting for Guidepoint Global LLC, BioMedical Insights, Inc., PharmaIntelligence, Recordati, Satellite BioSciences, Inc., Advisory Board member with Ultragenyx, ANTIBODY / Horizon, Cycle Pharma. PI on Clinical Studies and Lab Research with Aeglea, Sanfilippo Foundation, Cure Sanfilippo, Children's National.

Jagdeep Walia: Has licensed the patented technology and is an Advisory board member with Taysha Gene Therapies Inc and received research grants from Taysha Gene Therapies Inc, and Azafaros.

Danielle Stayzer: Employment with McMaster University, CPD.

Carol Stewart: Employment with McMaster University, CPD.

INFORMATION

PHOTOGRAPHY

Photos will be taken at this activity which may be used in future marketing materials. If you have questions, please visit the registration desk.

LANYARDS/ACTIVITY PACKAGES

As the registrant your activity package includes breakfast, nutritional breaks, lunch, and welcome reception. Your activity lanyard must be worn at all times throughout the conference.

CERTIFICATE OF ATTENDANCE

An email will be sent after the activity, confirming your certificate of attendance is ready to download or print.

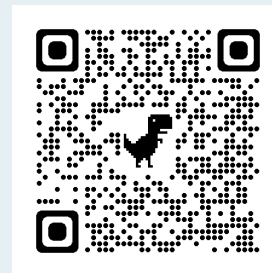
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Your feedback is valuable! Please take a moment to complete the evaluation survey for this CPD Conference.

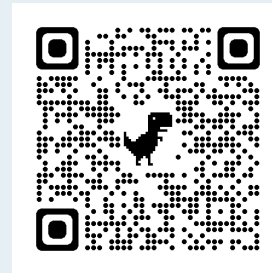
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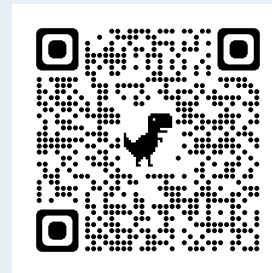
DAY 1



DAY 2



DAY 3





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PLANNING COMMITTEE

David Sinasac, PhD, FCCMG – Chair

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Professor and Chair
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McMaster University, Hamilton, ON

EXECUTIVE COMMITTEE

Andreas Schulze, MD, PhD, FRCPC, President of Garrod Association

Professor Paediatrics and Biochemistry
University of Toronto, Toronto, ON

Jagdeep S Walia, MBBS, FRCPC, FCCMG, Secretary/ Treasurer of Garrod Association

Clinical Geneticist
Professor
Director of Research (Department of Pediatrics)
Kingston Health Sciences Centre and
Queen's University, Kingston, ON

Paula J Waters, PhD, FCCMG, Vice President of Garrod Association

Biochemical Geneticist and Affiliate Professor, Medical Genetics Service
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