

Curriculum Vitae

Kevin Hsin-Mu Kuo

A. Date Curriculum Vitae is Prepared: 2026 March 5

B. Biographical Information

Primary Office	200 Elizabeth Street Toronto, Ontario, Canada M5G 2C4
Telephone	416-340-5233
Email	kevin.kuo@uhn.ca

1. EDUCATION

Degrees

2014 Jul - 2015 Jun	Clinical Research Methodology, American Society of Hematology Clinical Research Training Institute, United States
2010 Sep - 2014 Jun	MSc, Health Research Methodology, Clinical Epidemiology and Biostatistics, McMaster University, Hamilton, Ontario, Canada, Supervisor(s): Dr. Mark Crowther
2001 Sep - 2005 May	MD, Western University, London, Ontario, Canada
1997 Sep - 2001 Jun	BSc, Molecular Genetics & Molecular Biology Specialist program (with High Distinction), University of St. Michael's College, University of Toronto, Toronto, Ontario, Canada

Postgraduate, Research and Specialty Training

2010 - 2012	Clinical fellow in the Canadian Adult Comprehensive Hemoglobinopathy Fellowship, Department of Medicine, Division of Haematology, University of Toronto
2008 - 2010	Adult Hematology program, Department of Medicine, Division of Haematology, University of Toronto
2005 - 2008	Core General Internal Medicine program, Department of Medicine, Division of Haematology, University of Toronto

Qualifications, Certifications and Licenses

2012 Apr - present	GP License, United Kingdom General Medical Council, United Kingdom, License / Membership #: 7146199
2009 Aug - present	Fellow, Royal College of Physicians of Canada
2012 Jun	Certificate of Clinical Fellowship in Benign Hematology and Hemoglobinopathies, University of Toronto
2010 Oct	Sub-specialty Certificate of Hematology, Royal College of Physicians and Surgeons of Canada
2009 Jun	Specialty Certificate of Internal Medicine, Royal College of Physicians and Surgeons of Canada

Licentiate, Medical Council of Canada, License / Membership #: 112379

Licence, College of Physicians and Surgeons of Ontario, License / Membership #: 82448

2. EMPLOYMENT

Current Appointments

2025 Jan 1 - present	Vice President, Research and Innovation, Scarborough Health Network, Scarborough, Ontario, Canada
2025 Jan 1 - present	Active Staff, Department of Medicine, Scarborough Health Network, Scarborough, Ontario, Canada
2022 Jul 1 - present	Associate Professor, Division of Hematology, Medicine, Faculty of Medicine, University of Toronto, Toronto, Ontario, Canada
2021 Nov 1 - present	Assistant Professor (status), Health Policy, Management and Evaluation, Dalla Lana School of Public Health, University of Toronto, Toronto, Ontario, Canada
2021 Mar - present	Member of the College of Reviewers, Canadian Institutes of Health Research, Canada
2015 Jul 15 - present	Specialist consultant, Telemedicine Network, Medecins Sans Frontieres <i>Specialist consultant for the MSF telemedicine network</i>
2014 Jan 1 - present	Active Staff, Division of Medical Oncology and Hematology, University Health Network, Toronto, Ontario, Canada

Previous Appointments

CLINICAL

2016 Nov 29 - 2024 Dec 31	Associate Staff, Hospital for Sick Children, Toronto, Ontario, Canada
2014 Oct - 2024 Dec 31	Associate Staff, General Internal Medicine, Department of Medicine, Women's College Hospital, Toronto, Ontario, Canada
2014 Oct - 2024 Dec 31	Associate Staff, General Internal Medicine, Department of Medicine, Mount Sinai Hospital, Toronto, Ontario, Canada
2012 Jul 1 - 2013 Dec 31	Associate Staff, Division of Medical Oncology and Hematology, University Health Network, Toronto, Ontario, Canada

HOSPITAL

2023 Nov 17 - 2024 Nov 17	Clinical Lead and Research Lead - Red Blood Cell Disorders Clinic, Division of Medical Oncology and Hematology, Department of Medicine, University Health Network, Toronto, Ontario, Canada
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UNIVERSITY - RANK

2014 Oct 28 - 2022 Jun 30	Assistant Professor, Division of Hematology, Medicine, Faculty of Medicine, University of Toronto, Toronto, Ontario, Canada
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3. HONOURS AND CAREER AWARDS

Distinctions and Research Awards

INTERNATIONAL

Received

2011	Abstract Achievement Award , 53rd American Society of Hematology Annual Meeting, San Diego, California. (Distinction)
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For Abstract #2326: Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sickle Cell Disease.

- 2004 **Medical student scholarship**, American Society of Hematology. (Distinction)
1996 **Bausch & Lomb Honorary Science award**, Bausch & Lomb, New York. (Distinction)

NATIONAL

Received

- 2023 Feb **Hour Glass Award**, Thalassemia Foundation of Canada. (Distinction)
Demonstrating comfort, caring, dedication and compassion towards thalassemia.
- 2022 Mar **Recognition of outstanding contributions as CIHR peer reviewer**, Canadian Institutes of Health Research. (Distinction)
Dr. Kevin Kuo is among a select group of reviewers who have been identified through CIHR's Review Quality Assurance (RQA) Process as an Outstanding reviewer in recognition of his exemplary contribution to peer review for the Fall 2021 Project Grant competition. Among the 1,094 reviewers that participated in the competition, only 22.9% obtained this recognition.
- 2008 **John H. Crookston Award**, Canadian Hematology Society. (Distinction)

PROVINCIAL / REGIONAL

Received

- 2024 Jun **Lyonel G Israels Memorial Lectureship**, Cancer Care Manitoba, Winnipeg, Manitoba, Canada. (Distinction)
The Dr. Lyonel G. Israels Memorial Lecture was created to honour a man who tirelessly dedicated himself to providing the people of Manitoba with an organization that embodied the elements of empathetic patient care, outstanding teaching, and research, and to acknowledge his tremendous contributions as a clinician, teacher and researcher.

Nominated

- 2024 Feb **Medical Award of Excellence (declined)**, Sickle Cell Awareness Group of Ontario, Ontario, Canada. (Distinction)
This Award is given to an individual who has contributed significantly to the advancement of care and treatment for individuals with sickle cell disease in Ontario. The nominee could be a researcher, hematologist, or family doctor.

LOCAL

Received

- 2010 **Walker Dotten Award**, St. Michael's Hospital, Toronto. (Distinction)
- 2005 **The Dr. David Meltzer Undergraduate Award in Haematology & Oncology**, Western University. (Distinction)
- 2002 **Life Sciences Committee undergraduate summer studentship award**, University of Toronto. (Distinction)
- 2001 **Life Sciences Committee undergraduate summer studentship award**, University of Toronto. (Distinction)
- 2001 **Silver medal – in recognition of academic achievement**, St. Michael's College, University of Toronto. (Distinction)
- 1999 **In-course scholarship**, St. Michael's College, University of Toronto. (Distinction)
- 1998 - 2000 **Foundation scholarship**, St. Michael's College, University of Toronto. (Distinction)

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- 1997 - 2000 **Department of Biochemistry summer studentship award**, University of Toronto.
(Distinction)
- 1997 **Admission scholarship, St. Michael's College**, University of Toronto. (Distinction)

Student/Trainee Awards

INTERNATIONAL

Received

- 2022 May - 2023 Jul **Hematology Opportunities for the Next Generation of Research Scientists (HONORS)**, Hematology, Supervisor, Awardee Name: Budhram, Dalton. American Society of Hematology, United States
Total Amount: 5,000 CAD
- 2020 Dec **Abstract Achievement Award**, Supervisor, Awardee Name: Forte S. 62nd American Society of Hematology Annual Meeting, United States
For Abstract #369: Sickel Cell Cerebrovascular Reactivity to a CO2 Stimulus Is Both Too Little and Too Slow. Total Amount: 500 USD
- 2018 Dec **Abstract Achievement Award**, Supervisor, Awardee Name: Rosart R. 60th American Society of Hematology Annual Meeting, San Diego, California, United States
For Abstract #2287: Validation of Sickel Cell Disease Severity Score in a Cohort of Hemoglobin SC Disease Patients. Total Amount: 500 USD

LOCAL

Received

- 2015 Jul - 2016 Jun **MACK Foundation Bursary**, Supervisor, Awardee Name: Rotin LE. University of Toronto, Toronto, Canada
To reimburse expenses incurred while presenting: "Allogeneic hematopoietic stem cell transplant versus gene therapy review". Total Amount: 1,000 CAD
- 2014 Mar **Research Award runner up**, Hematology, Supervisor, Awardee Name: Veronique Naessens. University of Toronto, Toronto, Ontario, Canada
Effectiveness Of An Analgesia Protocol For The Treatment Of Painful Vaso-Occlusive Crisis Of Sickel Cell Disease Patients In Emergency Room: A Retrospective Cohort Study"
Naessens, Veronique MDCM, FRCPC, Richard Ward, MBBS, MSc, MRCP, FRCPath, and Kevin H.M. Kuo, MD, FRCPC. Total Amount: 750 CAD

4. PROFESSIONAL AFFILIATIONS AND ACTIVITIES

Administrative Activities

INTERNATIONAL

American Society of Hematology

- 2024 Jan 1 - 2024 Dec 31 Education Program Advisory Panel, Washington, District of Columbia, United States.
- 2024 Jan 1 - 2024 Dec 31 Annual Meeting Accredited Education Committee (AMAEC), Washington, District of Columbia, United States.
- 2017 Jan 1 - 2019 Dec 31 **Member**, Teaching Cases Working Group, United States.
- 2015 Jan 7 - 2017 Dec 31 **Member**, Teaching Cases Subcommittee, United States.
- 2011 **Member**, Mentor Award Study Section

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2010 - 2016	Member , ASH Web User Group
2010	Member , Mentor Award Study Section
2009 - 2011	Member , Trainee Council

NATIONAL

Royal College of Physicians and Surgeons of Canada

2009 Sep	On-site Surveyor , University of Saskatchewan's Residency Programs and Affiliated Education Sites
2008	Participant , Workshop for New Surveyors and Accreditation

PROVINCIAL / REGIONAL

Professional Association of Internes and Residents of Ontario

2006 - 2007	Member , Awards Selection Committee, Ontario, Canada.
2005 Jul 1 - 2009 Jul 30	Council Member , General Council, Ontario, Canada.

Western University

2010 Jun	External Reviewer , Faculty organization of Post-Graduate Medical Education
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LOCAL

Other Organizations

2025 Jan - present	Residency Program Committee, Faculty of Medicine, Dept of Medicine, Haematology, Postgraduate MD, Toronto, Ontario, Canada.
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University Health Network

2022 Feb 4 - present	Member , Pride in Patient Engagement in Research (PiPER) Steering Committee, Toronto, Ontario, Canada.
2019 Aug 14 - 2019 Sep 26	Member , "Engage every patient to strengthen our learning health system" Strategic Action Planning Working Group, Toronto, Ontario, Canada. <i>Developed key initiatives and operational plan around the "Engage Every Patient" Strategic Research Priority outlined in the UHN Strategic Research Plan to further patient engagement in research.</i>
2017 Mar 10 - 2024 Dec 31	DMOH Blood Disorders Program Recruitment Committee, Toronto, Ontario, Canada.
2015 Mar - 2016 Mar	Apheresis Governance Committee, Toronto, Ontario, Canada.

University of Toronto

2010 Sep	Internal Reviewer , Postgraduate Medical Education program of Orthopaedic Surgery
2010 Sep	Internal Reviewer , Postgraduate Medical Education program of Ophthalmology
2009 Jul 1 - 2010 Jun 30	Chief resident , Adult Haematology training program
2008 Apr	Internal Reviewer , Postgraduate Medical Education program of Nuclear Medicine
2008	Tutor , Post-graduate year 1 Bedside Procedures Session, Faculty of Medicine, Postgraduate MD
2006 - 2008	Member , Pandemic Influenza Committee, Faculty of Medicine
2005 - 2010	Member , Board of Examiners, Postgraduate Medical Education, Faculty of Medicine, Postgraduate MD
2005 - 2008	Member , Internal Review Committee, Postgraduate Medical Education
2005 - 2008	Member , Faculty Council, Faculty of Medicine

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2000 **President**, Molecular Genetics and Biology Students' Union

Western University

2004 - 2006 **Member**, Health Policy Initiative

2002 - 2003 **Senator**, University of Western Ontario Senate
Representing students of the Faculties of Health Sciences and Medicine & Dentistry.

2002 - 2003 **Member**, Student Caucus on Governance

Peer Review Activities

ASSOCIATE OR SECTION EDITING

Editor

2023 Jan 1 - 2023 Oct Orphanet Journal of Rare Diseases

EDITORIAL BOARDS

Editor

2022 Jan - present International Society of Hematology, Hematology journal (The official Journal of the International Society of Hematology (ISH))

GRANT REVIEWS

External Grant Reviewer

2018 Sep - present Canadian Institutes of Health Research, Clinical Investigation – C: Digestive, Endocrine and Excretory Systems (CIC) committee for the Project
Grant, Number of Reviews: 102

PRESENTATION REVIEWS

Reviewer

2023 Aug 4 - 2023 Aug 13 American Society of Hematology, 65th ASH Annual Meeting: scientific category 112--
Thalassemia and Globin Gene Regulation, Number of Reviews: 48

2018 Sep 8 Canadian Haemoglobinopathy Association (CanHaem), New Beginnings, the 5th Annual
Canadian Haemoglobinopathy Association Conference

Coordinating Reviewer

2024 Jan - 2024 Dec 66th Annual Meeting of American Society of Hematology

Other Research and Professional Activities

RESEARCH PROJECT

2022 Jan - present **co-Principal Investigator**. Prediction of Adverse Outcomes in Pregnancies of Individuals with Sickle Cell Disease: The Sickle Cell Risk in Pregnancy Tool. Collaborator(s): Malinowski KA, Shehata N, Bartolucci P, Forte S, Little J, Pecker L, Saif Ur Rehman S, Sharma D, Tomlinson G, Early M, Eke A, Habibi A, Kelly J, King A, Mahone M, Solow M, Soulières D, Williams V, Wo BL, Benachi A, Found W, Lecarpentier E, Sanon W, Tinga B, Waithe J.

2021 Feb - present **Principal Investigator**. The Role of Microvasculopathy in the Development of Neurocognitive Dysfunction in Patients with COVID-19. Toronto, Ontario, Canada.
Supervisor(s): **Kuo KHM**. Collaborator(s): Solow M, Sobczyk O, Fisher J, Mikulis D, Wilcox E.

2019 Feb - present **Principal Investigator**. Comparing Gene Therapy and Allogeneic Hematopoietic Stem Cell

	Transplant in Adults with Sickle Cell Disease. Toronto, Ontario, Canada. Supervisor(s): Kuo KHM . Collaborator(s): Rotin LE, Kumar R, Viswabandya A.
2018 Jul - present	Principal Investigator. The association between microvascular disease and abnormal cerebrovascular reactivity in adult sickle cell patients (RetroCVR). Toronto, Ontario, Canada. Supervisor(s): Kuo KHM . Collaborator(s): Forte S, Sobczyk O, Poubanc J, McKinnon L, Venkatraghavan L, Hare G, Fisher J, Mikulis D.
2018 Jun - present	Principal Investigator. Hemoglobin S Recovery, Erythropoiesis and Hemolysis Kinetics in Sickle Cell Disease Patients Undergoing Red Blood Cell Exchange Transfusion. Toronto, Ontario, Canada. Supervisor(s): Kuo KHM . Collaborator(s): Aneke JC, Forte S, Barth D, Ward R.
2018 Feb - present	Principal Investigator. Predicting Maximum Tolerated Dose of Hydroxyurea in Sickle Cell Disease Using Artificial Intelligence. Toronto, Ontario, Canada. Supervisor(s): Kuo KHM . Collaborator(s): Brailovski Eugène, Iaboni Aled, Lionel Anath.
2015 Jul 1 - present	Co-Principal Investigator. Tissue Hypoxia as a Central Initiating Mechanism for Multi-Organ Failure and Mortality in Patients with Sickle Cell Anemia. University Health Network, Toronto, Ontario, Canada. Collaborator(s): Hare G (co-PI), Sled J.
2018 Sep - 2019 Jun	Principal Investigator. A multicenter study evaluating the reliability and validity of the Rowland Universal Dementia Assessment Scale (RUDAS) and predictors of RUDAS performance in adults with Sickle Cell Disease. Canada. Supervisor(s): Kuo KHM . Collaborator(s): Forté S, Soulières D, Naessens V, Béland S, Cohn M. <i>Multicentered cross-sectional study (Toronto: UHN; Montreal: CHUM, MUHC; Paris, France: AP-HP).</i>
2018 Jul - 2022 Mar	Principal Investigator. Prophylactic Anticoagulation and Central Venous Access Device-related Thromboembolism In Adult Sickle Cell Patients (PAVIS): a case-control study. Toronto, Ontario, Canada. Supervisor(s): Kuo KHM . Collaborator(s): Forte S, Fadiga N, Aneke JC, Bhat S, Ward R.
2015 Oct - 2019 Dec	Principal Investigator. Validation of Sickle Cell Disease Severity Score in a Cohort of Hemoglobin SC Disease Patients. Canada. Supervisor(s): Kuo KHM . Collaborator(s): Rosart RM, Pestrin OA, Tomlinson GA, Ward R, Pendergrast J, Binding A, Kuo KHM . <i>Multicentered cross-sectional study (Toronto: UHN; Montreal: CHUM, MUHC; Paris, France: AP-HP).</i>
2014 Oct - 2015 Dec	Principal Investigator. Physician Survey of Practice Patterns in the Treatment of Osteonecrosis of the hip among US and Canadian Hematologists (MONSAPP). United States. Collaborator(s): Mulla-Ali DH, Brandow A, Hedde N, Tomlinson G. <i>International survey.</i>
2014 Sep - 2014 Oct	Co-investigator. Patient Engagement in the Success of a Shared Care Model. University Health Network, Ontario, Canada. Collaborator(s): Murray C, Maki K, Doherty MC. <i>Data analysis and interpretation.</i>
2014 May - 2014 Jul	Co-Investigator. Risk Factors Associated with Adverse Pregnancy Outcomes in Women with Sickle Cell Disease. Mount Sinai Hospital, Toronto, Ontario, Canada. Collaborator(s): A Malinowski, N Shehata, K Kuo , R D'Souza.
2014 Jan 7 - 2014 Dec	Principal Investigator. Management of cancer-associated thrombosis with concomitant thrombocytopenia: a survey of Canadian hematologists. University Health Network, Toronto, Ontario, Canada. <i>National, survey.</i>
2012 Jan - 2015 Nov	Co-Investigator. Human mesenchymal stromal cells (MSCs) for the treatment of early stage osteonecrosis of the hip; a phase I dose-escalation study. University of Toronto, Toronto, Ontario, Canada. Collaborator(s): Keating A.
2010 Jan - 2013 Jan	Co-Investigator. Conversion of human fibroblasts from patients with hemoglobinopathies to erythropoietic progenitors. University of Toronto, Toronto, Ontario, Canada. Collaborator(s): Keating A.
2008 - 2010	Co-Investigator. The effects of EspP on the human coagulation cascade. University of Toronto.

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- 2005 - 2010 Hyperleukocytosis in acute myelogenous leukemia. University of Toronto. Supervisor(s): Prof. H.A. Messner.
Resident research project.
- 2004 - 2009 **Co-Investigator.** Treatment-related changes in markers of neurodegeneration over time in the cerebrospinal fluid of adults with haematologic malignancies receiving chemotherapy. Western University.
- 2004 **Research Project Student.** The relationship between transfusion knowledge, risk perception and desire for alternatives to donor blood in medical trainees. Western University.
Supervisor(s): Dr. I.H. Chin-Yee.
- 2003 Case series review in the treatment of heparin induced thrombocytopenia with Fondaparinux. Western University. Supervisor(s): Dr. M.J. Kovacs.
- 2000 Isolated the gene of prepropenicillopepsin-JT3. University of Toronto. Supervisor(s): Professor Emeritus T. Hofmann, Department of Biochemistry.
(*GenBank accession number AF295432*).

CANHAEM ABSTRACT REVIEWER

- 2021 Aug 20 **Grant application reviewer.** 2021 Virtual CanHaem Annual Meeting, Canada.

CIHR PROJECT GRANT COMPETITION

- 2021 Aug 20 **Grant application reviewer.** CanHaem Annual Conference, Canada.
- 2018 Apr - 2020 Aug **Grant application reviewer.** Canadian Institutes of Health Research, Canada.

CONSULTATION

- 2021 Feb 19 **Participant.** Building a National Strategy for High-Cost Drugs for Rare Diseases. Office of Pharmaceuticals Management Strategies, Strategic Policy Branch, Health Canada, Canada.
Provided consultation to Health Canada on building a national strategy for high-cost drugs for rare diseases.

DATA SAFETY MONITORING COMMITTEE

- 2019 Mar - present **Member.** Protocol summary of the Comprehensive Anaemia Programme and Personalized Therapies (CAPPT). University College London (UCL), London School of Hygiene & Tropical Medicine, HERD International, United Kingdom.
- 2018 Sep 18 - present **Chair of the DSMC.** A Phase 1/2, Open-label, Multicenter, Single-arm Study to Assess the Safety, Tolerability and Efficacy of BIVV003 for Autologous Hematopoietic Stem Cell Transplantation in Patients with Severe Sickle Cell Disease. Bioverativ, Massachusetts, United States.

EDUCATION PROGRAM ADVISORY PANEL

- 2023 Jul - 2024 Dec **Member.** American Society of Hematology, United States.

GUIDELINES WORKING GROUP

- 2023 Jan - 2024 Jan **Member.** First International Guidelines for the Diagnosis and Management of Pyruvate Kinase Deficiency. United States.

MEASUREMENT PROTOCOL CONSENSUS WORKING GROUP

- 2023 Jan - present **Member.** The PhenX Toolkit Sickle Cell Disease Pregnancy Working Group. RTI

International, United States.

The PhenX Toolkit is a freely available, online resource that biomedical researchers can turn to with confidence to identify well-established, recommended measurement protocols. The working group used an established consensus process to identify 10 new measures relevant to SCD pregnancy complications to add to the PhenX Toolkit. These efforts will help ensure that the PhenX Toolkit continues to meet the needs of the research community.

RESEARCH INFRASTRUCTURE DEVELOPMENT AND ADMINISTRATION

- 2013 Jul - 2015 Dec **Chair of the research subcommittee.** Chair of the research subcommittee. Canadian Hemoglobinopathy Association, Canada. *National.*
- 2012 Jul - 2021 Jun **Researcher database developer lead.** Researcher database developer lead. Sickie Cell Adult Provider Network, United States. *International.*

TRIAL STEERING COMMITTEE

- 2019 Jul - present **Member.** International Pyruvate Kinase Deficiency Registry (PEAK). United States.

OTHER

- 2013 Nov **Reviewer-case content.** Internal Medicine Clinical Encounters Project (IMCE). University of Toronto, Toronto, Ontario, Canada. Supervisor(s): Dr. Lisa Richardson.

C. Academic Profile

1. RESEARCH STATEMENTS

- 2014 Jul - present Predicting adverse outcomes in pregnancies of individuals with sickle cell disease. Sickle Cell Disease (SCD) is a rare disorder with a high disease-burden, predominantly affecting persons of Black ancestry. These individuals face a triple disadvantage: a crippling chronic disease affecting quality of life and life potential, often limiting upward socio-economic mobility; negative bias frequently encountered during health-care interactions; and a scarcity of research addressing issues of importance to the Black population. Pregnancy compounds this further, whereby pregnant persons with SCD have more adverse pregnancy outcomes (APO): 6 times higher risk of maternal mortality, two times higher risk of preeclampsia and preterm birth, 4 times higher risk of growth restriction & stillbirth, 7 times higher admission rates (nearly 1 in 2 needing admission), often on multiple occasions and lasting days to months, resulting in higher resource utilization. Yet notably, up to 30% of pregnancies in people with SCD remain uncomplicated. A tool is urgently needed to separate high-risk pregnancies that may benefit from interventions like transfusion, from those likely to be uncomplicated, where transfusion would introduce needless harm. In collaboration with the maternal fetal medicine unit at Mount Sinai Hospital, Drs. Kinga Malinowski (high risk obstetrician) and Nadine Shehata (hematologist), we created a maternal and fetal risk prediction model using our SCD cohort to help guide therapy (Malinowski 2021; online calculator available at http://individual.utoronto.ca/kevinkuo/MF_risk_calculator.html). We are currently validating our model via a multinational collaboration involving centres from Canada, France, and US called Sickle cell Pregnancy Alliance and Research Knowledge Syndicate (SPARKS), a pregnancy-focused, unique, international collaborative that includes and patient partners, patient organisations, and SCD experts in maternal-fetal medicine (MFM) and hematology.

2014 Jan 1 - present

Neurocognitive impact of sickle cell disease on adults and young adults.

1) Neurocognitive impact of sickle cell disease on adults and young adults.

Up to 50% of patients with sickle cell disease (SCD), a form of congenital hemolytic anemias, suffer from neurologic sequelae. The vast majority suffer from mild cognitive impairment (MCI) without effective treatment or rehabilitation strategy. Access to neurocognitive tests and neuropsychologists, a pre-requisite for rehabilitation, remains a barrier due to its prohibitive costs, time, and inadequate availability of trained psychometrists. I designed a strategy where non-expert health care practitioners can employ rapid and easy to administer neurocognitive test to screen for possible MCI and dementia, and refer patients who screened positive for formal neurocognitive testing. Patients can then access neurorehabilitation resources once they are formally diagnosed with MCI or dementia. It has the potential of being easily integrated into existing comprehensive models of care for SCD patients, with minimal impact of resource utilization. This is particularly appealing to low to middle income countries where SCD is prevalent given the scarcity of diagnostic and rehabilitation resources. In order to implement the above:

A) I chose to examine the Rowland Universal Dementia Assessment Scale (RUDAS) as a possible screening test because it is paper-based and can be administered by a non-expert health care practitioner in 6 minutes, even for patients with low literacy. I have built and lead a binational (Canada, France), bilingual, multicentered, prospective study to examine the reliability and validity of RUDAS in SCD adults as a prerequisite for deployment in the clinical setting. More than 250 patients from UHN participated in this multinational study. Result from the study indicates that 10% of SCD patients under 60 years of age have suspected MCI/dementia, and that renal dysfunction and reticulocyte count are independent predictors of MCI/dementia (Forte 2021). Results from the study informed the feasibility of using Rowland Universal Dementia Assessment Scale as a rapid and easily to administer neurocognitive test to screen for possible mild cognitive impairment and dementia in SCD patients. It was also hypothesis-generating, that anemia-induced tissue hypoxia may be a modifiable risk factor for cerebral and renal injury and dysfunction in SCD patients. Current project aims to validate RUDAS and confirm the above findings in a larger multicenter cohort.

B) In parallel, I have searched for surrogate measures of early stages of cognitive impairment. I have established a collaborative effort with neuroimaging scientists at the Krembil Institute and anesthesiology scientists from the Department of Anesthesia and successfully conducted studies to examine the association between cerebral vascular reactivity (CVR) and severity of SCD complications, and changes in CVR before and after transfusion therapy. CVR was measured as the blood oxygen level dependent signal (a surrogate for cerebral blood flow) response to an increase in the end tidal partial pressure of CO₂ (PET CO₂). We found that cerebrovascular responsiveness to CO₂ in patients with SCD is both decreased and slowed compared to healthy controls and hematocrit is the strongest predictor of abnormal CVR (Forte 2022; Sayin 2022a). In addition, we were the first group to document resistance measures in the cerebral vasculature of SCD patients and to compare them to normal individuals (Sayin 2022b).

C) Following on the findings from aims A and B that renal dysfunction and erythroid mass (reticulocyte count and hematocrit) are critical predictor to MCI and CVR abnormality, I have established a collaboration with physiology scientists at the Keenan Research Centre to explore anemia-induced tissue hypoxia as a modifiable risk factor for cerebral and renal injury and dysfunction in SCD patients, using Sickle Townes mouse as the animal model. The results may provide insight on how to optimize transfusion practices for stroke and silent cerebral infarct prophylaxis, which will allow me to identify novel therapeutic targets in the treatment of cognitive impairment in SCD patients in the next 5 to 10 years. The study (Forte 2021) has shown that a large portion of sickle cell disease (SCD) patients have suspected dementia, and that renal dysfunction and reticulocyte count are independent risk factors of dementia. Results from the study informed the feasibility of using Rowland Universal Dementia Assessment Scale as a rapid and easily to administer neurocognitive test to screen for possible mild cognitive impairment and dementia in SCD patients. It was also hypothesis-generating, that anemia-induced tissue hypoxia may be a modifiable risk factor for cerebral and renal injury and dysfunction in SCD patients.

2014 Jan 1 - present

Non-chelating strategies to treat iron overload in congenital hemolytic anemias.

An inevitable complication of chronic hemolysis is iron overload. While iron chelators are highly efficacious, their effectiveness is limited by their significant toxicity and side-effect profile, which are major barriers in establishing good adherence.

A) Deferiprone has traditionally been regarded as a poor chelator to remove liver iron. I hypothesized that the lack of effectiveness was due to dosing was traditionally limited to below 100 mg/kg/day, and concern for emergence of side-effect at high doses.

B) In collaboration with researchers in the Department of Radiology, we established an in-house method of hepatic iron quantification, and determined that switching between this method and another standard of care method did not alter the clinical decision making on chelator dose adjustment. We are currently employing artificial intelligence to further understand and optimize clinical decision-making around iron chelation based on iron quantification by MRI (Sussman 2020, Jhaveri 2019)

C) In collaboration with UHN Peter Munk Cardiac Centre, I have repurposed existing drugs and natural compounds that have better side-effect profiles to block iron entry into cardiac, hepatic and endocrine tissues by conducting 2 pilot randomized controlled trials of non-chelating agents (amlodipine, taurine). The CANALI RCT (NCT02474420) is near conclusion. I have demonstrated international impact by collaborating in two large multinational-multicenter studies (BELIEVE and DRIVE-PK) where we demonstrated a reduction in transfusion via improving ineffective erythropoiesis and reducing hemolysis, resulting in co-authorship in 2 articles in the New England Journal of Medicine.

A) I have shown that hepatic iron removal is most effective at an actual dose of >100 mg/kg/day, and that the frequency and type of side-effect from the iron chelator deferiprone is not dose-related. This finding has been published (Binding 2019) and will allow clinicians to make informed decisions when considering dose-escalation of deferiprone.

B) As the current method of hepatic iron quantification is proprietary and costs ~\$500 per scan, the availability of a reliable non-proprietary method of iron quantification will translate into cost-savings to the provincial government in Ontario and potentially elsewhere in Canada.

C) One drug, luspatercept, is now approved for use in transfusion-dependent thalassemia patients in Canada.

2014 Jan 1 - present

Novel disease-modifying and curative therapies to treat congenital hemolytic anemias.

Amelioration of chronic hemolysis requires the identification and correction of the underlying genetic abnormality.

A) INHERENT is a multinational effort involving 72 participating centres in 33 countries to identify genetic modifiers of hemoglobinopathy phenotypes. As the coordinating investigator for Canada, chair of the knowledge translation working group, and member of the trial steering committee, I am responsible for overseeing the design, to lead the knowledge translation efforts, and to lead the execution of the study in Canada. Knowledge generated from INHERENT will allow identification of novel therapeutic targets.

B) Induction of the functional fetal globin to replace the non-functional globin is one strategy to correct the hemolytic anemias like thalassemia and SCD. Along with my collaborators from Boston University and Phoenicia Biosciences, I have been awarded \$5.7 million USD by NHLBI/NIH over the next 3 years to evaluate Benserazide, a repurposed drug used for treatment of Parkinson's disease, for tolerability, pharmacokinetics, and fetal globin expression in beta thalassemia intermedia and SCD.

C) While allogeneic hematopoietic stem cell transplant (HSCT) has traditionally been the only curative option for patients with congenital hemolytic anemias, the availability of gene therapy in clinical trial setting now provides patients with the ability to choose between these two potentially curative options. I have 1) successfully championed for the funding of allogeneic HSCT for non-malignant hematologic disorders in Ontario (see Creative Professional Activities section), 2) conducted a systematic review that compared the efficacy and safety outcomes between allo-HSCT and gene therapy, initially published as an abstract at the annual meetings of the American Society of Hematology (ASH) (Rotin 2019, 2020), and the manuscript has been submitted for publication. In addition, in collaboration with

Cincinnati Children's Hospital Medical Center (now Aruvant Sciences), I have been pursuing gene transfer using a fetal globin lentivirus vector with reduced intensity conditioning regimen. D) Directly reducing hemolysis by increasing available reductant in red blood cells is another method to modify the underlying hemolytic anemia. In collaboration with Agios, I am the lead investigator involved in the design and execution of a multicenter phase 2 trial evaluating mitapivat in treatment of thalassemia intermedia, the first study aimed at the treatment of alpha-thalassemia. Following the success of the phase 2 trial (12/13 met primary endpoint of hemoglobin increase > 1.0 g/dL), I am currently designing the phase 3 trial and am the global PI for the study. I also assisted in the design of the phase 3 trial of mitapivat in SCD. I am also collaborating with two other European centres to explore the use of mitapivat in the treatment of other congenital hemolytic anemias.

2016 Feb - 2019 May

Models of Care to Improve Adherence.

As a clinician-investigator caring for the largest adult population with sickle cell disease in Canada, my research focus has been on adolescents and young adults with sickle cell disease, in particular ameliorating the negative impact of transition from pediatric to adult care. As the PI on the retrospective analysis of the clinical impact of a comprehensive transition program with transition navigator, I designed the analysis to incorporate clinically meaningful outcomes, which had never been previously examined in the field. The results were presented in several major forums including oral presentations at the American Society of Hematology (<http://www.bloodjournal.org/content/128/22/317>) and the International Conference on Thalassemia & Haemoglobinopathies, and the manuscript was published in Pediatric Blood and Cancer.

The development and retrospective validation of a comprehensive transition program with transition navigator in collaboration with Hospital for Sick Children and dissemination of the results as oral presentations in international conferences (American Society of Hematology and the International Conference on Thalassemia & Haemoglobinopathies) resulted in several sickle cell disease comprehensive care centres in the United States and Canada expressing interest in adopting the program.

2. TEACHING PHILOSOPHY

My teaching philosophy centers around creating opportunities for learners to explore diagnostic and management dilemma in a safe and non-judgmental learning environment. It is only through exploration that the learner will critically think through the case, recognize their strengths and limits, and be able to communicate their findings and plan to their teachers in a non-confrontational manner. I also believe that such exploration will allow learners to extract common themes and thread underlying the approach to both common and esoteric problems, to see the forest from the trees. This is especially true in my clinical practice of rare congenital hemolytic anemias. The diagnosis is esoteric and often requires investigations by multiple subspecialists, but a learner can apply first principles learnt from their medical school or undergraduate experience to guide them in deriving a safe diagnostic and management plan. For example, internal medicine trainees are often flustered by the myriad of differential diagnoses underlying secondary erythrocytosis. I would first reassure the learners that getting the precise diagnosis is often not as important as managing the patient safely. I would then guide the learner to trace through the path of an oxygen molecule as it descends from the nostrils through the airways into the lungs, and the heart, etc. and ask the learner to imagine the ways that oxygen molecule can be diverted or sequestered. The learner would invariably be able to recall all the possible diagnoses and means of investigating them. These learners would often feel empowered by the fruits of their investigations yielding an esoteric diagnosis, but through a plan that invokes first principles. The same goes for teaching clinical research methodology. I would conceptualize projects in collaboration with the trainee to meet the trainee's own interest and engenders creativity. I provide trainees the opportunity to participate in a project from start to finish (protocol design, literature review, grant/proposal writing, drafting of research ethics board application and informed consent form writing, recruitment of subjects, knowledge dissemination including oral and poster presentations, and manuscript writing) but with ample of assistance from research personnel. I feel that it is important for trainees to experience the "big picture" of research rather than focus on one aspect of the study, such as data collection. Again, first principles and fundamentals are invoked in the teaching of research methodology. For example, I would reinforce the FINER principles (feasible, interesting, novel, ethical, relevant) with trainees during study conceptualization and design. It is my hope that this teaching philosophy will create learners that are flexible and adept at

tackling novel and unknown challenges in their future career.

In the July 2018 to June 2019 academic year I have received two feedback from the trainees pertaining to late start to review consults while on general hematology consult service. I have reflected on these comments and discussed ways of improving upon these feedback with my department head. The root cause to these issues were thought to be due to overcommitment to multiple clinical services simultaneously (e.g. seeing patients in clinics, the transfusion unit, and consult service at the same time), in addition to research commitments. Rebalancing of clinical workload has led to an improvement of teaching effectiveness scores (mean global teaching effectiveness score increased from 4.38 in 2018-2019 to 4.71 2019-2020 academic year).

D. Research Funding

1. GRANTS, CONTRACTS AND CLINICAL TRIALS

PEER-REVIEWED GRANTS

FUNDED

2024 Sep - 2026 Aug	Co-Principal Investigator. Thalassemia - REactive oXygen. Topconsortium voor Kennis en Innovatie (TKI) Life Sciences and Health. Public-Private Partnerships. HH-PPS-24028-MKB. 450,000 EUR. [Grants]
2024 Jul - 2027 Jun	Co-Investigator. Seeking Treatment for Pregnant and Postpartum/lactating Persons with OvaRies and SickLe Cell Disease (STORK): Establishing a Framework for Pharmacotherapeutic Research in SickLe Cell Disease Pregnancy to Modernize Development and Evaluation of FDA Regulated Products. The United States Food and Drug Administration. Center of Excellence in Regulatory Science and Innovation. PI: Pecker, Lydia. Collaborator(s): Kuo, Kevin H.M.; Malinowski, Ann Kinga; Farrell, Ruth M.; Sinkey, Rachel; Little, Jane; Saade, George. 250,000 USD. [Grants]
2023 Dec - 2026 Mar	Co-Principal Applicant. Prediction of Adverse Outcomes in Pregnancies of Individuals with SickLe Cell Disease: the SickLe Cell Risk in Pregnancy Tool - SCRIPT. MSH-UHN Academic Medical Organization. MSH-UHN AMO Innovation Fund Competition. 199,900 CAD. [Grants]
2023 Nov - 2024 Nov	Principal Applicant. Bringing the Diagnosis of Congenital Hemolytic Anemia to the 21st Century with RBC Deformability Testing. University Health Network. Blood Disorders Program Clinical Innovation Fund. PI: Kuo, Kevin . Collaborator(s): Barth D. 55,760 CAD. [Grants]
2020 Jul - 2023 Jun	Co-Principal Investigator. THromboprophylaxis In SickLe Cell Disease with Central Venous Catheters (THIS): A Pilot Study. Paul B Helliwell Foundation. Fund for Innovative Research in Non-Malignant Hematology. PI: Abdulrehman J, Kuo KHM . Collaborator(s): Forté S, Tomlinson G, Carrier M, Bartolucci P, Naessens V. 100,000 CAD. [Grants]
2020 Jun - 2023 May	Co-Principal Investigator. A Ph1b Trial of a Repurposed Therapeutic for Gene Modulation in beta-Hemoglobinopathies. National Heart, Lung, and Blood Institute (NHLBI) (USA). NHLBI Early Phase Clinical Trials (PAR-18-684). R33HL147845. PI: Faller DV, Kuo KHM , Perrine SP, Singer ST. 5,467,187 USD. [Grants]
2020 Jan - 2021 Dec	Co-Principal Investigator. Development and Validation of a Predictive Clinical Decision-Making Algorithm Using Artificial Intelligence (AI) for the Management of Patients with Iron Overload on Chelation Therapy. Paul B Helliwell Foundation. Fund for Innovative Research in Non-Malignant Hematology. PI: Jhaveri KS, Ward R, Kuo KHM . Collaborator(s): Mcintosh

C, Pendergrast J, Binding A. 189,182 CAD. [Grants]

- 2019 Jul - 2022 Jun **Principal Investigator.** A multicenter study evaluating the reliability and validity of the Rowland Universal Dementia Assessment Scale (RUDAS) and predictors of RUDAS performance in adults with sickle cell disease. Canadian Hematology Society. RK Smiley Research Grant. Collaborator(s): Soulières D, Forté S, Naessens V, Béland S, and Cohn M. 20,000 CAD. [Grants]
- 2019 Jul - 2022 Jun **Principal Investigator.** A multicenter study evaluating the reliability and validity of the Rowland Universal Dementia Assessment Scale (RUDAS) and predictors of RUDAS performance in adults with sickle cell disease. Pfizer Inc. (USA). 2019 US, Canada and Saudi Arabia SCD ASPIRE Research Award. Collaborator(s): Forté S, Soulières D, Ward R, Naessens V, Béland S, and Cohn M. 37,151.25 USD. [Grants]
- 2018 Dec - 2029 Dec **Principal Site Investigator.** Gene Transfer in Patients with Sickle Cell Disease Using a Gamma Globin Lentivirus Vector: An Open Label Phase I/II Pilot Study (SCGT). Doris Duke Charitable Foundation. PI: Malik, Punam. Collaborator(s): Parshad-Asnani, Monika; Knight-Madden, Jennifer; Kuo, Kevin H.M.; Quinn, Charles; Grimley, Michael. 165,324.24 USD. [Clinical Trials]
- 2018 Jul - 2019 Jun **Co-Principal Investigator.** Thalassemic Iron Overload Cardiomyopathy is Ameliorated by Taurine Supplementation. Peter Munk Cardiac Centre. Peter Munk Cardiac Centre Innovation Committee. PI: **Kuo KHM** and Sole M. Collaborator(s): Winterspberger B, Anderson GH, Billia P, Kublant R, Tomlinson G, Ward R, Farkouh M. 80,878 CAD. [Clinical Trials]
- 2016 Dec - 2017 Nov **Principal Investigator.** A Cross-Sectional Comparison of the Prevalence of Cerebral White Matter Hyperintensities in Adults with and without Sickle Cell Disease. University of Toronto. Alexandra Yeo Grant in Benign Hematology. Collaborator(s): Jaigobin C, Lacasse M-C, Mandel DM, Rawal S, Ward R. 19,939.66 CAD. [Clinical Trials]
- 2016 Jul - 2019 Jun **Principal Site Investigator.** Metabolomics biomarker profiles to predict cardiac disease in transfusion dependent beta thalassemia. Cooley's Anemia Foundation. Collaborator(s): Ward R, Pendergrast J. 3,356.25 USD. [Clinical Trials]
- 2013 Jan - 2019 Dec **Principal Investigator.** The use of the calcium channel blocker amlodipine as an adjuvant treatment to iron chelation for the prevention of iron overload cardiomyopathy in patients with thalassemia. Thalassemia Foundation of Canada. 410003159. Collaborator(s): Trivieri MG, Ward R, Kobulnik J, Ross H, Backx PH, Farkouh M, Thorpe K. 80,000 CAD. [Grants]
Hypothesis
1. The adjuvant use of amlodipine and chelation therapy with deferasirox (DFX) or deferiprone (DFP) will result in a significantly greater reduction in myocardial iron deposition as assessed by cardiac T2 magnetic resonance imaging after 1 year period of treatment compared to DFX or DFP monotherapy alone.*
2. Adjuvant therapy will be well tolerated, without an increase in the rates of adverse events.
Objectives
• To obtain data on the efficacy of amlodipine as adjuvant therapy to DFX or DFP in reducing cardiac iron overload as measured by cardiac T2 MRI in subjects with thalassemia and moderate iron overload (T2* <20 ms but ≥8 ms).*
• To detect lack of deterioration in LV function as assessed by MRI To determine the safety of amlodipine as adjuvant therapy to DFX or DFP for the treatment of subjects with thalassemia and moderate cardiac iron overload.
<https://www.clinicaltrials.gov/ct2/show/NCT02474420>.

NON-PEER-REVIEWED GRANTS

FUNDED

2023 Nov - 2026 Dec	Principal Site Investigator. An Adaptive, Randomized, Placebo-controlled, Double-blind, Multi-center Study of Oral Etavopivat, a Pyruvate Kinase Activator in Patients with Sickle Cell Disease. NovoNordisk. [Clinical Trials]
2023 Nov - 2025 Dec	Principal Site Investigator. A Phase 2 Open-Label Study to Evaluate Safety and Clinical Activity of FT-4202 in Patients with Thalassemia or Sickle Cell Disease. NovoNordisk. [Clinical Trials]
2023 Nov - 2024 Dec	Principal Site Investigator. An open-label, dose-escalation, dose-finding, and proof-of-concept trial of SP-420 in subjects with transfusion-dependent [beta]-thalassemia. Pharmacosmos. [Clinical Trials]
2023 May - 2025 Apr	Principal Site Investigator. A multicentre trial evaluating the efficacy and safety of oral decitabine-tetrahydouridine (NDec) in patients with sickle cell disease. NovoNordisk. [Clinical Trials]
2023 Jan - 2026 Dec	Principal Applicant. Safety and Efficacy of Mitapivat in Adult Patients with Erythrocyte Membranopathies (SATISFY-CA). Agios Pharmaceuticals. 499,000 CAD. [Industrial Grants]
2022 Dec - 2032 Dec	Global Principal Investigator. A Phase 3, Double-blind, Randomized, Placebo-Controlled, Multicenter Study Evaluating the Efficacy and Safety of Mitapivat in Subjects With Non-Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE). Agios Pharmaceuticals. [Clinical Trials]
2022 Feb - 2030 Feb	Principal Site Investigator. A Phase 3, Double-blind, Randomized, Placebo-Controlled, Multicenter Study Evaluating the Efficacy and Safety of Mitapivat in Subjects With Non-Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE). Agios Pharmaceuticals. [Clinical Trials]
2021 Dec - 2027 Jun	Site Investigator. A Phase III, Multicenter, Single-Arm Study Evaluating the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of Crovalimab in Adult and Adolescent Patients with Atypical Hemolytic Uremic Syndrome (aHUS). F. Hoffmann-La Roche Ltd. and Chugai Pharmaceutical Co. Ltd. [Clinical Trials]
2021 Nov - 2031 Jun	Principal Site Investigator. A Phase 3, Double-blind, Randomized, Placebo-Controlled, Multicenter Study Evaluating the Efficacy and Safety of Mitapivat in Subjects With Transfusion-Dependent Alpha- or Beta-Thalassemia (ENERGIZE-T). Agios Pharmaceuticals. [Clinical Trials]
2021 Oct - 2033 Sep	Principal Site Investigator. A Long-term Follow-up Study of Subjects With β -thalassemia or Sickle Cell Disease Treated with Autologous CRISPR-Cas9 Modified Hematopoietic Stem Cells (CTX001). Vertex Pharmaceuticals Incorporated. [Clinical Trials]
2020 Nov - 2023 Dec	Site Investigator. Danicopan as Add-on Therapy to a C5 Inhibitor in Paroxysmal Nocturnal Hemoglobinuria (PNH) Participants Who Have Clinically Evident Extravascular Hemolysis (EVH)(ALPHA). Alexion Pharmaceuticals Inc. PI: Patriquin, Christopher. [Clinical Trials]
2020 Feb - 2025 Jan	Principal Site Investigator. A Phase 3b, open-label, single-arm, rollover study to evaluate long-term safety in subjects who have participated in other luspatercept (ACE-536) clinical trials. Celgene Corporation. Collaborator(s): Ward R, Patriquin C, Pendergrast J. [Clinical Trials]

2020 Jan - 2030 Dec	Site Investigator. A Phase 1/2 Study to Evaluate the Safety and Efficacy of a Single Dose of Autologous CRISPR-Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (CTX001) in Subjects With Severe Sick Cell Disease. Vertex Pharmaceuticals Incorporated. [Clinical Trials]
2019 Jan - 2029 Dec	Site Investigator. Phase 1/2 Study of the Safety and Efficacy of a Single Dose of Autologous CRISPR-Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (hHSPCs) in subjects with Transfusion-Dependent β -Thalassemia. Vertex Pharmaceuticals Incorporated. [Clinical Trials]
2018 Nov - 2021 Oct	Principal Site Investigator. A Phase 2, Open-Label, Multicenter Study to Determine the Efficacy, Safety, Pharmacokinetics, and Pharmacodynamics of AG-348 in Adult Subjects With Non-Transfusion-Dependent Thalassemia. Agios Pharmaceuticals. Collaborator(s): Layton DM, Lal A, Al-Samkari H, Tai F, Lynch M, Uhlig K, Vichinsky EP. [Clinical Trials]
2018 Aug - 2020 Jul	Principal Site Investigator. A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of AG-348 in Not Regularly Transfused Adult Subjects with Pyruvate Kinase Deficiency. Agios Pharmaceuticals. Collaborator(s): Ward R, Patriquin C, Pendergrast J. [Clinical Trials]
2018 Jul - 2020 Jun	Principal Site Investigator. An Open-Label Study to Evaluate the Efficacy and Safety of AG-348 in Regularly Transfused Adult Subjects with Pyruvate Kinase (PK) Deficiency. Agios Pharmaceuticals. Collaborator(s): Ward R, Patriquin C, Pendergrast J. [Clinical Trials]
2018 Apr - 2027 Sep	Principal Site Investigator. Pyruvate Kinase Deficiency Global Longitudinal Registry. Agios Pharmaceuticals. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials] <i>International, Multicentre, study.</i>
2017 Nov - 2022 Oct	Principal Site Investigator. A Phase 3, Double-blind, Randomized, Placebo-controlled, Multicenter Study of GBT440 Administered Orally to Patients With Sick Cell Disease. Global Blood Therapeutics. [Clinical Trials]
2017 Oct - 2020 Mar	Principal Site Investigator. A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Rivipansel (GMI-1070) in the Treatment of Vaso-Occlusive Crisis in Hospitalized Subjects with Sick Cell Disease. Pfizer. [Clinical Trials]
2017 Jul - 2021 Jun	Principal Site Investigator. A Phase 3, Randomized, Open-Label, Active-Controlled Study of ALXN1210 Versus Eculizumab in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) Currently Treated With Eculizumab. Alexion Pharmaceuticals Inc. Collaborator(s): Patriquin C. [Clinical Trials]
2016 Dec - 2018 Jan	Principal Site Investigator. An Observational, Non-Interventional, Multi-Center, Multi-National Study of Patients with Atypical Hemolytic-Uremic Syndrome (aHUS Registry). Alexion Pharmaceuticals Inc. Collaborator(s): Patriquin C. [Clinical Trials]
2016 Sep - 2019 Aug	Co-Principal Investigator. A Study of Oral Hemokine Stimulants in Blood Cell Deficiency. Phoenicia Biosciences. PI: Perrine SP. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
2016 Jun - 2021 May	Principal Site Investigator. A Phase 3, double-blind, randomized, placebo-controlled, multicenter study to determine the efficacy and safety of luspatercept (ACE-536) plus best supportive care versus best supportive care in adults who require regular red-blood cell

transfusions due to beta-thalassemia. Celgene Corporation. Collaborator(s): Ward R, Patriquin C, Pendergrast J. [Clinical Trials]

2016 Jan - 2016 Dec

Principal Site Investigator. An Observational, Multi-Center, Multi-National, Long Term Follow-Up Study of Atypical Hemolytic Uremic Syndrome (aHUS) Patients Treated with Eculizumab in a Prior Clinical Study. Alexion Pharmaceuticals Inc. [Clinical Trials]

2015 Jun - 2025 Dec

Principal Site Investigator. Paroxysmal Nocturnal Hemoglobinuria (PNH) Registry. Alexion Pharmaceuticals. Collaborator(s): Patriquin, C, Ward R. [Clinical Trials]
International, Multicentre, study.

2015 Jun - 2018 Oct

Principal Site Investigator. A Phase 2, Open Label, Randomized, Dose Ranging, Safety, Efficacy, Pharmacokinetic and Pharmacodynamic Study of AG-348 in Adult Patients with Pyruvate Kinase Deficiency. Agios Pharmaceuticals. Collaborator(s): Ward R, Patriquin C. [Clinical Trials]

2015 Jan - 2015 Oct

Principal Site Investigator. Safety and Pharmacokinetic Study of Escalating Doses of SP-420, an Iron Chelator, in Patients With β -Thalassemia. Sideris Pharmaceuticals. 410003654. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study
<https://www.clinicaltrials.gov/ct2/show/NCT02274233>.

2014 Dec - 2020 May

Principal Site Investigator. Pyruvate Kinase Deficiency Natural History Study. Boston Children's Hospital. 410003655. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study.

2014 Dec - 2016 Jun

Principal Site Investigator. A Phase III Double Blind Clinical Trial to Study the Efficacy and Safety of the Combination Regimen of MK-5172 and MK-8742 in Subjects with Chronic HCV GT1, GT4 and GT6 Infection with Inherited Blood Disorders with and without HIV Co-Infection. Merck Canada. 410003654. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study.

2013 Aug - 2014 Sep

Site Investigator. A Phase 2, Open-label, Multicentre, Extension Safety and Tolerability Study for Transfusionally Iron Overloaded Children, Adolescents and Adults Using SSP-004184 (SPD602). Shire Pharmaceuticals Group. 410000893. PI: Ward R. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study.

2012 Oct - 2016 Sep

Principal Investigator. The use of the calcium channel blocker amlodipine as an adjuvant treatment to iron chelation for the prevention of iron overload cardiomyopathy in patients with thalassemia. Peter Munk Cardiac Centre Foundation. 410003159. Collaborator(s): Trivieri MG, Ward R, Ross H, Backx PH, Farkouh M, Thorpe K. 30,000 CAD. [Grants]
<https://www.clinicaltrials.gov/ct2/show/NCT02474420>.

2012 Oct - 2014 Aug

Site Investigator. FBS0701-CTP-16: A Phase 2, 24 Week, Randomized, Open Label, Multi-Center Study to Assess the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Two FBS0701 Doses in the Treatment of Chronic Iron Overload Requiring Chelation Therapy. Ferrokin Biosciences/Shire Pharmaceuticals. 410000893. PI: Ward R. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study.

2012 Aug - 2014 Jan

Site Investigator. A Randomised, Placebo-controlled, Phase 2 study of HQK-1001 in Sickle Cell Disease (HQP 1001-SCD-007). Hemaquest Pharmaceuticals Inc. PI: Ward R. Collaborator(s): Ward R, Pendergrast J. [Clinical Trials]
International, Multicentre, study.

2004 - 2006

Co-Investigator. Treatment-related changes in markers of neurodegeneration over time in the cerebrospinal fluid of adults with haematologic malignancies receiving chemotherapy. Lawson Health Research Institute. Internal Research Fund. PI: Xenocostas, A. Collaborator(s): Rylett RJ, Pasternak SH, **Kuo KHM**. 7,500 CAD. [Grants]

2. SALARY SUPPORT AND OTHER FUNDING

Personal Salary Support

2020 Jun - 2023 May

Development of a Clinical Hemoglobin Modulator. National Heart, Lung and Blood Institute. 170,100 USD. United States.

E. Publications

1. MOST SIGNIFICANT PUBLICATIONS

1. Taher AT, Al-Samkari H, Aydinok Y, Besser M, Boscoe AN, Dahlin JL, De Luna G, Estep JH, Gheuens S, Gilroy KS, Glenthøj A, Sim Goh A, Iyer V, Kattamis A, Loggetto SR, Morris S, Musallam KM, Osman K, Ricchi P, Salido-Fierrez E, Sheth S, Tai F, Tevich H, Uhlig K, Urbstonaitis R, Viprakasit V, Cappellini MD, **Kuo KHM**, ENERGIZE investigators. Mitapivat in adults with non-transfusion-dependent α -thalassaemia or β -thalassaemia (ENERGIZE): a phase 3, international, randomised, double-blind, placebo-controlled trial. Lancet. 2025 Jul 5;406(10498):33-42. Impact Factor 88.5. **Senior Responsible Author.**

The phase 3 randomised controlled trial validated the findings from the phase 2 study. As the global principal investigator in the study, I was instrumental in the design and execution of the trial.

2. **Kuo KHM**, Layton DM, Lal A, Vichinsky EP, Dahlin JL, Shen S, Price GM, Gilroy KS, Estep JH, Al-Samkari H. Long-term efficacy and safety of mitapivat in non-transfusion-dependent α - or β -thalassaemia: An open-label phase 2 study. Br J Haematol. 2025 Jun 1;206(6):1764-1773. Impact Factor 3.8. **Principal Author.**

This long-term follow up of the phase 2 study further confirmed the long term efficacy of mitapivat, a pyruvate kinase activator, in patients with non-transfusion dependent thalassemia.

3. **Kuo KHM**, Layton DM, Lal A, Al-Samkari H, Bhatia J, Kosinski PA, Tong B, Lynch M, Uhlig K, Vichinsky EP. Safety and efficacy of mitapivat, an oral pyruvate kinase activator, in adults with non-transfusion dependent α -thalassaemia or β -thalassaemia: an open-label, multicentre, phase 2 study. Lancet. 2022 Aug 13;400(10351):493-501. Impact Factor 202.7. **Principal Author.**

The phase 2 study of mitapivat, a pyruvate kinase activator, in patients with non-transfusion dependent thalassemia established safety and efficacy.

4. Malinowski AK, **Kuo KHM**, Tomlinson GA, Palcu P, Ward R, Shehata N. Distinct maternal and fetal pregnancy outcomes in women with sickle cell disease can be predicted using routine clinical and laboratory data. Br J Haematol. 2021 Sep 1;194(6):1063-1073. Impact Factor 5.5. **Co-Principal Author.**

(A.K.M. and K.H.M.K contributed equally and share first authorship)

Our study created three risk models that predicted adverse maternal and fetal outcomes in pregnant women with sickle cell disease with moderate discrimination. These prediction rules enable identification of women with SCD at high-risk of adverse events, allowing for consideration of targeted interventions.

5. Forté S, Blais F, Castonguay M, Fadiga N, Fortier-St-Pierre M, Couette M, Ward R, Béland S, Cohn M, Soulières D, **Kuo KHM**. Screening for Cognitive Dysfunction Using the Rowland Universal Dementia Assessment Scale in Adults With Sickle Cell Disease. JAMA Netw Open. 2021 May 3;4(5):e217039. Impact Factor 5. **Senior Responsible Author**.

The study has shown that a large portion of sickle cell disease (SCD) patients have suspected dementia, and that renal dysfunction and reticulocyte count are independent risk factors of dementia. Results from the study informed the feasibility of using Rowland Universal Dementia Assessment Scale as a rapid and easily to administer neurocognitive test to screen for possible mild cognitive impairment and dementia in SCD patients. It was also hypothesis-generating, that anemia-induced tissue hypoxia may be a modifiable risk factor for cerebral and renal injury and dysfunction in SCD patients.

6. Binding A, Ward R, Tomlinson G, **Kuo KHM**. Deferiprone exerts a dose-dependent reduction of liver iron in adults with iron overload. European Journal of Haematology. 2019 May 8. Impact Factor 2.22. **Senior Responsible Author**.

I have shown that hepatic iron removal is most effective at an actual dose of >100 mg/kg/day, and that the frequency and type of side-effect from the iron chelator deferiprone is not dose-related. This finding will allow clinicians to make more informed decisions when considering dose-escalation of deferiprone.

7. Allemang B, Allan K, Johnson C, Cheong M, Cheung P, Odame I, Ward R, Williams S, Mukerji G, **Kuo KHM**. Impact of a transition program with navigator on loss to follow-up, medication adherence, and appointment attendance in hemoglobinopathies. Pediatric Blood and Cancer. 2019 May 2:e27781. Impact Factor 2.355. **Senior Responsible Author**.

Development and validation of a structured comprehensive transition program with dedicated transition navigator. Also first study to incorporate clinically relevant outcomes in the assessment of a novel comprehensive transition program. The transition navigator role in hemoglobinopathies has been designated as a Leading Practice by the Health Standards Organization.

8. **Kuo KH**, Ward R, Kaya B, Howard J, Telfer P. A comparison of chronic manual and automated red blood cell exchange transfusion in sickle cell disease patients. British Journal of Haematology. 2015 Jan 27. Impact Factor 5.5. **Principal Author**.

My research on the comparative effectiveness of partial manual and automated RBC exchange transfusion (RBCX) has been utilized in the synthesis of a Health Technology Assessment report by the National Institute for Health and Care Excellence (NICE) in the UK and a guideline by the British Society of Haematology (Spectra Optia for automatic red blood cell exchange in patients with sickle cell disease. NICE Medical technologies guidance (2016) [MTG28]; Guidelines on red cell transfusion in sickle cell disease. Part I: principles and laboratory aspects).

2. PEER-REVIEWED PUBLICATIONS

Journal Articles

1. Vlachodimitropoulou E, Balasubramaniam T, Shehata N, Ward R, **Kuo KHM**, Kingdom JC, Malinowski AK. Utility of placental growth factor for preeclampsia prediction in pregnancies complicated by sickle cell disease. Blood Adv. 2026 Jan 27;10(2):402-412. **Coauthor or Collaborator**.
2. Lilly L, Udeze C, Nightingale N, Wang I, Murray J, Penaranda C, Neish CS, Li N, **Kuo KHM**. Clinical Complications, Mortality, and Healthcare Resource Utilization of Patients with Sickle Cell Disease with Recurrent Vaso-Occlusive Crises in Ontario, Canada: A Retrospective Cohort Study. Adv Ther. 2026 Jan 1;43(1):271-287. **Senior Responsible Author**.
3. Musallam KM, Sheth S, Cappellini MD, Shah F, Rivella S, Sankaran VG, **Kuo KHM**, Viprakasit V, Eleftheriou A, Angastiniotis M, Locatelli F, Taher AT. Management of transfusion-dependent β -thalassaemia in the era of novel therapies: a prioritisation-based matrix for settings with limited resources. Lancet Haematol. 2026 Jan 1;13(1):e49-e54. **Coauthor or Collaborator**.

4. Sharma A, Locatelli F, Bhatia M, Molinari L, Mapara MY, Liem RI, Dedeken L, Wall D, Eckrich MJ, **Kuo KHM**, Smith W, Imren S, Kohli P, Li N, Liu T, Rubin J, Hobbs W, Grupp SA, Frangoul H. Improvements in health-related quality of life in patients with severe sickle cell disease after exagamglogene autotemcel. *Blood Adv.* 2025 Dec 23;9(24):6481-6490. **Coauthor or Collaborator.**
5. Fuente JDL, Frangoul H, Lang P, Wall D, Meisel R, Corbacioglu S, Li AM, Shah AJ, Carpenter B, Kwiatkowski JL, Mapara MY, Liem RI, Rupprecht J, **Kuo KHM**, Merkeley H, Algeri M, Smith W, Kohli P, Li N, Rubin J, Zhang S, Hobbs W, Locatelli F. Improvements in health-related quality of life in patients with transfusion-dependent β -thalassemia after exagamglogene autotemcel. *Blood Adv.* 2025 Dec 23;9(24):6502-6510. **Coauthor or Collaborator.**
6. Abdulrehman J, Forté S, Tomlinson G, Solh Z, Bolster L, Sun H, **Kuo KHM**. Challenges in Trials in Sickle Cell Disease: Thromboprophylaxis in Sickle Cell Disease With Central Venous Catheters (THIS) Pilot Study. *J Clin Apher.* 2025 Dec 1;40(6). **Senior Responsible Author.**
7. de la Fuente J, Frangoul H, Locatelli F, Lang P, Wall D, Sharma A, Meisel R, Corbacioglu S, Mapara M, Bhatia M, Molinari L, Liem RI, Dedeken L, Li AM, Shah AJ, Algeri M, Carpenter B, Kwiatkowski JL, Eckrich MJ, Rupprecht J, Merkeley H, **Kuo KHM**, Boerner K, Zhang S, Zairis S, Liu T, Imren S, Hobbs W, Grupp SA. Durable clinical benefits with exagamglogene autotemcel for transfusion-dependent thalassemia and severe sickle cell disease. *Br J Haematol.* 2025 Oct;207:S19-S20. **Coauthor or Collaborator.**
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59. **Kuo KH**, Kovacs MJ. Fondaparinux: a potential new therapy for HIT. *Hematology*. 2005 Aug 1;10(4):271-5. **Principal Author**.
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Book Chapters

1. Taher AT, Farmakis D, Porter JB, Cappellini MD, Musallam KM, Musallam KM, Sheth S, Cappellini MD, **Kuo KHM**, Rivella S, Maggio A, Viprakasit V, Taher AT. NOVEL DISEASE-MODIFYING AGENTS. In: Taher AT, Farmakis D, Porter JB, Cappellini MD, Musallam KM, editor(s). Guidelines for the Management of Transfusion-Dependent β -Thalassaemia (TDT). 5th edition. Nicosia (Cyprus): Thalassaemia International Federation; 2025. **Coauthor or Collaborator**.
2. **Kuo KHM** and Merrill SA. Hemolytic Anemias Excluding Hemoglobinopathies. In: American Society of Hematology Self-Assessment Program. 8. (United States); 2022. In Press. **Principal Author**.
3. Go RS and **Kuo KHM**. Hemolytic Anemias Excluding Hemoglobinopathies. In: Cuker A, editor(s). American Society of Hematology Self-Assessment Program. 7. (United States); 2018. **Co-Principal Author**.

Editorials

1. Musallam KM, Sheth S, Cappellini MD, **Kuo KHM**, Kattamis A, Aydinok Y, Viprakasit V, Taher AT. Luspatercept versus mitapivat for non-transfusion-dependent β -thalassemia: Dare to compare? Hemasphere. 2025 Jul 1;9(7):e70165. **Coauthor or Collaborator**.
2. Musallam KM, Sheth S, Cappellini MD, Kattamis A, **Kuo KHM**, Taher AT. Luspatercept for transfusion-dependent β -thalassemia: time to get real. Ther Adv Hematol. 2023 Jan 1;14:20406207231195594. **Coauthor or Collaborator**.
3. Musallam KM, Taher AT, Cappellini MD, Hermine O, **Kuo KHM**, Sheth S, Viprakasit V, Porter JB. Untreated Anemia in Nontransfusion-dependent β -thalassemia: Time to Sound the Alarm. Hemasphere. 2022 Nov 15;6(12):e806. **Coauthor or Collaborator**.

Letters to Editor

1. **Kuo KHM**, Grace RF, van Beers EJ, Barcellini W, Glenthøj A, Holzhauer S, Beynon V, Morris S, Li J, Zagadailov E, Patel P, Al-Samkari H. Clinically meaningful improvements in patient-reported outcomes in mitapivat-treated patients with pyruvate kinase deficiency. Am J Hematol. 2024 Jul 1;99(7):1415-1419. **Principal Author**.
2. Kountouris P, Stephanou C, Archer N, Bonifazi F, Giannuzzi V, **Kuo KHM**, Maggio A, Makani J, Mañú-Pereira MDM, Michailidou K, Nkya S, Nnodu OE, Trompeter S, Tshilolo L, Wonkam A, Zifalil BA, Inusa BPD, Kleanthous M, on behalf of the International Hemoglobinopathy Research Network (INHERENT). The International Hemoglobinopathy Research Network (INHERENT): An international initiative to study the role of genetic modifiers in hemoglobinopathies. Am J Hematol. 2021 Nov 1;96(11):E416-E420. **Coauthor or Collaborator**.
3. Al-Samkari H, van Beers EJ, Morton DH, Barcellini W, Eber SW, Glader B, Yaish HM, Chonat S, **Kuo KHM**, Kollmar N, Despotovic JM, Pospíšilová D, Knoll CM, Kwiatkowski JL, Pastore YD, Thompson AA, Wlodarski MW, Ravindranath Y, Rothman JA, Wang H, Holzhauer S, Breakey VR, Verhovsek MM, Kunz J, Sheth S, Sharma M, Rose MJ, Bradeen HA, McNaull MN, Addonizio K, Al-Sayegh H, London WB, Grace RF. Characterization of the severe phenotype of pyruvate kinase deficiency. Am J Hematol. 2020 Jul 3. Impact Factor 6.973. **Coauthor or Collaborator**.
4. Aneke J, Barth D, Ward R, Pendergrast J, **Kuo K**, Cserti-Gazdewich C. The rationale for abandoning sickle trait screening of red blood cell units for patients with sickle cell disease. Transfusion Medicine. 2019 May 16. Impact Factor 2.159. **Coauthor or Collaborator**.
5. Malinowski AK, Parrish J, Shehata N, Ward R, **Kuo KHM**. Approach to transfusion in pregnant women with sickle cell disease: a survey of physicians. Br J Haematol. 2018 Nov 1;183(3):516-519. Impact Factor 5.5. **Coauthor or Collaborator**.

6. van Beers EJ, van Straaten S, Morton DH, Barcellini W, Eber SW, Glader B, Yaish HM, Chonat S, Kwiatkowski JL, Rothman JA, Sharma M, Neufeld EJ, Sheth S, Despotovic JM, Kollmar N, Pospíšilová D, Knoll CM, **Kuo K**, Pastore YD, Thompson AA, Newburger PE, Ravindranath Y, Wang WC, Wlodarski MW, Wang H, Holzhauer S, Breakey VR, Verhovsek M, Kunz J, McNaull MA, Rose MJ, Bradeen HA, Addonizio K, Li A, Al-Sayegh H, London WB, Grace RF. Prevalence and management of iron overload in pyruvate kinase deficiency: report from the Pyruvate Kinase Deficiency Natural History Study. *Haematologica*. 2018 Sep 13;104(2):e51-e53. Impact Factor 7.116. **Coauthor or Collaborator**.
7. Malinowski AK, Cheung P, Yang J, D'Souza R, Shehata N, Ward R, **Kuo KHM**, Murphy K. Cord gas parameters in infants born to women with sickle cell disease: a retrospective matched cohort study. *Br J Haematol*. 2018 Feb 22;184(4):653-657. Impact Factor 5.5. **Coauthor or Collaborator**.
8. Naessens V, Ward R, **Kuo KH**. A proposed treatment algorithm for adults with Haemoglobin SC disease. *British Journal of Haematology*. 2017 Jul 12. Impact Factor 5.5. **Senior Responsible Author**.
9. **Kuo KH**, Ward R, Kaya B, Howard J, Telfer P. A comparison of chronic manual and automated red blood cell exchange transfusion in sickle cell disease patients. *British Journal of Haematology*. 2015 Jan 27. Impact Factor 5.5. **Principal Author**.

Brief Communication

1. Tole S, Amid A, Baker J, **Kuo K**, Pugi J, Carcao M. Mild Hereditary Spherocytosis without Accompanying Hereditary Haemochromatosis: An Unrecognised Cause of Iron Overload. *Acta Haematologica*. 2019 Apr 9;141(4):256-260. Impact Factor 1.47. **Coauthor or Collaborator**.

3. NON-PEER-REVIEWED PUBLICATIONS

Journal Articles

1. **Kuo K**, Dennis R. The Modern Era of Transfusion Medicine. *The Hematologist*. 2011;8(5):14.
2. **Kuo K**, Palmeri M, Woyach J. The Perpetual Fellow — Extending Fellowship Training in Hematology: The Trainee Perspective. *The Hematologist*. 2011;8(2):2.
3. Heerema T, **Kuo K**, Lee P, Lim R, Rader J, Wu H. "Born to be a Burden" A dramatic way to educate medical students about the ethics of prenatal screening. *The University of Western Ontario Medical Journal*. 2005;73(1):1. **Coauthor or Collaborator**.

Multimedia

1. Career-Development Timeline. American Society of Hematology career planning website (<http://www.hematology.org/Training/Trainees/7113.aspx>).

Clinical Care Guidelines

1. Canadian Haemoglobinopathy Association. Consensus Statement on the Care of Patients with Sickle Cell Disease in Canada. Ottawa (Canada); 2014. **Reviewer**.

F. Intellectual Property

1. DISCLOSURES

- 2015 Jul **Partial manual red blood cell exchange transfusion using the Alyx component collection system.** Applied, Filing Date: 2017 Feb 11. Disclosures #: TDC reference #2017-006, Ontario, Canada.
Partial manual along with automated red blood cell exchange transfusion (RBCX) are the currently accepted methods to treat or prevent multisystem complications in patients with sickle cell disease (SCD) but each has its own drawbacks (Kuo et al. 2015). I proposed the use of the Alyx component collection system for the phlebotomy phase of the partial manual RBCX procedure to overcome these existing barriers. The expected advantages derived from this modified method of partial manual RBCX include:
1. maintaining euvolemia and avoid complications such as hypotension and dizziness, directly reducing the number of aborted procedures
2. reduction in the time required to perform the phlebotomy phase of the procedure from 2 hours to 30 minutes with the use of the Alyx
3. reduced nursing resource and ancillary cost to the health care system.
- 2015 Jul **Tissue Iron Overload is Ameliorated by Taurine Supplementation.** Applied, Filing Date: 2016 Sep 13. Disclosures #: UHN TDC IP Disclosure Reference No. 2018-055, Ontario, Canada. Joint Holder Name(s): Sole, Michael; Kuo, Kevin H.M.
An inevitable complication of chronic hemolysis is iron overload. Current management is limited to iron chelators, all of which have significant toxicity and side-effect profile, and are major barriers in establishing good adherence. Taurine exhibits anti-oxidant effect and reduce cardiac and hepatic iron overload in animal models. Unlike iron chelators, taurine is a nutritional supplement generally regarded as safe (GRAS). We propose to use taurine by itself or concurrently with an iron chelator to reduce transfusion-dependent or hemolysis associated iron overload.

G. Presentations and Special Lectures

1. INTERNATIONAL

Invited Lectures and Presentations

- 2024 May 19 **Invited Speaker.** 1st RAIN Summit for Patients with Rare Anaemias. Rare Anaemias International Network (RAIN). Bucharest, Bucuresti, Romania.
- 2024 May 17 **Invited Speaker.** Capacity Building Workshop for Thalassemia Patients and Healthcare Professionals. Thalassemia International Federation. Bucharest, Bucuresti, Romania.
- 2024 Apr 19 **Invited Speaker.** ASH Highlights of ASH Latin America - Hemoglobinopathies. American Society of Hematology and Colombian Association of Hematology and Oncology (ACHO). Medellin, Antioquia, Colombia.
- 2024 Mar 2 **Invited Speaker.** ASH Highlights of ASH Mediterranean - Hemoglobinopathies. American Society of Hematology and Emirates Society of Haematology. Dubai, Dubayy [Dubai], United Arab Emirates.
- 2024 Jan 26 **Invited Speaker.** 3rd Inherited Blood Disorders Summit (IHBD). Ministry of Health of the Kingdom of Saudi Arabia. Riyadh, Not Required, Saudi Arabia.
- 2024 Jan 19 **Invited Speaker.** ASH Highlights of ASH North America - Hemoglobinopathies. American Society of Hematology. Chicago, Illinois, United States.
- 2024 Jan 12 **Invited Speaker.** ASH Highlights of ASH North America - Hemoglobinopathies. American Society of Hematology. Seattle, Washington, United States.

- 2023 Dec 11 **Invited Speaker.** Pyruvate Kinase Activators: Targeting Red Cell Metabolism in Thalassemia. American Society of Hematology. San Diego, California, United States.
- 2023 Dec 10 **Moderator.** 112. Thalassemia and Globin Gene Regulation: Finding New Pathways to Treat Thalassemia. American Society of Hematology. San Diego, California, United States.
- 2023 Jun 29 **Presenter.** Cases in α -Thalassemia: What Is the Role of Primary Care? Medscape. United States. Available from: <https://www.medscape.org/viewarticle/993764>.
- 2023 May 8 **Invited Speaker.** Novel Treatment Targets and Agents in Alpha Thalassemia. Fondazione Franco e Piera Cutino. Palermo, Sicilia, Italy.
- 2023 Mar 23 **Invited Lecturer.** The Known Knowns, _Known Unknowns, and _Unknown Unknowns _in α -Thalassemia. European Hematology Association and the Lebanese Society of Hematology and Blood Transfusion. EHA-LSHBT Virtual Hematology Updates 2022-2023 - HU7. Alpha Thalassemia.
- 2023 Mar 5 **Invited Speaker.** Clinical Application of Pyruvate Kinase Activation. European School of Hematology. Paris, Paris, France. ESH 3rd Translational Research Conference: Erythropoiesis Control and Ineffective Erythropoiesis.
- 2022 Aug 17 **Lecturer.** Strategies to Manage Transfusional Iron Overload in Patients with beta-Hemoglobinopathies: Optimizing Patient Outcomes. Physicians' Education Resource, LLC. United States.
- 2022 Jan 28 **Presenter.** Best of Hematology Conference 2022 - Sickle Cell Disease: Updates from ASH. OncologyEducation. Canada. Best of Hematology Conference 2022 (Digital Meeting).
- 2022 Jan 28 **Invited Speaker.** The Promise of Novel Pharmacological Agents in Thalassaemia. The Annual Scientific Conference on Sickle Cell and Thalassaemia (ASCAT). London, City of, United Kingdom.
- 2021 Nov 26 **Invited Speaker.** Novel Therapies in Thalassemia. 2nd Saudi Sickle Cell Disease and Thalassemia Summit. Jeddah, Makkah, Saudi Arabia.
- 2021 Nov 24 **Invited Speaker.** Evidence and Guideline Based Management of Sickle Cell Disease Complications. 2nd Saudi Sickle Cell Disease and Thalassemia Summit. Jeddah, Makkah, Saudi Arabia.
- 2020 Oct 23 **Invited Speaker.** How can we access preventive and risk-reduction therapies: examples from rare blood disorders. Canadian Organization for Rare Disorders. Canada. Available from: <https://www.slideshare.net/raredisorders/preventive-and-riskreduction-therapies-examples-from-rare-blood-disorders>.
- 2020 Aug 11 **Chair.** Walking Down Multiple Yellow Brick Roads – Evolving Paradigms of Care in Sickle Cell Disease. Sickle Cell Adult Provider Network / National Heart, Lung and Blood Institutes / National Institutes of Health. Bethesda, Maryland, United States. Presenter(s): Samir Ballas, Swee Lay Thein, Lakshmanan Krishnamurti, Julie Kanter, Jane Little, Mark Gladwin, Sophie Lanzkron, Fuad El Rassi, Payal Desai, Jane Kwiatkowski, Danny Dalton, Charity Oyedeki, Kelli Newman, Brenda Adimora, Michael Feast, Celeste Barton.
- 2017 Dec 9 **Chair.** Health Services Research: Non-Malignant Conditions: Sickle Cell Disease. American Society of Hematology. Atlanta, Georgia, United States.
- 2015 Aug 20 **Presenter.** Results from a survey of practice patterns in the treatment of osteonecrosis of the hip amongst US and Canadian hematologists. Sickle Cell Adult Provider Network, National Heart Lung and Blood Institute, National Institute of Health. Bethesda, Maryland, United States. Presenter(s): Kevin H.M. **Kuo**.

Presented Abstracts

- 2011 The Effect of Comprehensive Care on Maternal and Fetal Outcomes in Sickle Cell Disease Pregnancies. 53rd annual meeting of the American Society of Hematology. **Kuo KHM**, Warner E, Sermer M, Ward R. Abstract #4842 (Online publication).

- 2011 Application of Self-Efficacy Theory in Adherence to Iron Chelation Therapy: A Single-Center Cross-Sectional Study. 53rd annual meeting of the American Society of Hematology. **Kuo KHM**, Ward R. Abstract #5284 (Online publication).
- 2011 The Effect of Comprehensive Care and Degree of Iron Overload on Maternal and Fetal Outcomes in Pregnancies of Transfusion-Dependent Beta-Thalassemia Patients. 53rd annual meeting of the American Society of Hematology. **Kuo KHM**, Warner E, Sermer M, Ward R. Abstract #5297 (Online publication).
- 2011 The Relationship Between Pre-Pregnancy Sickle Cell Disease-Specific Complications and Maternal and Fetal Outcomes in Sickle Cell Disease Pregnancies. 53rd annual meeting of the American Society of Hematology. Mulla-Ali DH, **Kuo KHM**, Sermer M, Mcleod A. Abstract #4848 (Online publication).

Presented and Published Abstracts

- 2024 Jun 13 **Presenter.** Characterizing patterns of transfusion burden (TB) reduction in patients (pts) with transfusion-dependent (TD) beta-thalassemia treated with luspatercept in the BELIEVE trial. European Hematology Association. Madrid, Madrid, Spain. Presenter(s): **Kuo KHM**, Kattamis A, Taher AT, Motta I, Cappellini MD, Musallam KM, Bueno LM, Perin M, Medlin LF, Martin-Regueira P, Lai Y, Ugidos M, Sujit S.

Publication Details:

Kuo KHM, Kattamis A, Taher AT, Motta I, Cappellini MD, Musallam KM, Bueno LM, Perin M, Medlin LF, Martin-Regueira P, Lai Y, Ugidos M, Sujit S. Characterizing patterns of transfusion burden (TB) reduction in patients (pts) with transfusion-dependent (TD) beta-thalassemia treated with luspatercept in the BELIEVE trial. 2024 May 14. **Principal Author.**

- 2024 Jun 13 **Presenter.** Improvements in fatigue and 6-minute walk test in adults with alpha- or beta-non-transfusion-dependent thalassemia: the phase 3 ENERGIZE trial of mitapivat. European Hematology Association. Madrid, Madrid, Spain. Presenter(s): **Kuo KHM**, Al-Samkari H, Aydinok Y, Besser M, Boscoe A, De luna G, Estepp JH, Gheuens S, Gilroy KS, Glenthøj A, Goh AS, Kattamis A, Loggetto SR, Morris S, Musallam KM, Osman K, Ricchi P, Salido-Fiérrez E, Sheth S, Tai F, Uhlig K, Viprakasit V, Cappellini MD, Taher AT.

Publication Details:

Kuo KHM, Al-Samkari H, Aydinok Y, Besser M, Boscoe A, De luna G, Estepp JH, Gheuens S, Gilroy KS, Glenthøj A, Goh AS, Kattamis A, Loggetto SR, Morris S, Musallam KM, Osman K, Ricchi P, Salido-Fiérrez E, Sheth S, Tai F, Uhlig K, Viprakasit V, Cappellini MD, Taher AT. Improvements in fatigue and 6-minute walk test in adults with alpha- or beta-non-transfusion-dependent thalassemia: the phase 3 ENERGIZE trial of mitapivat. 2024 May 14. **Principal Author.**

- 2023 Dec 11 **Presenter.** Efficacy and Safety of Luspatercept in Patients with HbE/β Thalassemia from the BELIEVE Study: A Subgroup Analysis. American Society of Hematology. San Diego, California, United States.

Publication Details:

Kuo KHM, Sutcharitchan P, Bee PC, Chew LP, Lim SM, Goh AS, Vodala S, Kuo W-L, Lai Y, Medlin LF, Perin M, Bueno LM, Coates TD, Viprakasit V. Efficacy and Safety of Luspatercept in Patients with HbE/β Thalassemia from the BELIEVE Study: A Subgroup Analysis. Blood. 2023 Nov 28;142(Supplement 1):3848. **Principal Author.**

- 2023 Dec 11 Efficacy and Safety of Luspatercept in Patients Enrolled in the BELIEVE Trial: Data from the Phase 3b Long-Term Rollover Study. American Society of Hematology. San Diego, California, United States.

Publication Details:

Cappellini MD, Taher AT, Viprakasit V, Georgiev P, **Kuo KHM**, Holot N, Vilmont V, Medlin LF, Kuo W-L, Lai Y, Bueno LM, Sheth S. Efficacy and Safety of Luspatercept in Patients Enrolled in the BELIEVE Trial: Data from the Phase 3b Long-Term Rollover Study. Blood. 2023 Nov 28;142(Supplement 1):3849. **Coauthor or Collaborator.**

- 2023 Dec 11 Risk Factors for Thrombosis in Pregnant People with Sickle Cell Disease: A Multinational Study. American

Society of Hematology. San Diego, California, United States.

Publication Details:

Choupa SBM, Luo A, Zarka C, Malinowski AK, **Kuo KHM**, Shehata N, Lanzkron SM, Pecker LH. Risk Factors for Thrombosis in Pregnant People with Sickle Cell Disease: A Multinational Study. Blood. 2023 Nov 28;142(Supplement 1):807. **Coauthor or Collaborator**.

- 2023 Dec 11 Comparison of Alloimmunization in Pregnant People with Sickle Cell Disease Receiving Chronic Versus on-Demand Transfusions: A Multinational Study. American Society of Hematology. San Diego, California, United States.

Publication Details:

Zarka C, Luo A, Choupa SBM, Malinowski AK, **Kuo KHM**, Shehata N, Lanzkron SM, Pecker LH. Comparison of Alloimmunization in Pregnant People with Sickle Cell Disease Receiving Chronic Versus on-Demand Transfusions: A Multinational Study. Blood. 2023 Nov 28;142(Supplement 1):927. **Coauthor or Collaborator**.

- 2023 Dec 11 Risk Factors for Postnatal Complications in People with Sickle Cell Disease: A Preliminary Analysis. American Society of Hematology. San Diego, California, United States.

Publication Details:

Loh J, Malinowski AK, Ward R, Shehata N, **Kuo KHM**, Georgescu I. Risk Factors for Postnatal Complications in People with Sickle Cell Disease: A Preliminary Analysis. Blood. 2023 Nov 28;142(Supplement 1):3904. **Coauthor or Collaborator**.

- 2023 Dec 10 Cross-Community Collaboration and Data Collection to Optimize Patient Care in Hemolytic Anemias. American Society of Hematology. San Diego, California, United States.

Publication Details:

Andemariam B, Badalamenti N, Blaylark R, Colasanti R, Colombatti R, Grace RF, Jonassaint CR, **Kuo KHM**, Levine LM, Lobo C, Schyrver T, Sheth S, Trimnell C, Watson A, Welle J, Woolford T, John H, Davis J, Zaidi AU, Gallagher E, Gheuens S, Shah N. Cross-Community Collaboration and Data Collection to Optimize Patient Care in Hemolytic Anemias. Blood. 2023 Nov 28;142(Supplement 1):3692. **Coauthor or Collaborator**.

- 2023 Dec 10 **Presenter**. Durable Symptom Improvement for Patients with Non-Transfusion Dependent Thalassemia Treated with Luspatercept: Patient-Reported Outcomes from the BEYOND Study. American Society of Hematology. San Diego, California, United States.

Publication Details:

Musallam KM, Taher AT, Kattamis A, Cappellini MD, Lord-Bessen J, Glassberg M, Pelligra C, Guo S, Medlin LF, Bueno LM, **Kuo KHM**. Durable Symptom Improvement for Patients with Non-Transfusion Dependent Thalassemia Treated with Luspatercept: Patient-Reported Outcomes from the BEYOND Study. Blood. 2023 Nov 28;142(Supplement 1):2474. **Senior Responsible Author**.

- 2023 Dec 9 **Presenter**. Initial Results in a Phase 1b Trial of PB-04 in Sickle Cell Disease Demonstrate Fetal Hemoglobin Induction, Additive Activity with Hydroxyurea, and Improved Red Blood Cell Sickling Parameters. American Society of Hematology. San Diego, California, United States.

Publication Details:

Kuo KHM, Singer ST, Blyden GT, Bronté-Hall L, Faller A, Kutlar A, Kuypers FA, Zaidi AU, Hines PC, Patel N, Larkin S, Al-Samkari H, Sheth S, Nouraie SM, Perrine S. Initial Results in a Phase 1b Trial of PB-04 in Sickle Cell Disease Demonstrate Fetal Hemoglobin Induction, Additive Activity with Hydroxyurea, and Improved Red Blood Cell Sickling Parameters. Blood. 2023 Nov 28;142(Supplement 1):1148. **Principal Author**.

- 2023 Dec 9 Safety and Efficacy of Mitapivat in Adult Patients with Erythrocyte Membranopathies (SATISFY). American Society of Hematology. San Diego, California, United States.

Publication Details:

Glenthøj A, van Beers EJ, van Wijk R, Rab MAE, Groot E, Vejlsstrup NG, Bendtsen SK, Toft N, Petersen J, Helby J, Chermat F, Fenaux P, **Kuo KHM**. Safety and Efficacy of Mitapivat in Adult Patients with Erythrocyte Membranopathies (SATISFY). *Blood*. 2023 Nov 28;142(Supplement 1):1085. **Senior Responsible Author**.

- 2023 Dec 9 Clocking the Processes and Challenges of Urgent Red Blood Cell Exchange for Acute Complications of Sickle Cell Disease. American Society of Hematology. San Diego, California, United States.

Publication Details:

Matusiak K, Cserti-Gazdewich CM, Shehata N, KHM Kuo, Kim J, Barth D, Patriquin CJ. Clocking the Processes and Challenges of Urgent Red Blood Cell Exchange for Acute Complications of Sickle Cell Disease. *Blood*. 2023 Nov 28;142(Supplement 1):2308. **Coauthor or Collaborator**.

- 2022 Dec 11 **Presenter**. Long-Term Improvements in Patient-Reported Outcomes in Patients with Pyruvate Kinase Deficiency Treated with Mitapivat. American Society of Hematology. New Orleans, Louisiana, United States. Available from: <https://doi.org/10.1182/blood-2022-168954>.

Publication Details:

Kuo KHM, Grace RF, van Beers EJ, Barcellini W, Glenthøj A, Holzhauer S, Patel P, Beynon V, Li J, Zagadailov E, Al-Samkari H. Long-Term Improvements in Patient-Reported Outcomes in Patients with Pyruvate Kinase Deficiency Treated with Mitapivat. *Blood*. 2022 Nov 15;140(Supplement 1):1223–1225. **Principal Author**.

- 2022 Dec 10 **Presenter**. Mitapivat Improves Iron Overload in Patients with Pyruvate Kinase Deficiency. American Society of Hematology. New Orleans, Louisiana, United States. Available from: <https://doi.org/10.1182/blood-2022-169098>.

Publication Details:

van Beers EJ, Al-Samkari H, Grace RF, Barcellini W, Glenthøj A, Dibacco M, Kosinski PA, Xu E, Beynon V, Patel P, Porter JB, **Kuo KHM**. Mitapivat Improves Iron Overload in Patients with Pyruvate Kinase Deficiency. *Blood*. 2022 Nov 15;140(Supplement 1):2460–2462. **Senior Responsible Author**.

- 2022 Dec 10 **Presenter**. Mitapivat Improves Markers of Erythropoietic Activity in Long-Term Study of Adults with Alpha- or Beta-Non-Transfusion-Dependent Thalassemia. American Society of Hematology. New Orleans, Louisiana, United States. Available from: <https://doi.org/10.1182/blood-2022-163493>.

Publication Details:

Kuo KHM, Layton DM, Lal A, Al-Samkari H, Kosinski PA, Tong B, Estepp JH, Uhlig K, Vichinsky EP. Mitapivat Improves Markers of Erythropoietic Activity in Long-Term Study of Adults with Alpha- or Beta-Non-Transfusion-Dependent Thalassemia. *Blood*. 2022 Nov 15;140(Supplement 1):2479–2480. **Principal Author**.

- 2022 Jun 10 **Presenter**. Long-term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia. European Hematology Association. Wien, Wien, Austria.

Publication Details:

Kuo KHM, Layton DM, Lal A, Al-Samkari H, Bhatia J, Kosinski PA, Tong B, Lynch M, Uhlig K, Vichinsky EP. Long-term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia. *Hemasphere*. 2022 May 12;358379:P1522. **Principal Author**.

- 2021 Dec 13 **Presenter**. Long-Term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

Kuo KHM, Layton DM, Lal A, Al-Samkari H, Bhatia J, Kosinski PA, Tong B, Lynch M, Uhlig K, Vichinsky E. Long-Term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia. Blood. 138((Supplement 1)):576. **Principal Author**.

- 2021 Dec 13 Characterizing Iron Overload By Age in Patients Diagnosed with Pyruvate Kinase Deficiency – a Descriptive Analysis from the Peak Registry. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

Bianchi P, Grace RF, Corrons J-LV, Glader B, Glenthøj A, Kanno H, **Kuo KHM**, Lander C, Layton DM, Pospíšilová D, Viprakasit V, Williams J, Yan Y, McGee B, van Beers EJ. Characterizing Iron Overload By Age in Patients Diagnosed with Pyruvate Kinase Deficiency – a Descriptive Analysis from the Peak Registry. Blood. 138((Supplement 1)):3074. **Coauthor or Collaborator**.

- 2021 Dec 13 Mitapivat Improves Ineffective Erythropoiesis and Reduces Iron Overload in Patients with Pyruvate Kinase Deficiency. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

Van Beers EJ, Al-Samkari H, Grace RF, Barcellini W, Glenthøj A, Judge MP, Kosinski PA, Xu E, Beynon V, McGee B, Porter JB, **Kuo KHM**. Mitapivat Improves Ineffective Erythropoiesis and Reduces Iron Overload in Patients with Pyruvate Kinase Deficiency. Blood. 138((Supplement 1)):2005. **Senior Responsible Author**.

- 2021 Dec 13 **Presenter**. The International Hemoglobinopathy Research Network (INHERENT): An International Initiative to Study the Role of Genetic Modifiers in Hemoglobinopathies. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

Kountouris P, Stephanou C, Archer NM, Bonifazi F, Giannuzzi V, **Kuo KHM**, Maggio A, Makani J, Mañú Pereira MDM, Michailidou K, Nkya SW, Nnodu OE, Trompeter S, Tshilolo L, Wonkam A, Zilfalil BA, Inusa BPD, Kleanthous M. The International Hemoglobinopathy Research Network (INHERENT): An International Initiative to Study the Role of Genetic Modifiers in Hemoglobinopathies. Blood. 138((Supplement 1)):948. **Coauthor or Collaborator**.

- 2021 Dec 13 A Phase 2/3, Randomized, Double-Blind, Placebo-Controlled Study of Mitapivat in Patients with Sickle Cell Disease. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

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- 2021 Dec 13 A Cognitive Profile of Adults with Sickle Cell Disease - Cluster Analysis. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

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- 2021 Dec 13 Bone Mineral Density Remains Stable in Pyruvate Kinase Deficiency Patients Receiving Long-Term Treatment with Mitapivat. 63rd annual meeting of the American Society of Hematology. Georgia, United States.

Publication Details:

Al-Samkari H, Grace RF, Glenthøj A, Andres O, Barcellini W, Galactéros F, **Kuo KHM**, Layton DM, Morado M, Viprakasit V, Dong Y, Tai F, Grekas L, Khoja K, Gheuens S, McGee B, Porter JB, van Beers EJ. Bone Mineral Density Remains Stable in Pyruvate Kinase Deficiency Patients Receiving Long-Term

Treatment with Mitapivat. Blood. 138((Supplement 1)):924. **Coauthor or Collaborator.**

2021 Jun 9 Ischemic White Matter Lesions are Associated with Cognitive Dysfunction in Adults with Sickle Cell Disease. European Hematology Association. (Trainee Presentation)

Publication Details:

Blais F, Padilha I, Castonguay M, Fadiga N, Letourneau-Guillon L, Ward R, Guilbert F, **Kuo K**, Soulières D, Forté S. Ischemic White Matter Lesions are Associated with Cognitive Dysfunction in Adults with Sickle Cell Disease. EHA Library. 2020 Jun 12;(324670):S262. **Coauthor or Collaborator.**

2021 Jun 9 Benefit of Continuing Therapy with Luspatercept in Patients with Beta-Thalassemia Who Do Not Achieve $\geq 33\%$ Reduction in Red Blood Cell (RBC) Transfusion Burden (TB) in Weeks 13–24 in the BELIEVE Trial. European Hematology Association. (Trainee Presentation)

Publication Details:

Piga A, Cappellini MD, Porter JB, Hermine O, **Kuo KHM**, Coates TD, Kattamis A, Shah F, Khelif A, Aydinok Y, Zhang G, Shetty JK, Miteva D, Zinger T, Bueno L, Yucel A, Tang D, Taher AT. Benefit of Continuing Therapy with Luspatercept in Patients with Beta-Thalassemia Who Do Not Achieve $\geq 33\%$ Reduction in Red Blood Cell (RBC) Transfusion Burden (TB) in Weeks 13–24 in the BELIEVE Trial. EHA Library. 2020 Jun 12;(325026):EP1306. **Coauthor or Collaborator.**

2020 Dec 7 Sickle Cell Cerebrovascular Reactivity to a CO₂ Stimulus Is Both Too Little and Too Slow. American Society of Hematology. United States. Presenter(s): Forté S. (Trainee Presentation)

Publication Details:

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2020 Dec 6 Allogeneic Hematopoietic Stem Cell Transplant Versus Gene Therapy in Sickle Cell Disease: Updated Results from a Systematic Review. American Society of Hematology. United States. Presenter(s): Rotin LE. (Trainee Presentation)

Publication Details:

Rotin LE, Viswabandya A, Kumar R, **Kuo KHM**. Allogeneic Hematopoietic Stem Cell Transplant Versus Gene Therapy in Sickle Cell Disease: Updated Results from a Systematic Review. Blood. 2020 Nov 5;136(Supplement 1):11-12. **Senior Responsible Author.**

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Publication Details:

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2020 Dec 5 Sustained Reductions in Red Blood Cell (RBC) Transfusion Burden and Events in β -Thalassemia with Luspatercept: Longitudinal Results of the Believe Trial. American Society of Hematology. United States. Presenter(s): Taher AT.

Publication Details:

Taher AT, Viprakasit V, Hermine O, Porter JB, Piga A, **Kuo KHM**, Coates TD, Voskaridou E, Khelif A, Kattamis A, Shetty JK, Zhang G, Tian Y, Miteva D, Zinger T, Tang D, Backstrom JT, Cappellini MD. Sustained Reductions in Red Blood Cell (RBC) Transfusion Burden and Events in β -Thalassemia with Luspatercept: Longitudinal Results of the Believe Trial. Blood. 2020 Nov 5;136(Supplement 1):45-46. **Coauthor or Collaborator.**

2020 Dec 5 Early-Onset Osteopenia and Osteoporosis in Patients with Pyruvate Kinase Deficiency. American Society of Hematology. United States. Presenter(s): Al-Samkari H.

Publication Details:

Al-Samkari H, Grace RF, Glenthøj A, Andres O, Barcellini W, Galactéros F, **Kuo KHM**, Layton DM, Morado M, Viprakasit V, Dong Y, Tai F, Hawkins P, Gheuens S, Bowden C, Porter JB, van Beers E. Early-Onset Osteopenia and Osteoporosis in Patients with Pyruvate Kinase Deficiency. Blood. 2020 Nov 5;136(Supplement 1):30-32. **Coauthor or Collaborator.**

2020 Jun 13 **Presenter.** Proof of Concept for the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-Transfusion-Dependent Thalassemia: Interim Results From an Ongoing, Phase 2, Open-Label, Multicenter Study. European Hematology Association. Frankfurt, Hessen, Germany.

Publication Details:

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Publication Details:

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Publication Details:

Al-Samkari H*, van Beers EJ, Barcellini W, Galactéros F, Glader B, **Kuo KHM**, Layton DM, Ravindranath Y, Yaish H, Dong Y, Tai F, Hawkins P, Beynon V, Gilroy K, Grace RF. Bone mineral density is stable in adults with pyruvate kinase deficiency receiving long-term treatment with Mitapivat. 2020 Jun 12. **Coauthor or Collaborator.**

2020 Jun 13 **Presenter.** Energize and energize-T: Two phase 3, Randomized, double-blind, placebo-controlled studies of Mitapivat in Adults with non-transfusion-dependant or transfusion-dependant Alpha-or Beta-Thalassemia. European Hematology Association.

Publication Details:

Kuo KHM*, Layton DM, Al-Samkari H, Kattamis A, Sheth S, Taher A, Viprakasit V, Chamberlain CX, Czaplá L, Gheuens S, Jiang L, Lynch M, Tong B, Uhlig K, Cappellini MD. Energize and energize-T: Two phase 3, Randomized, double-blind, placebo-controlled studies of Mitapivat in Adults with non-transfusion-dependant or transfusion-dependant Alpha-or Beta-Thalassemia. 2020 Jun 12. **Principal Author.**

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Publication Details:

Glenthøj A*, van Beers EJ, Al-Samkari H, Viprakasit V, **Kuo KHM**, Galactéros F, Chonat S, Porter J, Gheuens S, Beynon V, Xu E, Hawkins P, Zagadailov E, Oluyadi A, Barcellini W. Activate-T: A Phase 3, Open-label, multicenter study of Mitapivat in adults with Pyruvate Kinase deficiency who are regularly transfused. 2020 Jun 12. **Coauthor or Collaborator**.

- 2020 Jun 13 Ischemic white matter lesions are associated with cognitive dysfunction in adults with Sickle cell disease. European Hematology Association.

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Publication Details:

Boscoe A, Yan Y, Hedgeman E, van Beers EJ, Al-Samkari H, Barcellini W, Eber SW, Glader B, Yaish HM, Chonat S, Rothman JA, Sharma M, Kollmar N, Knoll CM, **Kuo KHM**, Kwiathkowski J, Pastore YD, Thompson AA, Ravindranath Y, Neufeld EJ, Wang H, Holzhauer S, Breakey VR, Verhovsek M, Kunz JB, Sheth S, McNaull MA, Rose MJ, Pospisilova DD. Comorbidities and Complications in Adults with Pyruvate Kinase Deficiency. Blood. 2019 Nov 13;134(Suppl 1):2175. **Coauthor or Collaborator**.

- 2019 Nov 13 Rate of Sickle Hemoglobin Recovery in Sickle Cell Disease Patients Undergoing Red Blood Cell (RBC) Exchange Transfusion is Associated with Age of Patients and Number of RBC Units Transfused. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Aneke JC. (Trainee Presentation)

Publication Details:

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- 2019 Nov 13 Predictors of the Rowland Universal Dementia Assessment Scale (RUDAS) Performance in Adults with Sickle Cell Disease. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Forte S. (Trainee Presentation)

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- 2019 Nov 13 Prediction of Risk for Adverse Pregnancy Outcomes in Women with Sickle Cell Disease. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Malinowski AK.

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2019 Nov 13 **Presenter**. Mitapivat (AG-348), an Oral PK-R Activator, in Adults with Non-Transfusion Dependent Thalassemia: A Phase 2, Open-Label, Multicenter Study in Progress. American Society of Hematology. Orlando, Florida, United States. Presenter(s): **Kuo KHM**.

Publication Details:

Kuo KHM, Layton DM, Uhlig K, Lynch M, Liu L, Vichinsky E. Mitapivat (AG-348), an Oral PK-R Activator, in Adults with Non-Transfusion Dependent Thalassemia: A Phase 2, Open-Label, Multicenter Study in Progress. Blood. 2019 Nov 13;134(Suppl 1):2249. **Principal Author**.

2019 Nov 13 Evaluating Luspatercept Responders in the Phase 3, Randomized, Double-Blind, Placebo-Controlled BELIEVE Trial of Luspatercept in Adult Beta-Thalassemia Patients (Pts) Who Require Regular Red Blood Cell (RBC) Transfusions. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Viprakasit V.

Publication Details:

Viprakasit V, Taher A, Hermine O, Porter J, Piga A, **Kuo KHM**, Coates T, Voskaridou E, Liew HK, Perrotta S, Khlif A, Kattamis A, Laadem A, Shetty JK, Kuo W-L, Miteva D, Zinger T, Sinsimer D, Linde PG, Cappellini MD. Evaluating Luspatercept Responders in the Phase 3, Randomized, Double-Blind, Placebo-Controlled BELIEVE Trial of Luspatercept in Adult Beta-Thalassemia Patients (Pts) Who Require Regular Red Blood Cell (RBC) Transfusions. Blood. 2019 Nov 13;134(Suppl 1):3545. **Coauthor or Collaborator**.

2019 Nov 13 Pyruvate Kinase (PK) Protein and Enzyme Levels in the Diagnosis and Clinical Phenotype of PK Deficiency. American Society of Hematology. Florida, United States.

Publication Details:

Addonizio Kathryn, Al-Samkari Hanny, Glader Bertil, Morton D Holmes, Chonat Satheesh, Thompson Alexis A, Kuo Kevin H M, Ravindranath Yaddanapudi, Wang Heng, Rothman Jennifer A, Kwiatkowski Janet L, Kung Charles, Kosinski Penelope A, Al-Sayegh Hasan, London Wendy B, Grace Rachael G. Pyruvate Kinase (PK) Protein and Enzyme Levels in the Diagnosis and Clinical Phenotype of PK Deficiency. Blood. 2019 Nov 13;134(Suppl 1):3515. **Coauthor or Collaborator**.

2019 Nov 13 Long-Term Safety and Efficacy of Mitapivat (AG-348), a Pyruvate Kinase Activator, in Patients with Pyruvate Kinase Deficiency: The DRIVE PK Study. American Society of Hematology. Florida, United States.

Publication Details:

Grace Rachael F, Layton Mark, Galacteros Frederic, Barcellini Wilma, van Beers Eduard J, Yaish Hassan M, Ravindranath Yaddanapudi, Kuo Kevin H M, Sheth Sujit, Kwiatkowski Janet L, Hua Lei, Hawkins Pter F, Mix Chris, Glader Bertil. Long-Term Safety and Efficacy of Mitapivat (AG-348), a Pyruvate Kinase Activator, in Patients with Pyruvate Kinase Deficiency: The DRIVE PK Study. Blood. 2019 Nov 13;134(Suppl 1):3512. **Coauthor or Collaborator**.

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Publication Details:

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Publication Details:

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- 2018 Dec 3 Should Women with Sick Cell Disease be Administered Prophylactic Transfusions to Avoid Complications during Pregnancy? American Society of Hematology. San Diego, California, United States. (Trainee Presentation)

Publication Details:

Palcu P, **Kuo KHM**, Ward R, Zaffar N, Malinowski AK, Shehata N. Should Women with Sick Cell Disease be Administered Prophylactic Transfusions to Avoid Complications during Pregnancy? Blood. 132(Suppl 1):3679. **Coauthor or Collaborator.**

- 2018 Dec 2 **Presenter.** Is There a Role for Thromboprophylaxis in Sick Cell Patients with Central Venous Access Devices? American Society of Hematology. San Diego, California, United States. (Trainee Presentation)

Publication Details:

Forté S, Fadiga N, Pestrin O, Aneke JC, Bhat S, Ward R, **Kuo KHM**. Is There a Role for Thromboprophylaxis in Sick Cell Patients with Central Venous Access Devices? Blood. 132(Suppl 1):3518. **Senior Responsible Author.**

- 2018 Dec 1 **Presenter.** Validation of Sick Cell Disease Severity Score in a Cohort of Hemoglobin SC Disease Patients. American Society of Hematology. San Diego, California, United States. (Trainee Presentation)

Publication Details:

Rosart RM, Pestrin OA, Tomlinson GA, Ward R, Pendergrast J, Binding A, **Kuo KHM**. Validation of Sick Cell Disease Severity Score in a Cohort of Hemoglobin SC Disease Patients. Blood. 132(Suppl 1):2287. **Senior Responsible Author.**

- 2018 Dec 1 The Believe Trial: Results of a Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Luspatercept in Adult Beta-Thalassemia Patients Who Require Regular Red Blood Cell (RBC) TransfusionsClinically Relevant Abstract. American Society of Hematology. San Diego, California, United States.

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Publication Details:

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Publication Details:

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- 2017 Dec 10 Results Update from the DRIVE PK Study: Effects of AG-348, a Pyruvate Kinase Activator, in Patients with Pyruvate Kinase Deficiency. American Society of Hematology. Atlanta, Georgia, United States. Presenter(s): Grace RF. Available from: <https://ash.confex.com/ash/2017/webprogram/Paper102236.html>.

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California, United States. Presenter(s): Grace RF. Available from:
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- 2015 Dec 3 **Presenter**. Management of Osteonecrosis By Adult and Pediatric Sickle Cell Providers. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Mulla-Ali DH, **Kuo KHM**. Available from: <http://www.bloodjournal.org/content/126/23/4465>. (Trainee Presentation)

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- 2015 Dec 3 **Presenter**. The Phenotypic Spectrum of Pyruvate Kinase Deficiency (PKD) from the PKD Natural History Study (NHS): Description of Four Severity Groups By Anemia Status. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Grace RF. Available from: <http://www.bloodjournal.org/content/126/23/2136>. (Trainee Presentation)

Publication Details:

Grace RF, Morton DH, Barcellini W, Eber SW, Depsotovic JM, Knoll CM, Yaish HM, Newburger PE, Rothman J, Thompson AA, Ravindranath Y, Kunz J, van Beers EJ, Wlodarski MW, Nottage KA, Wang H, Sharma M, Rose MJ, Pastore YD, Giardina PJ, Rhodes M, **Kuo KHM**, Breakey VR, Neufeld EJ, Guo D, London WB, Falcone J, Glader B. The Phenotypic Spectrum of Pyruvate Kinase Deficiency (PKD) from the PKD Natural History Study (NHS): Description of Four Severity Groups By Anemia Status. *Blood*. 2015 Dec 3;126(23):4465. **Coauthor or Collaborator**.

- 2015 Dec 3 **Presenter**. Molecular Characterization of 140 Patients in the Pyruvate Kinase Deficiency (PKD) Natural History Study (NHS): Report of 20 New Variants. American Society of Hematology. Orlando, Florida, United States. Presenter(s): Grace RF. Available from: <http://www.bloodjournal.org/content/126/23/3337>. (Trainee Presentation)

Publication Details:

Bianchi P, Fermo E, Lezon-Geyda K, Gallagher PK, Morton DH, Barcellini W, Glader B, Eber SW, Despotovic JM, Knoll CM, Yaish HM, Newburger PE, Rothman J, Thompson AA, Ravindranath Y, Nottage KA, Wang H, van Beers EJ, Kunz J, Wlodarski MW, Sharma M, Rose MJ, Pastore YD, Giardina PJ, Rhodes M, **Kuo KHM**, Guo D, London WB, Grace RF. Molecular Characterization of 140 Patients in the Pyruvate Kinase Deficiency (PKD) Natural History Study (NHS): Report of 20 New Variants. *Blood*. 2015 Dec 3;126(23):3337. **Coauthor or Collaborator**.

- 2014 Dec 6 **Presenter**. Management of Cancer-Associated Venous Thromboembolism in Patients with Concomitant Thrombocytopenia: A Survey of Canadian Practitioners. American Society of Hematology. San Francisco, California, United States. Presenter(s): Kopolovic I, Conter HJ, **Kuo KH**. Available from: <http://www.bloodjournal.org/content/124/21/2174>. (Trainee Presentation)

Publication Details:

Management of Cancer-Associated Venous Thromboembolism in Patients with Concomitant Thrombocytopenia: A Survey of Canadian Practitioners.

- 2013 Dec Treatment Patterns and Outcomes Of Sickle Cell Patients With Frequent ER Visits: A Single Center Experience. American Society of Hematology.

Publication Details:

Naessens V, Ward R, **Kuo KHM**. Treatment Patterns and Outcomes Of Sickle Cell Patients With Frequent ER Visits: A Single Center Experience. Blood. 2013 Nov;122(21):1010. **Senior Responsible Author**.

- 2013 Nov 15 Characterization Of Pulmonary Compliance In Sickle Cell Patients Revealed Wide Variability. American Society of Hematology. United States.

Publication Details:

Mian HS, **Kuo KHM**, Wang VN, Ward R, Mak S. Characterization Of Pulmonary Compliance In Sickle Cell Patients Revealed Wide Variability. Blood. 2013 Nov 15;122(21):989. **Coauthor or Collaborator**.

- 2013 Nov 15 A North American Experience Of Hemoglobin SC Disease, Its Complications, and Management. American Society of Hematology.

Publication Details:

Naessens V, Ward R, **Kuo KHM**. A North American Experience Of Hemoglobin SC Disease, Its Complications, and Management. Blood. 2013 Nov 15;122(21):2221. **Senior Responsible Author**.

- 2013 Nov 15 Treatment Patterns and Outcomes Of Sickle Cell Patients With Frequent ER Visits: A Single Center Experience. American Society of Hematology.

Publication Details:

Naessens V, Ward R, **Kuo KHM**. Treatment Patterns and Outcomes Of Sickle Cell Patients With Frequent ER Visits: A Single Center Experience. Blood. 2013 Nov 15;122(21):1010. **Senior Responsible Author**.

- 2013 Nov 15 Effectiveness Of An Analgesia Protocol For The Treatment Of Painful Vaso-Occlusive Crisis Of Sickle Cell Disease Patients In Emergency Room: A Retrospective Cohort Study. American Society of Hematology. United States.

Publication Details:

Naessens V, Ward R, **Kuo KHM**. Effectiveness Of An Analgesia Protocol For The Treatment Of Painful Vaso-Occlusive Crisis Of Sickle Cell Disease Patients In Emergency Room: A Retrospective Cohort Study. Blood. 2013 Nov 15;122(21):2222. **Senior Responsible Author**.

- 2012 Dec **Presenter**. A Comparison of Chronic Manual and Automated Red Blood Cell Exchange Transfusion in Sickle Cell Disease Patients From Two Comprehensive Care Centres in the United Kingdom. 63rd annual meeting of the American Society of Hematology. United States. **Kuo KHM**, Ward R, Howard J, Telfer P. Abstract #3430 (Poster presentation).

Publication Details:

A Comparison of Chronic Manual and Automated Red Blood Cell Exchange Transfusion in Sickle Cell Disease Patients From Two Comprehensive Care Centres in the United Kingdom.

- 2011 Jun EspP, an Extracellular Serine Protease from Enterohemorrhagic E. coli, Induces Coagulopathy in Human Plasma and Fibrinolysis in Whole Blood. XXIII Congress of the International Society of Thrombosis and Haemostasis. Kyoto, Kyôto [Kyoto], Japan. Presenter(s): Khan S.

Publication Details:

Khan S, **Kuo KHM**, Brnjac E, Pai EF, Chesney AE. EspP, an Extracellular Serine Protease from

Enterohemorrhagic E. coli, Induces Coagulopathy in Human Plasma and Fibrinolysis in Whole Blood. Journal of Thrombosis and Haemostasis. 2011 Jun;9(Suppl 2):1359. **Co-Principal Author**.

- 2011 Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sick Cell Disease. 53rd annual meeting of the American Society of Hematology. **Kuo KHM**, Barth D, Ward R. Abstract #2326 (Poster presentation).

Publication Details:

Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sick Cell Disease.

- 2011 **Presenter**. Single-Centre, North American Experience with Compassionate Use of Deferiprone in Patients with Beta-Thalassemia Major. 53rd annual meeting of the American Society of Hematology. Miscevic F, **Kuo KHM**, Ward R. Abstract #3185 (Poster presentation).

Publication Details:

Single-Centre, North American Experience with Compassionate Use of Deferiprone in Patients with Beta-Thalassemia Major.

- 2008 Neuroinflammatory and Neurodegenerative Markers in Non-Hodgkin's Lymphoma and Hematologic Malignancies with Central Nervous System Involvement. 50th annual meeting of the American Society of Hematology.

Publication Details:

Tang W, **Kuo KHM**, Lazo-Langner A, Pasternak SH, Xenocostas A. Neuroinflammatory and Neurodegenerative Markers in Non-Hodgkin's Lymphoma and Hematologic Malignancies with Central Nervous System Involvement. Blood. 2008;112(11). Abstract #2829.

- 2006 Management of Hyperleukocytosis in Acute Myelogenous Leukemia Using Hydroxyurea Rather Than Leukopheresis. 48th annual meeting of the American Society of Hematology.

Publication Details:

Kuo KHM, Callum J, Brandwein J, Crump M, Curtis JE, Gupta V, Lipton JH, Pantalony D, Schimmer S, Schuh A, Sher G, Sutton DM, Yee KWL, Keating A, Minden MD, Messner MA. Management of Hyperleukocytosis in Acute Myelogenous Leukemia Using Hydroxyurea Rather Than Leukopheresis. Blood. 2006;108(11):568a. Abstract #2007.

- 2003 Successful Treatment of Heparin Induced Thrombocytopenia (HIT) with Fondaparinux. 45th annual meeting of the American Society of Hematology.

Publication Details:

Kuo KHM, Kovacs MJ. Successful Treatment of Heparin Induced Thrombocytopenia (HIT) with Fondaparinux. Blood. 2003;102(11):319a. Abstract #1147. **Principal Author**.

Other Lectures and Presentations

- 2012 May 28 Manual and Automated RBC Exchange Transfusion in Patients with Sick Cell Disease: the Canadian Experience. Bart's and the London NHS Trust. London, United Kingdom.
- 2011 Dec 8 Be a Blood Detective. American Society of Hematology High School Student Symposium. San Diego, California.
- 2011 **Presenter**. High School Student Symposium on Sick Cell Disease, American Society of Hematology.
- 2010 **Presenter**. High School Student Symposium on Sick Cell Disease, American Society of Hematology.
- 2009 **Presenter**. High School Student Symposium on Sick Cell Disease, American Society of Hematology.

2. NATIONAL

Invited Lectures and Presentations

- 2023 Oct 24 **Invited Speaker.** Rare Disease Strategy – Evolution, Needs and Future Steps. Canadian Association of Population Therapeutics. Toronto, Ontario, Canada.
- 2023 Sep 9 **Invited Speaker.** Forks in the Road in the Treatment of Sickle Cell Disease. Sickle Cell Disease Association of Canada (SCDAC). Montréal, Quebec, Canada. National Conference on Sickle Cell Disease.
- 2023 May 26 **Keynote Speaker.** Therapeutic Approaches to the Treatment of Thalassemia. NEJM 医学前沿 (Frontiers in Medicine). China.
- 2022 Sep 17 **Speaker.** IHERENT: an international initiative to study the role of genetic modifiers in hemoglobinopathies. Canadian Haemoglobinopathy Association. Canmore, Alberta, Canada. CanHaem Annual Meeting.
- 2022 May 24 **Invited Speaker.** 23 Egyptian Thalassemia Association (ETA & TIF) Conference 2022 - Novel Agents Targeting Ineffective Erythropoiesis and Iron Dysregulation in Thalassemia. Egyptian Thalassemia Association. Cairo, Al Qahirah, Egypt.
- 2022 Feb 8 **Invited Lecturer.** It Takes Two to Tango: Interplay Between Ineffective Erythropoiesis and Iron Overload in the Pathogenesis of Thalassemia. Canadian Blood Services. Toronto, Ontario, Canada.
- 2022 Feb 3 **Invited Speaker.** The Dawn of Targeted Therapies in Thalassemia. Thalassemia Foundation Canada. Toronto, Ontario, Canada.
- 2022 Jan 28 **Invited Speaker.** The Promise of Novel Pharmacological Agents in Thalassemia. The Annual Scientific Conference on Sickle Cell and Thalassemia (ASCAT). Cairo, Al Qahirah, Egypt.
- 2020 Nov 5 **Invited Speaker.** Access to Innovative and Curative Therapies - Highlights on Gene Therapy _in Rare Blood Disorders. Network of Rare Blood Disorder Organizations. Canada. Available from: <https://www.youtube.com/watch?v=LR7okCcKDpM&feature=youtu.be>.
- 2020 May 20 **Chair.** Q&A Session on Managing Thalassemia During the COVID-19 Pandemic. Thalassemia Foundation Canada. Canada. Presenter(s): **Kuo KHM**, Pendergrast J, Johnson JC.
- 2020 Apr 27 **Chair.** How to Manage Sickle Cell Disease During COVID-19. Sickle Cell Disease Association of Canada (SCDAC/AAFC). Canada. Presenter(s): **Kuo KHM**, Pendergrast J, Johnson JC.
- 2019 Nov 29 **Invited Speaker.** National Research Meeting: Could We ., Would We ., Should We ...? _Choices, Challenges, and Opportunities in the Era of Potentially Curative Therapies in Sickle Cell Disease. Sickle Cell Disease Association of Canada. Montreal, Quebec, Canada.
- 2019 Nov 13 **Presenter.** Health Canada luspatercept pre-submission meeting. Health Canada. Canada.
- 2019 Oct 18 **Presenter.** CADTH luspatercept pre-submission meeting. Canadian Agency for Drugs and Technologies in Health (CADTH). Canada.
- 2019 Oct 15 **Invited Speaker.** Transfusion Medicine Physician Trainees Teaching Session: Changing Paradigms in Iron Overload Management. Canadian Blood Services. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**. Webcast.
- 2019 Sep 28 **Invited Speaker.** 6th Annual Canadian Haemoglobinopathy Conference: Challenging Cases in _Non-Transfusion Dependent Thalassemia. The Canadian Haemoglobinopathy Association. Hamilton, Ontario, Canada. Presenter(s): Klaassen R, **Kuo KHM**.
- 2019 Sep 17 **Invited Speaker.** When Thalassemia Trait is Not a Trait. CARE Education. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**.
- 2019 Jul 3 **Invited Speaker.** Gene Editing/Therapy for Thalassemia: Is It the Beginning of the End? Thalassemia

Foundation Canada. Canada.

- 2019 Jun 24 **Presenter.** CADTH ravulizumab pre-submission meeting. Canadian Agency for Drugs and Technologies in Health (CADTH). Canada.
- 2019 May 11 **Invited Speaker.** A Rare International Dialogue - Advancing Cell and Gene Therapies for Hemoglobinopathies. Canadian Organization for Rare Disorders. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**. Available from: http://www.raredisorders.ca/content/uploads/RDI-CORD-Conference_May-2019_agenda-FINAL1.pdf.
- 2019 Apr 27 **Invited Speaker.** Hopes and Opportunities for Rare Anemias in the Era of Gene Therapy. Network of Rare Blood Disorder Organizations. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**.
- 2018 Nov 10 **Invited Speaker.** Gene Therapy as a cure for Sickle Cell Disease. Sickle Cell Disease Association of Canada. Toronto, Ontario, Canada. 4th National Biennial Conference on Sickle Cell Disease.
- 2018 Oct 14 **Invited Speaker.** Update on New Approaches to the Treatment of Thalassemia. Thalassemia Foundation Canada. Toronto, Ontario, Canada. National Thalassemia Patient and Family Conference.
- 2017 Sep 21 **Invited Speaker.** Health Canada Preceptorship Day. Princess Margaret Cancer Centre Cancer Clinical Research Unit. Toronto, Ontario, Canada. Challenges and Opportunities in Red Blood Cell Clinical Research.
- 2017 Sep 9 **Invited Speaker.** Mechanisms of Cardiac Iron Overload Cardiomyopathy. The Canadian Haemoglobinopathy Association. Edmonton, Alberta, Canada.
- 2017 Mar 31 **Invited Speaker.** Novel and High-Risk, High-Reward Therapies in Sickle Cell Disease. Sickle Cell Disease Association of Canada. Toronto, Ontario, Canada.
- 2017 Feb 28 **Invited Speaker.** Transfusion Medicine Physician Trainees Teaching Session: Changing Paradigms in Iron Overload Management. Canadian Blood Services. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**. Webcast.
- 2016 Jul 16 **Invited Speaker.** Red Cell Disorders excluding Hemoglobinopathies. National Hematology Retreat. Toronto, Ontario, Canada. Presenter(s): **Kuo KHM**. Jerry Scott Academic Day.
- 2015 Sep 15 **Invited Speaker.** Lifelines: The 2015 Pan-Canadian Thalassemia Conference - Why Research Matters _In Thalassemia. Canadian Haemoglobinopathy Association. Toronto, Ontario, Canada.
- 2015 Apr 7 **Invited Speaker.** Hemoglobin-based Oxygen Carriers. Canadian Blood Services. Toronto, Ontario, Canada. Presenter(s): Kevin H.M. **Kuo**. Webcast.
- 2013 Feb 12 Transfusion for the Treatment and Prevention of Sickle Cell Complications. Canadian Blood Services and the Royal College of Physicians and Surgeons of Canada Transfusion Medicine Fellowship Program. Canada. Transfusion Medicine Resident Education Seminar.
- 2013 Jan 16 Evidence on the Effectiveness of Red Blood Cell Exchange Transfusion in Sickle Cell Disease. University of Alberta Hematology Grand Rounds. Edmonton, Alberta, Canada. (Continuing Education).
- 2013 Jan 15 Decoding Hemoglobinopathies. University Alberta academic half day for adult hematology, pediatric hematology-oncology and hematopathology trainees.

Media Appearances

- 2023 Nov 20 **Invited Speaker.** CRISPR treatment for sickle cell disease approved in U.K. Interviewer: Matt Galloway. The Current, CBC Radio. Toronto, Ontario, Canada. Presenter(s): Beverley Ndukwu, Dr. Kevin Kuo, Jason Mast. <https://www.cbc.ca/radio/thecurrent/monday-november-20-2023-full-transcript-1.7034176>. Available from: <https://www.cbc.ca/listen/live-radio/1-63/clip/16024274> <https://www.cbc.ca/listen/live-radio/1-63/clip/16024274>.

Other Lectures and Presentations

- 2013 Jan 15 Combination or Monotherapy in Iron Chelation: Where is the Evidence? University of Alberta Hematology CME Event. Edmonton, Alberta, Canada. (Continuing Education).
- 2012 Apr 14 Exchange Transfusion and Sickle Cell Disease. Canadian Apheresis Group/Canadian Association of Apheresis Nurses 32nd Annual General Meeting and Scientific Sessions. Toronto, Canada.
- 2012 Jan 26 Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sickle Cell Disease. Canadian Blood Services special seminar. (webcast available at <http://www.transfusionmedicine.ca/node/1533>).
- 2011 Sep 20 Sickle Cell Disease in the Genomic Era. Canadian Haemoglobinopathy Group Videoconference Rounds.
- 2011 Jul 15 Hemoglobinopathy Workshop Case Studies. 5th Annual National Hematology Residents' Retreat. Toronto, Canada.
- 2011 May 13 "The diagnosis of transfusional iron overload" in Simultaneous Session: Transfusional Iron Overload. Canadian Society for Transfusion Medicine Conference. Toronto, Canada.
- 2011 Apr 14 Does Chronic Transfusion Prevent Silent Cerebral Infarcts in Patients with Sickle Cell Disease? Canadian Blood Services Rounds. Toronto, Canada.
- 2010 Apr 14 Acquired von Willebrand Disease. Canadian von Willebrand Disease Update. Case presentation.

3. PROVINCIAL / REGIONAL

Invited Lectures and Presentations

- 2023 Nov 3 **Invited Speaker.** Beyond Hydroxyurea: Emerging Therapies for Sickle Cell Disease. Sickle Cell Awareness Group of Ontario (SCAGO). Ontario, Canada. 3rd Annual SCAGO Summit Theme: Advocacy in Sickle Cell Disease.
- 2023 May 11 **Invited Speaker.** The Role of Curative Therapy in Sickle Cell Disease. University of British Columbia. Vancouver, British Columbia, Canada.
- 2023 Apr 19 **Invited Speaker.** Forks in the Road in the Treatment of Sickle Cell Disease. Centre hospitalier de l'Université de Montréal. Montreal, Quebec, Canada.
- 2023 Apr 19 **Invited Speaker.** In Search of the Holy Grail in Fetal Globin Reactivation. Centre hospitalier de l'Université de Montréal. Montréal, Quebec, Canada.
- 2022 Nov 5 **Invited Speaker.** Sickle Cell Disease Care and a Comparative Analysis of SCD Health Practices in Canada and Lebanon. Sickle Cell Awareness Group of Ontario. Ontario, Canada. Sickle Cell Summit.
- 2022 May 28 **Presenter.** Attacking Sickle Cell Disease from All Fronts. Sickle Cell Awareness Group of Ontario. Toronto, Ontario, Canada.
- 2020 May 23 Novel Disease Modifying Therapies: Are we There Yet? Sickle Cell Awareness Group of Ontario. Ontario, Canada.
- 2018 Apr 27 **Invited Speaker.** A No Cookie-Cutter Approach to Cancer Related Anemia. Grey Bruce Health Services. Owen Sound, Ontario, Canada.
- 2016 Oct 31 **Invited Speaker.** Changing Paradigms in Thalassemia Management. Western University. London, Ontario, Canada.
- 2016 Oct 7 **Invited Speaker.** Peculiar and Parlous Anemias. Memorial University. St. John's, Newfoundland and Labrador, Canada. •Discuss investigation options for anemia to identify fatal hematologic disorders which

can often have a benign or complex presentation

- Review the diagnosis and management of rare hematologic disorders which can present with anemia or mild cytopenias
- Review the multi-organ complications of complement-mediated disease
- Review cases highlighting diagnostic and prognostic challenges in managing unusual hemolytic anemias.

- 2016 Sep 24 **Invited Speaker.** An Update on Clinical Trials in Sickle Cell Disease. Sickle Cell Awareness Group of Ontario. Toronto, Ontario, Canada.
- 2016 Aug 4 **Invited Speaker.** No More Transfusion? Changing Paradigm in Thalassemia Management. University of Calgary. Calgary, Alberta, Canada. 1. Review the history of thalassemia management and it's association with iron chelation
2. Discuss the obstacles to thalassemia management in the context of the current paradigm
3. Examine possible non-chelating means of managing iron overload
4. Explore the possibility of pharmacologic means to achieve transfusion-independence in transfusion-dependent thalassemia.
- 2013 Sep 15 **Invited Lecturer.** Alberta Hematology Fellows Retreat: Decoding Hemoglobinopathies. University of Alberta and University of Calgary. Banff, Alberta, Canada.
- 2013 Sep 12 Transfusions for Sickle Cell Disease Complications: Evidence-Based or Faith-Based? University of Calgary Division of Hematology and Hematologic Malignancies Education Rounds. Calgary, Alberta, Canada.

Media Appearances

- 2017 Mar 2 **Invited Speaker.** Thalassemia and the Red Blood Cell Disorders Clinic at Toronto General Hospital. 8th annual Fairchild Chinese-Canadian Radiothon in support of TGWHF, Fairchild Radio. Toronto, Ontario, Canada. End date: 2017 Mar 2.

Other Lectures and Presentations

- 2012 Sep 21 Sickle Cell and Avascular Necrosis - A Hematologist's Perspective. Humber River Regional Hospital. Toronto, Canada.
- 2012 Sep 14 Sickle Cell and the Brain. Rouge Valley Health System. Toronto, Canada.
- 2012 Jul 28 Preventing Both the Silent and Symptomatic Strokes in Sickle Cell Disease. Sickle Cell Awareness Group of Ontario.

4. LOCAL

Invited Lectures and Presentations

- 2023 Sep 28 **Invited Speaker.** Reducing risk from transfusion in patients with sickle cell disease. University of Toronto. Toronto, Ontario, Canada. Presenter(s): Pendergrast J and **Kuo KHM**. University of Toronto Transfusion Medicine Monthly Rounds.
- 2023 May 29 **Keynote Speaker.** Forks in the Road in Haemoglobinopathy Research. University of Toronto. Montréal, Quebec, Canada. University of Toronto Divisions of Hematology & Hematopathology 16th Annual Academic Day.
- 2022 Aug 9 **Invited Lecturer.** It Takes Two to Tango: Interplay Between Ineffective Erythropoiesis and Iron Overload in the Pathogenesis of Thalassemia. John Hopkins Hospital. Baltimore, Maryland, United States. John Hopkins Heme School lecture series.
- 2022 Jul 25 **Invited Lecturer.** Tackling Common Pathways in Thalassemia and Sickle Cell Disease. University of

Western Ontario, Division of Hematology, Department of Medicine. London, Ontario, Canada. Division noon rounds.

- 2022 Jul 25 **Invited Lecturer.** Approach to Congenital Hemolytic Anemias. University of Western Ontario, Division of Hematology, Department of Medicine. London, Ontario, Canada. Haematology subspecialty trainee rounds.
- 2022 Jun 16 **Invited Lecturer.** Novel Therapies for Patients with Sickle Cell Disease. Nnamdi Azikiwe University Teaching Hospital. Nnewi, Anambra, Nigeria. 2022 World Sickle Cell Day lecture.
- 2022 May 27 **Lecturer.** Thalassemias and Sickle Cell Disease. University of Toronto Maternal Fetal Medicine Fellowship Training Program. Toronto, Ontario, Canada. Academic Half Day.
- 2021 Mar 4 **Invited Lecturer.** Approach to Hemolytic Anemias: Beyond the Usual. University of Calgary. Calgary, Alberta, Canada.
- 2021 Feb 10 **Invited Lecturer.** Approach to Hemolytic Anemias: Beyond the Usual. McGill University. Montreal, Quebec, Canada.
- 2020 Dec 17 **Invited Lecturer.** Conventional and Novel Treatments in Sickle Cell Disease, a Balancing Act. University Health Network. Toronto, Ontario, Canada.
- 2020 Nov 26 **Invited Speaker.** Gene Therapy for Rare Inherited Anemias: Reality Behind the Hype. Rare Disease Review Western. London, Ontario, Canada.
- 2019 Jul 23 **Invited Speaker.** Approach to Hemolytic Anemias: Beyond the Usual. GIM club. Toronto, Ontario, Canada.
- 2017 Sep 15 **Invited Speaker.** A No-Cookie Cutter Approach to Cancer-Related Anemia. Windsor Regional Cancer Centre. Windsor, Ontario, Canada.
- 2017 May 14 **Invited Speaker.** Standing at the crossroads of sickle cell disease therapy: cure or control? Brampton Civic Hospital. Brampton, Ontario, Canada.
- 2017 May 8 **Invited Speaker.** A No-Cookie Cutter Approach to Cancer-Related Anemia. Joseph Brant Hospital. Burlington, Ontario, Canada.
- 2017 Apr 8 **Invited Speaker.** No More Transfusion in Thalassemia: _Hype or Hope? Thalassemia Foundation Canada - Guelph Chapter. Guelph, Ontario, Canada.
- 2016 Oct 19 **Invited Speaker.** Complement _Dysregulation and Disease. Niagara Health System's Medical Staff Association. Niagara-on-the-Lake, Ontario, Canada.
- 2016 Aug 4 **Invited Speaker.** Hematology residency training program academic half-day - Diagnosis of Thalassemia. University of Calgary. Calgary, Alberta, Canada.
- 2016 Feb 1 **Invited Speaker.** In Search of Home Runs in Hemoglobinopathies. Princess Margaret Cancer Centre. Toronto, Ontario, Canada. Princess Margaret Cancer Conference, Personalizing Cancer Medicine in 2016.
- 2015 Nov 4 **Invited Speaker.** Osteonecrosis in Sickle cell disease. CHU Ste-Justine. Montreal, Quebec, Canada. Presenter(s): **Kuo KHM**. Rendez-vous d'hématologie du CHU Ste-Justine.
- 2009 Review of Hemolytic Uremic Syndrome. Adult Haematology Grand Rounds, University of Toronto. Toronto, Canada. (Continuing Education).
- 2007 Utility of CSF Serological Testing in the Diagnosis of Creutzfeldt-Jakob Disease. Krembil Neuroscience Grand Rounds, University of Toronto. Toronto, Canada.

Other Lectures and Presentations

- 2012 Aug 2 Red Blood Cell Exchange Transfusion in Sickle Cell Disease. Toronto Haemoglobinopathy Rounds,

Kevin Hsin-Mu KUO

University of Toronto. Toronto, Canada.

2005 In Search of Neurodegenerative Markers for Patients Undergoing Chemotherapy. Haematology Rounds, London Health Sciences Centre. London, Ontario, Canada.

5. OTHER

Presented and Published Abstracts

2011 Dec Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sickle Cell Disease. American Society of Hematology. United States.

Publication Details:

Kuo KHM, Barth D, Ward R. Effectiveness, Time Utilization and Clinical Outcome of Partial Manual Red Cell Exchange in Patients with Sickle Cell Disease. Blood. 2011 Nov;118:2326. **Principal Author**.

2011 Nov Single-Centre, North American Experience with Compassionate Use of Deferiprone in Patients with Beta-Thalassemia Major. Presenter(s): Miscevic F, **Kuo KHM**, Ward R.

Publication Details:

Miscevic F, **Kuo KHM**, Ward R. Single-Centre, North American Experience with Compassionate Use of Deferiprone in Patients with Beta-Thalassemia Major. Blood. 2011 Nov;118:3185. **Coauthor or Collaborator**.

2011 Nov EspP, An Extracellular Serine Protease From Enterohemorrhagic E. Coli, Induces Coagulopathy In Human Whole Blood and Plasma. Presenter(s): **Kuo KHM**, Khan S, Brnjac E, Pai EF, Chesney AE.

Publication Details:

Kuo KHM, Khan S, Brnjac E, Pai EF, Chesney AE. EspP, An Extracellular Serine Protease From Enterohemorrhagic E. Coli, Induces Coagulopathy In Human Whole Blood and Plasma. Blood. 2011 Nov;116(21):4416. **Principal Author**.

Other Presentations

2019 May 10 **Invited Speaker**. Approach to Cancer-Related Anemias. Pri-Med Canada. Mississauga, Ontario, Canada.

H. Teaching and Design

My teaching responsibilities as an assistant professor in the Faculty of Medicine include the following:

1. supervision and education of clinical trainees at the post-graduate and fellowship level
 - outpatient clinic and inpatient consult service: internal medicine trainees on selective and elective rotations, subspecialty residents, hemoglobinopathy fellows
 - academic half day for hematology subspecialty residents
2. supervision and education in clinical research methodology to post-graduate specialty and subspecialty trainees, and fellows
 - internal medicine and subspecialty residents on research electives
 - lecturer on observational studies methodology, health measurement during the hematology and medical oncology subspecialty resident research rotation
 - coordinator of the hematology and medical oncology subspecialty resident research rotation.

1. INNOVATIONS AND DEVELOPMENT IN TEACHING AND EDUCATION

2014 Jan - 2014 Jul Decoding hemoglobinopathies: a self-learning module, Multilevel Education, Faculty of Medicine, Dept of Medicine, Haematology, University of Toronto
A six-module self-learning module on the diagnostic techniques, laboratory interpretation and management of hemoglobinopathies and red blood cell disorders for subspecialty residents in hematology and clinical fellows in red blood cell disorders.
Material constructed based on feed back from trainees
Complemented the trainees' laboratory component of their red blood cell disorders rotation
Enabled and accelerated the trainees' training in diagnosis and management of hemoglobinopathies.

I. Research Supervision

1. PRIMARY OR CO-SUPERVISION

Undergraduate MD

2016 Jan - 2016 Dec **Primary Supervisor.** Year 2. Dhoot, Arti. Supervisee Position: EEE-R Medical Student Research, Supervisee Institution: University of Toronto. *A Cross-sectional Comparison of the Burden and Prevalence of White Matter Changes in Young adults and Adults With and Without Sickle Cell Disease, Non-thesis Project.* Collaborator(s): Lacasse M-C, Mandell D, Rawal S, Jaigobin C, Ward R.

2015 Jul - 2015 Dec **Primary Supervisor.** Year 2. Dhoot, Arti. Supervisee Position: EEE-R Medical Student Research, Supervisee Institution: University of Toronto. *A Cross-sectional Comparison of the Burden and Prevalence of White Matter Changes in Young adults and Adults With and Without Sickle Cell Disease, Non-thesis Project.* Collaborator(s): Mandell D, Rawal S.

Postgraduate MD

2021 Feb - 2023 Dec **Primary Supervisor.** Core Program. Solow, Marissa. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *The Role of Microvasculopathy in the Development of Neurocognitive Dysfunction in Patients with COVID-19, Non-thesis Project.* Supervisor(s): **Kuo KHM.**

2018 Sep - 2023 Jun **Primary Supervisor.** Core Program. Rotin, Lianne E. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *A cohort study on hematopoietic stem cell transplant versus standard medical therapy in young adults with sickle cell disease, Non-thesis Project.* Supervisor(s): **Kuo KHM.** Collaborator(s): Rotin LE, Viswabandya A, Kumar R,

Kuo KHM.

- 2018 Aug - 2020 Jul **Primary Supervisor.** Clinical Fellow. Forte, Stephanie. Supervisee Position: Clinical Fellow, Supervisee Institution: University Health Network. *A multicenter study evaluating the reliability and validity of the Rowland Universal Dementia Assessment Scale (RUDAS) and predictors of RUDAS performance in adults with Sickle Cell Disease, Non-thesis Project.* Collaborator(s): Ward R.
- 2018 Jan - 2019 Dec **Primary Supervisor.** Clinical Fellow. Aneke, John C. Supervisee Position: Clinical Fellow, Supervisee Institution: University Health Network. *Optimizing red blood cell exchange transfusion in sickle cell disease, Non-thesis Project.* Supervisor(s): Ward R.
- 2018 Jan - 2018 Feb **Primary Supervisor.** Core Program. Iaboni, Aled. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *Redefining Maximum Tolerated Dose of Hydroxyurea in Sickle Cell Disease Using the Reticulocyte Production Index, Non-thesis Project.* Supervisor(s): **Kuo KHM.** Collaborator(s): Ward R.
- 2016 Jan - 2018 Jun **Primary Supervisor.** Core Program. Rosart (nee Smith), Rebecca. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *Genetic associations with clinical outcomes in adult SC disease patients, Non-thesis Project.* Collaborator(s): Binding A, Tomlinson G, Ward R.
- 2016 Jan - 2017 Dec **Co-Supervisor.** Clinical Fellow. Lacasse, Marie-Constance. Supervisee Position: Clinical fellow, Supervisee Institution: University of Toronto. *A Cross-sectional Comparison of the Burden and Prevalence of White Matter Changes in Young adults and Adults With and Without Sickle Cell Disease, Non-thesis Project.* Collaborator(s): Mandell D, Rawal S, Jaigobin C, Ward R.
- 2016 Jan - 2017 Jun **Primary Supervisor.** Core Program. Hammond, Danielle. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *Neurocognitive function and testing in sickle cell disease patients, Non-thesis Project.* Collaborator(s): Binding A, Jaigobin C, Ward R.
- 2016 Jan - 2016 Oct **Primary Supervisor.** Clinical Fellow. Binding, Andrew. Supervisee Position: Clinical Research Fellow, Supervisee Institution: University of Toronto. *Impact of high dosage deferiprone on liver iron concentration in transfusion-dependent hemoglobinopathy patients, Non-thesis Project.* Collaborator(s): Tomlinson G, Ward R.
- 2016 Jan - 2016 Oct **Primary Supervisor.** Clinical Fellow. Binding, Andrew. Supervisee Position: Clinical Research Fellow, Supervisee Institution: University of Toronto. *Neurocognitive function and testing in sickle cell disease patients, Non-thesis Project.* Collaborator(s): Ward R, Rawal S, Mandel D, Jaigobin C.
- 2016 Jan - 2016 Oct **Primary Supervisor.** Clinical Fellow. Binding, Andrew. Supervisee Position: Clinical Research Fellow, Supervisee Institution: University of Toronto. *Genetic associations with clinical outcomes in adult SC disease patients.* Collaborator(s): Ward R, Lettre G.
- 2015 Jul - 2015 Dec **Co-Supervisor.** Clinical Fellow. Lacasse, Marie-Constance. Supervisee Position: Clinical fellow, Supervisee Institution: University of Toronto. *A Cross-sectional Comparison of the Burden and Prevalence of White Matter Changes in Young adults and Adults With and Without Sickle Cell Disease, Non-thesis Project.* Collaborator(s): Mandell D, Rawal S.
- 2015 Jul - 2015 Dec **Primary Supervisor.** Core Program. Smith, Rebecca. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *Genetic associations with clinical outcomes in adult SC disease patients, Non-thesis Project.* Collaborator(s): Binding A, Tomlinson G, Ward R.
- 2015 Jul - 2015 Dec **Primary Supervisor.** Core Program. Hammond, Danielle. Supervisee Position: PGY2, Supervisee Institution: University of Toronto. *Neurocognitive function and testing in sickle cell disease patients, Non-thesis Project.* Collaborator(s): Jaigobin C, Ward R.
- 2014 Jul - 2015 Jun **Primary Supervisor.** Core Program. Parmar, Ambika. Supervisee Position: Resident, General Internal Medicine, Supervisee Institution: University of Toronto. *Osteonecrosis in Sickle Cell Disease: A Systematic Review, Non-thesis Project.*
- 2014 Jul - 2015 Jun **Primary Supervisor.** Subspecialty. Kopolovic, Ilana. Supervisee Position: Subspecialty Resident, Supervisee Institution: University of Toronto. *Management of Cancer-Associated Thrombosis with Concomitant Thrombocytopenia: A Survey of Canadian Hematologists,*

Non-thesis Project.

- 2012 Jun - 2014 Dec **Primary Supervisor.** Core Program. Mian, Hira S. Supervisee Position: Specialty Resident, Supervisee Institution: University of Toronto. *Optimal Manual Exchange Transfusion Protocol for Sickle Cell Disease: A Retrospective Comparison of Two Comprehensive Care Centers in United Kingdom and Canada, Non-thesis Project.*
- 2012 Jun - 2014 Dec **Primary Supervisor.** Core Program. Mian, Hira S. Supervisee Position: Specialty Resident, Supervisee Institution: University of Toronto. *Characterization Of Pulmonary Compliance In Sickle Cell Patients Revealed Wide Variability, Non-thesis Project.*

Faculty Development

- 2021 Jan - present **Co-Supervisor.** Assistant Professor. Abdulrehman, Jameel. Supervisee Position: Clinician-Investigator, Supervisee Institution: University Health Network. *THromboprophylaxis In Sickle Cell Disease with Central Venous Catheters (THIS): A Pilot Study, Non-thesis Project.* Supervisor(s): Carrier M. Collaborator(s): Forté S, Tomlinson G, Carrier M, Bartolucci P, Naessens V.
- 2019 Sep - 2020 Dec **Co-Supervisor.** Clinical Associate. Abdulrehman, Jameel. Supervisee Position: Clinical Fellow, Supervisee Institution: University Health Network. *THromboprophylaxis In Sickle Cell Disease with Central Venous Catheters (THIS): A Pilot Study, Non-thesis Project.* Supervisor(s): Carrier M. Collaborator(s): Forté S, Tomlinson G, Carrier M, Bartolucci P, Naessens V.

Clinical Research Fellow (MD)

- 2014 Sep - 2015 Dec **Primary Supervisor.** Binding, Andrew. Supervisee Position: Clinical Research Fellow, Supervisee Institution: University of Toronto. *Neurocognitive function and testing in sickle cell disease patients, Non-thesis Project.* Collaborator(s): Ward R, Rawal S, Mandel D.
- 2014 Sep - 2015 Dec **Primary Supervisor.** Binding, Andrew. Supervisee Position: Clinical Research Fellow, Supervisee Institution: University of Toronto. *Genetic associations with clinical outcomes in adult SC disease patients.* Collaborator(s): Ward, Richard.
- 2014 Aug - 2015 Jul **Primary Supervisor.** Sabbatical. Mulla-Ali, Dalal H. Supervisee Position: Assistant Professor, Supervisee Institution: University of Kuwait. *Management of Osteonecrosis in Sickle cell Adult and Pediatric Providers (MONSAPP survey), Non-thesis Project.*
- 2014 Aug - 2015 Jul **Primary Supervisor.** Sabbatical. Mulla-Ali, Dalal H. Supervisee Position: Assistant Professor, Supervisee Institution: University of Kuwait. *Osteonecrosis in Sickle Cell Disease: A Systematic Review, Non-thesis Project.*
- 2012 Jul - 2014 Jul **Primary Supervisor.** Trivieri, Maria G. *The use of the Calcium Channel Blocker Amlodipine as an adjuvant treatment to iron chelation for the prevention of Iron Overload Cardiomyopathy (CANALI study), Non-thesis Project.* Awards: Thalassemia Foundation Canada Fellowship Research Grant. Supervisor(s): Ward R. Collaborator(s): Ross H, Farkouh M, Backx PH, Nguyen E, Thorpe K.

2. OTHER SUPERVISION

Graduate Education

Thesis Committee Member

- 2024 Jan - present **MSc.** Hillis, Lindsay. *Investigating the Role of M1/M2 Macrophages in Sickle Cell Anemia.* Supervisor(s): Branch, Donald.
- 2023 Dec - present **PhD.** Doeven, Thomas. *A Multicenter, Single-Arm Phase 2 Trial of Mitapivat in Adult Patients with Erythrocyte Membranopathies and Congenital Dyserythropoietic Anemia Type II (SATISFY-EU).* Supervisor(s): van Beers, Eduard.
- 2021 Nov - 2024 Jan **MSc.** Diatlov, Daniel. *Investigating the role of the complement system in pediatric patients*

with sickle cell disease. Supervisor(s): Licht, Christoph.

2021 Jul - 2023 Jun **MSc.** Diatlov, Daniel, Medical Science. *Investigating the Role of Complement in Sickle Cell Disease*. Supervisor(s): Licht, Christoph. Completed 2023.

Clinical Research Fellow (MD)

Secondary Supervisor

2012 Oct - 2014 May Naessens, Veronique. Supervisee Position: Clinical fellow, Supervisee Institution: University Health Network. *Efficacy of Emergency Department Protocol for Managing Acute Sickle Cell Crisis, Non-thesis Project*. Supervisor(s): Ward R. Completed 2015.

2012 Oct - 2014 May Naessens, Veronique. Supervisee Position: Clinical fellow, Supervisee Institution: University Health Network. *SC project, Non-thesis Project*. Supervisor(s): Ward R. Completed 2015.

J. Creative Professional Activities

1. PROFESSIONAL INNOVATION AND CREATIVE EXCELLENCE

2017 Jan - present Health Research Methodology Education and Development.
My CPA focus on health research methodology education, in overcoming logistical barrier in executing clinical trials in rare diseases. Driven by my experience, I created the "Methodology Café" at UHN where junior investigators can discuss logistical issues faced in executing clinical trials in a stress-free and informal environment. Since taking over as the rotation coordinator of the research rotation for PGY4 hematology and medical oncology trainees, I have widened the scope of the trainee projects to include education research, quality improvement, and health technology assessment. I have demonstrated national impact in my service as a member on the CIHR project grant review panel for the past 3 years and as invited speakers on clinical research in rare hematologic diseases in various national organizations (CORD, NRBDO, SCDAC, TFC). I have also made international impact by serving as a member of the trial steering committee in the international pyruvate kinase registry, and on the data and safety monitoring committee of an international phase 1 gene therapy study in sickle cell disease (as chair), and the Comprehensive Anaemia Programme and Personalized Therapies (CAPPT) Trial being conducted in UK, India, and Nepal.

2014 Jan 1 - present Creation of the Red Blood Cell Disorders Research Unit at UHN.
Since my appointment as clinician-investigator at UHN in 2014, I have created a clinical trial infrastructure for the investigation of rare blood disorders. It has now grown from one investigator with two clinical trials to 5 active investigators with 9 research staff dedicated to rare blood disorders, with 23 active interventional trials (investigator initiated and industry), 20 active observational studies, 24 completed trials, and an "evergreening" trial strategy to ensure sustainability. Two clinical investigators from apheresis/complement disorders and thrombosis have since adapted my clinical trial infrastructure and joined my group as investigators. One of my mentees (Stephanie Forte), who is now a clinical investigator and assistant professor at Université de Montréal, has modelled her clinical trial infrastructure after mine and is now garnering awards and funding.

2014 Sep - 2019 Jun Allogeneic stem cell transplantation for hemoglobinopathies.
Championed the provision of funding to provide allogeneic stem cell transplantation to adults patients with sickle cell disease and thalassemia.
Positive response from Cancer Care Ontario and will move to implementation phase