Colorectal and Pelvic Reconstruction Service

CPRS

MELBOURNE ● AUSTRALIA

Research Newsletter

2023

murdoch children's research institute

The Royal Children's Hospital Melbourne

THE UNIVERSITY OF MELBOURNE

Contents

Dear CPRS patients and families,

On behalf of the Colorectal and Pelvic Reconstruction Service (CPRS) Research Team, we would like to thank you for generously supporting our research. Your support makes it possible for us to do this work.

This newsletter tells you about each project, including progress updates and results. We hope this information will be useful to you, regardless of whether you have participated in this research.

If you have any feedback, questions, or if you would like to be removed from the distribution list, please email us: colorectal.research@rch.org.au.

We will aim to distribute this newsletter once each year.

CPRS Research Team

Quality of Life

Children born with complex colorectal conditions: patient and parent outcomes

ARM HD

Overview

Children born with an anorectal malformation (ARM) or Hirschsprung disease (HD) usually need an operation in the first year of life. They also need ongoing medical attention. This experience can be challenging for both the child and their family.

This project will help us better understand the impact ARM and HD has on the child and their family.

Aims

- To explore the quality of life of children born with ARM or HD.
- To explore the quality of life, experience of illness, and coping for parents of children born with ARM or HD.

Study Design

Longitudinal (multiple years)

Online parent survey

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- We will continue to collect data for this project every year
- Participation:
    - **2019** = 61 ARM, 40 HD
    - **2020** = 67 ARM, 49 HD
    - **2021** = 67 ARM, 45 HD
    - **2022** = 49 ARM, 34 HD
    - **2023** = 52 ARM, 40 HD

Findings to Date

Emotional wellbeing

- We found that parents had normal levels of anxiety and depression. However, parents' anxiety levels were higher in 2020.

Experience of management and adjustment

- Managing an ARM or HD caused changes to the everyday life of parents and their families. However, we found that parents adjusted over time and were able to incorporate the management of ARM and HD into their everyday life.
- We found that caring for and adjusting to an ARM or HD could be more difficult for parents of children who were: under the age of three, girls, had VACTERL conditions, had severe HD or ARM types, or had a stoma.

> “Parents adjusted over time and were able to incorporate the management of ARM and HD into their everyday life.”

Project Team

Investigators: Professor Sebastian King, Dr Misel Trajanovska, Associate Professor Frank Muscara | **Research:** Ms Helena Parthimos | **Honours students:** Mr Dean Mandilas, Ms Charlotte Flook

Funding: CPRS Federal Grant

Publications

- Koo FEC, Chan MCE, King SK, Trajanovska M. The early years: Hirschsprung disease and health-related quality of life. Qual Life Res. 2023;10.1007/s11136-023-03482-2. doi:10.1007/s11136-023-03482-22

Understanding the parental experience of the colorectal journey

ARM HD

Overview

Children born with HD or ARM, and their families, may experience more social and psychological difficulties than children without these conditions. This project will help us understand the experience of being a parent caring for a child with ARM or HD.

Aims

- To describe the healthcare experience of parents.
- To identify the child, parent and hospital factors associated with better or worse parental experiences in healthcare, when caring for a child with ARM or HD.
- To increase healthcare provider communication, support and education with patients and families in a targeted way.
- To identify ways to improve the way healthcare services are provided to this population.

Study Design

Parent interviews

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- We have completed 50 interviews with parents of children with ARM or HD.
- Data analysis is ongoing.

Project Team

Investigators: Professor Sebastian King, Professor Phil Dinning, Associate Professor Warwick Teague, Professor John Hutson, Dr Mark Safe | **PhD student:** Dr Hannah Evans-Barns

Funding: The Royal Children's Hospital Foundation, University of Melbourne PhD Scholarship, One in 5000 Foundation, MCRI Student Conference Support Award

Functional pathology of Hirschsprung disease

HD

Overview

HD is a condition where some nerves in the muscle of the bowel are missing. As a result, children with HD have trouble passing stool (poo). Even after surgery, these troubles can continue. This project will help us understand the biological and medical factors that negatively impact the bowel in children with HD.

Aims

- To know what cell types, other than neurons, are affected in the HD bowel.
- To compare bowel movement across regions of the HD bowel.
- To study immune cells of the bowel and blood to know why some children develop Hirschsprung Associated Enterocolitis.
- To assess genetic factors involved in the development of the HD bowel.

Study Design

Collection of tissue and blood samples

Laboratory-based investigation

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- Data collection and analysis is ongoing.

Project Team

SITE: The Royal Children's Hospital | **Investigator:** Professor Sebastian King

SITE: Monash Institute of Pharmaceutical Sciences | **Investigators:** Dr Simona Carbone | **PhD student:** Ms Ashleigh Gould

SITE: University of Melbourne | **Investigators:** Dr Lincon Stamp, Dr Marlene Ho

SITE: Murdoch Children's Research Institute | **Investigator:** Professor David Eisenstat

Funding: Brian Smith Endowment award, Takeda Pharmaceuticals, The Royal Children's Hospital Foundation

Publications

- Yang, W et al. A novel method for identifying the transition zone in long-segment Hirschsprung disease: Investigating the muscle unit to ganglion ratio. Biomolecules. 2022;12(8):1101. doi:10.3390/biom12081101

Colonic and anorectal motility in children with complex colorectal conditions

ARM HD

Overview

Even after surgery, many children with ARM and HD suffer from long term bowel dysfunction, which is usually faecal incontinence (soiling) and constipation. This is thought to be related to problems with the movement of the bowel. This project will help us understand bowel movement and its involvement in bowel dysfunction.

Aims

- To describe bowel movement patterns in children with bowel dysfunction after surgery for ARM or HD.
- To understand the surgery-related and biological factors that may be linked to the child's bowel movement after surgery.
- To compare movements between different patients with bowel dysfunction.

Study Design

Review of existing research

Testing children's bowel movement (manometry)

Patient quality of life survey

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- We have completed data collection and analysis is in progress.

Project Team

Investigators: Professor Sebastian King, Professor Phil Dinning, Associate Professor Warwick Teague, Professor John Hutson, Dr Mark Safe | **PhD student**: Dr Hannah Evans-Barns

Funding: The Royal Children's Hospital Foundation, University of Melbourne PhD scholarship, One in 5000 Foundation

Publications

- Evans-Barns HME, et al. Post-operative anorectal manometry in children following anorectal malformation repair: A systematic review. J Clin Med. 2023; 12(7):2543. doi: 10.3390/jcm12072543
- Evans-Barns HME, et al. Post-operative colonic manometry in children with anorectal malformations: A systematic review. Neurogastroenterol Motil. 2022; 34(12):e14415. doi:10.1111/nmo.14415
- Evans-Barns HME, et al. Post-operative anorectal manometry in children with Hirschsprung disease: A systematic review. Neurogastroenterol Motil. 2022;34(8):e14311. doi:10.1111/nmo.14311
- Evans-Barns HME, et al. Post-operative colonic manometry in children with Hirschsprung disease: A systematic review. Neurogastroenterol Motil. 2021;33(11):e14201. doi:10.1111/nmo.14201.

Vascular access in children with complex colorectal conditions

ARM HD

Overview

Surgically accessing the patient's vascular system (veins and arteries) are common procedures in paediatric care. We are looking at whether the access to these systems changes in children with ARM or HD after they have had surgery (reconstructive or stoma closure).

Aims

- To describe access to vascular structures in children with ARM or HD after reconstructive surgery or stoma closure.
- To evaluate differences in vascular access after surgery between different age groups and surgery types.
- To understand what factors are linked with successful vascular access.

Study Design

Audit of current practice

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- We have collected and analysed the data and are currently writing a report of the findings.

Project Team

Investigators: Professor Sebastian King, Professor Phil Dinning, Associate Professor Warwick Teague, Professor John Hutson, Dr Mark Safe | **PhD student**: Dr Hannah Evans-Barns

Funding: Philanthropic funding (via The Royal Children's Hospital Foundation), University of Melbourne PhD scholarship, One in 5000 Foundation

Service improvement

Anal dilatations for children with an anorectal malformation

ARM

Overview

Children born with ARM need anal dilatations to help the new anus heal after surgery. There is a lack of research into the parental experience of performing anal dilatations, and the supports that may be needed.

This study will explore the experience of anal dilatations from multiple perspectives.

Part A: Parental experience of anal dilatations

Aims

- To explore the parental experience of performing anal dilatations on infants with ARM.
- To assess the impact of a multi-disciplinary colorectal service and educational tool on parental experience of anal dilatations.

Study Design

Review of existing research

Parent self-report survey

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- Systematic review of current research in the area is complete and we are currently writing a report of the findings.
- Pilot testing for the parent survey is complete and ready to be sent to families.

Project Team

Investigators: Professor Sebastian King, Professor Fiona Newall, Dr Misel Trajanovska | **PhD student:** Ms Jessica Taranto

Funding: Elizabeth Fearon Scholarship, Australian Nurses Memorial Centre Scholarship, Philanthropic funding (via The Royal Children's Hospital Foundation)

Service improvement

Colorectal transition: paediatric to adult care

ARM HD CC

Overview

We are looking at how young people with complex colorectal conditions such as ARM, HD and chronic constipation (CC) transition into adult care. We want to find out how we can better support them with this transition.

Part A: Understanding transitional care

Aims

- To explore the barriers and facilitators of transition to adult care based on previous research.

Study Design

Review of existing research

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- A review of existing research in patients with ARM and HD has been completed and we are currently writing a report of the findings.
- Existing research in patients with CC is currently being done.

Project Team

Investigators: Professor Sebastian King, Professor Susan Sawyer, Dr Misel Trajanovska | **PhD student:** Ms Emma Moore

Funding: University of Melbourne PhD scholarship, Philanthropic funding (via The Royal Children's Hospital Foundation)

Service improvement

Health economic evaluation of the Colorectal and Pelvic Reconstruction Service

ARM HD

Overview

The project looks at the value of a new multidisciplinary service, like the CPRS, for children with complex colorectal conditions and their families. It will involve parents and the general public to explore challenges, opportunities, and individual preferences associated with a service for children with complex colorectal conditions. The cost-effectiveness of the service will also be evaluated.

Aim

- Part A of this project aims to identify service characteristics that are important to families of children experiencing complex colorectal conditions.
- Part B of this project aims to measure preferences and values for service characteristics from the general public.

Study Design

Parent group focus group interviews

Consumer survey

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- We have completed data collection from focus group interviews with families. These findings were used to develop a survey.
- The survey data has been collected and analysed from the general public. We are currently writing up a report of the findings.

Project Team

Investigators: Professor Sebastian King, Associate Professor Ilias Goranitis, Dr Misel Trajanovska, Associate Professor Stephanie Best | **Research:** Dr Yan Meng, Dr Tianxin Pan

Funding: CPRS Federal Grant, The Royal Children's Hospital Foundation

Service improvement

Multidisciplinary models of care for surgical colorectal conditions

ARM HD

Overview

Children with complex colorectal conditions need care by many medical and allied health specialists. Multidisciplinary health services have been shown to benefit patients with other paediatric conditions. However, these services are limited for complex colorectal patients in Australia.

Part A: Review

Aim

- To review multidisciplinary models for other paediatric surgical conditions and understand how they work.

Study Design

Review of existing literature

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- A review of existing models of care has been completed. We are currently writing up a report of the findings.

Part B: Evaluation

Aims

- To assess existing pathways for patients with complex colorectal conditions at RCH before and after the CPRS.
- To evaluate the medical outcomes of patients with ARM or HD before and after the CPRS was introduced.

Study Design

Audit of current practice

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- Data has been collected in partnership with the Centre for Health Analytics (RCH).

Project Team

Investigators: Professor Sebastian King, Dr Misel Trajanovska, Dr Mary White | **Masters student:** Dr Liesel Porrett

murdoch children's research institute

Service improvement

Psychological care of children and families in the Colorectal and Pelvic Reconstruction Service

ARM HD CC

Overview

There are currently no guidelines for the type of specialised psychological support needed for colorectal conditions at paediatric health services around Australia. This study will explore what psychological support parents and caregivers need within multidisciplinary paediatric care teams for complex colorectal conditions (ARM, HD and CC).

Part A: Review

Aim

- To create a survey that will assess families preferences for psychological care within the CPRS.

Study Design

Survey development and parent feedback

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Survey development

- This part of the project is complete.
- Data from the review was used to develop a survey for Part B.

Part B: Parent survey

Aim

- To know what families need for CPRS psychology support to increase their child's psychological wellbeing during their treatment.

Study Design

Online survey

Timeline / Progress Update

Project planning → Participant recruitment → Data collection → Data analysis → Report writing

- Data from parent surveys has been collected and analysed.
- We are currently writing up a report of the findings.

Part C: Parent perspectives of psychological care

Overview

This will build on previous parts of this project by exploring psychological standards of care for those with complex colorectal conditions (ARM, HD and CC). It will explore the impact of caring for a child with a complex colorectal condition and parent perspectives of care needs for themselves and their children.

Aims

- To explore the impact of diagnosis, hospitalisation and surgery on the parent and child, and the psychological care that parents perceive as important for their child.
- To explore what parents perceive is important psychological care for themselves and their child.

Study Design


Semi structured interviews

Timeline / Progress Update

- Interviews of parents in the HD cohort are complete and analysis is in progress.
- Recruitment of parents in the ARM cohort is ongoing.

Project team

Investigators: Dr Kim-Michelle Gilson, Professor Sebastian King, Dr Misel Trajanovska, Associate Professor Frank Muscara, Dr Alice Morgan | **Research:** Dr Penelope Hartmann, Ms Helena Parthimos | **Honours student:** Ms Lily Jackson-Martin

Funding: CPRS Federal Grant, MCRI theme funding

Service improvement

VACTERL screening in children with an anorectal malformation

ARM

Overview

Many patients with an ARM are also born with related difficulties, called VACTERL anomalies. It is well established that all patients diagnosed with an ARM should be checked, otherwise known as screened, for these anomalies shortly after birth. Screening includes renal, spinal, and cardiac imaging. We want to understand how well our current methods of screening achieve this purpose.

Aim

- To evaluate the current protocol used at RCH for screening VACTERL anomalies.

Study design

Audit of current practice

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- Data collection and analysis is complete, and a report has been written and published.
- The findings from this report were presented at the Pacific Association of Pediatric Surgeons (PAPS) Annual Scientific Meeting, 2022 (Quito, Ecuador).

Project Team

Investigators: Professor Sebastian King | **Research:** Dr Liesel Porret, Dr Penelope Hartmann | **PhD student:** Dr Hannah Evans-Barns

Funding: The Royal Children's Hospital Foundation, University of Melbourne PhD scholarship, One in 5000 Foundation.

Publications

- Evans-Barns HME, et al. Screening for VACTERL anomalies in children with anorectal malformations: outcomes of a standardized approach. J Pediatr Surg. 2023;58(7):1263-1268. doi:10.1016/j.jpedsurg.2023.01.051

Service improvement

Towards the Australian-New Zealand Congenital Colorectal Registry (ANZCCoRe)

ARM HD CC

Overview

International data have confirmed the value of rare disease registries, which is a collection of patient data. These registries can be used to help understand and improve clinical care and patient outcomes. The end goal of this project is to develop a multi-centre colorectal registry for ARM and HD patients within Australia and New Zealand (ANZCCoRe).

Part A: Quality review

Aim

- To understand what factors are associated with successful development, implementation and sustainability of patient registries.

Study Design

Analysis of existing registry data

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- Data collection and analysis have been completed and a report of the findings has been written.

Part B: Europe vs RCH

Aim

- To compare registry data and practices between centres included in the Anorectal Malformations Network (ARM-Net) and RCH.

Timeline / Progress Update

Project planning → Data collection → Data analysis → Report writing

- We have collected the data from the ARM-Net and RCH registries and are currently comparing the data.

Project team

Investigators: Professor Ivo de Blaauw, Professor Iris Van Rooij, Professor Sebastian King, Dr Misel Trajanovska | **PhD student:** Dr Isabel Hageman

Funding: Academy Ter Meulen Grant (The Netherlands)

Publications

- Hageman IC, et al. A systematic overview of rare disease patient registries: challenges in design, quality management, and maintenance. Orphanet J Rare Dis. 2023;18(1):106. doi:10.1186/s13023-023-02719-0