Two-Day Research Symposium 2015
Discipline of Paediatrics

Monday 16th November & Tuesday 17th November 2015
It gives me great pleasure to welcome you all to the Two-Day Research Symposium to open UNSW Paediatric Research Week for 2015.

This new format incorporates annual presentations from Higher Degree Research students within the Discipline of Paediatrics and Children’s Cancer Institute and provides a fantastic opportunity for these students to present their research to the broader medical community, while promoting collaborative opportunities between researchers within the School.

Thank you to Dr David Ziegler, Dr Michelle Farrar, Dr Keith Ooi, Dr Jennifer Lynch, Dr Tiina Jaaniste and Dr Ursula Sansom-Daly for giving keynote addresses and opening each of the sessions over the course of the two days. Also, to four of our Honours students who will present their research

A special thank you to Samantha McFedries for her hard work and outstanding effort in organising this event.

Congratulations to our Independent Learning Project (ILP) Finalists who will be presenting their research on Wednesday 18th November. We will be announcing the recipients of the two prizes (Overall Winner and People’s Choice), following the presentations. We will also be announcing the winner of the Margaret Dance Prize for the highest ranked BSc (Med) Hons student in the Discipline of Paediatrics for 2014.

I’d like to draw your attention to the other events happening this week:

- The 4th Annual Health Science Alliance Scientific Symposium is being held on Thursday 19th November at the Royal Hospital for Women Lecture Theatre. This inaugural symposium will feature three sessions in Gynaecological Cancer, Research in Patient-Centred Care and Primary Care.
- The 43rd Annual Tow Research Awards Day is being held on Friday 20th November and is a great opportunity to hear presentations from early researchers and junior clinicians from across the Randwick Hospital campus, concluding with an Awards ceremony.

Once again, thank you for joining us and showing your support for paediatric research within the Discipline of Paediatrics and across the Randwick Hospitals Campus.

Professor Adam Jaffe

John Beveridge Professor of Paediatrics
Head of Discipline of Paediatrics
School of Women’s & Children’s Health

Associate Director of Research
Sydney Children’s Hospitals Network (Randwick)
SNAPSHOT: DISCIPLINE OF PAEDIATRICS

IN BRIEF

The Discipline of Paediatrics is part of the wider School of Women’s & Children’s Health and a department within UNSW Medicine, The University of New South Wales. The Discipline of Paediatrics is involved with the teaching of undergraduate medical students including supervision of Honours and Independent Learning Projects; and postgraduate supervision of higher degree candidates – PhD, Masters of Science, and Masters of Medicine. The School of Women’s & Children’s Health also offers two Masters by coursework programmes, both of which the Discipline is involved with: 1) Masters of Reproductive Medicine; and 2) Masters of Women’s Health Medicine. The Discipline supports and encourages the research activities of clinical academics, hospital scientists, allied health, and nursing staff.

LOCATION

Located at the Randwick Hospital Campus, the heart of the Discipline of Paediatrics is at Sydney Children’s Hospital. However, teaching and research is not only limited to this Campus and Hospital – staff are also based at the Royal Hospital for Women, St George, Bankstown and Liverpool. The Discipline also contributes to the teaching of paediatrics at Sutherland and Campbelltown Hospitals; Albury-Wodonga, Wagga Wagga, Port Macquarie, and Coffs Harbour Base Hospitals and campuses of UNSW’s Rural Clinical School.

RESEARCHERS

Researchers within the Discipline of Paediatrics are contributing both nationally and internationally with novel and innovative discoveries and interventions in behavioural sciences, cancer, endocrinology, gastrointestinal, genetics & genomics, immunology & infectious diseases, neonatology, nephrology, neuroscience, population health, and respiratory – as well as other priority-research areas.

The Discipline of Paediatrics is comprised of academic, professional and technical, research support, operational, and conjoint appointments. The combined School of Women’s & Children’s Health currently has 48 research and teaching academic staff.

Conjoint staff are also an extremely valuable research and teaching resource to the Discipline of Paediatrics. Approximately two-thirds of the research output from the Discipline is generated from UNSW conjoint staff. Conjoint staff are defined as hospital employees who have an honorary appointment at UNSW.

The Discipline comprises clinical academics, lab-based hospital scientists, allied health and nursing staff.

In October 2015, the Discipline of Paediatrics had a total of 166 conjoint appointments, lecturer level and above. Of these, 132 were involved in research and
teaching, or research only activities within the Discipline.

**HIGHER DEGREE RESEARCH CANDIDATES**

There are currently 54 higher degree candidates enrolled in the Discipline of Paediatrics. Children’s Cancer Institute, although an independent institute, also enrols its research students through the Paediatrics. At present, 26 students are supervised by CCI, the remaining 28 are supervised by the Discipline. In 2015, we had 9 students commence higher degrees with the Discipline of Paediatrics and CCI; and a further 10 submit their theses and have their degree conferred.

**GRANTS**

In 2015, researchers in the Discipline of Paediatrics, UNSW have been affiliated with at least $7 million worth of successful funding applications. Please note that not all of these grants are administered by the Discipline. Our researchers collaborate with many institutions including University of Sydney, University of Melbourne, Murdoch Children’s Research Institute, and the University of Adelaide - amongst others.

Organisations who have awarded funding have included the National Health and Medical Research Council (NHMRC), the Australia Research Council (ARC), Cancer Institute NSW, and the National Institutes of Health (NIH) in the US. Our researchers were also fortunate to receive two donations from philanthropic foundations.

**PUBLICATIONS**

In 2014, the School of Women’s & Children’s Health researchers authored approximately 287 publications. Of these, 207 met the criteria of the Higher Education Research Data Collection (HERDC). These are approximate figures and may change when final data is released by UNSW.

To date, the Discipline’s researchers have authored approximately 232 publications in 2015.

**WHAT IS HERDC?**

The Higher Education Research Data Collection (HERDC) comprises research income and research publications data, provided annually by Australian universities.

The specifications for what can be included in the HERDC are updated every year by the Department of Industry, Innovation, Climate Change, Science, Research and Tertiary Education in consultation with universities and key stakeholders.

Ultimately, the HERDC is designed to ensure the Australian Government’s research block grants are allocated in a fair and transparent way to universities.
RESEARCH WEEK ACKNOWLEDGMENTS

A large amount of work goes into organising Research Week and we would like to acknowledge and thank the following people for giving up their time and sharing their expertise.

RESEARCH WEEK WORKING GROUP

- Prof Adam Jaffe
- Prof Anne Cunningham
- Prof Maria Craig
- Conjoint Prof John Ziegler
- Conjoint A/Prof Julee Oei
- Conjoint A/Prof Avi Lemberg
- Conjoint A/Prof Charles Verge
- Conjoint A/Prof Tracey O’Brien
- Dr Michelle Farrar
- Dr Susan Adams
- Dr Keith Ooi
- Dr Belamy Cheung
- Dr Tejaswi Kandula
- Dr Nusrat Homaira
- Dr Joanna Fardell
- Dr Christoph Camphausen
- Dr Sean Kennedy
- Laura Mitchell
- Samantha McFedries
- Melinda Bresolin
- Deborah Broder
- Isabel Lewis
- Sara Savige

HIGHER DEGREE RESEARCH COORDINATORS

- Prof Richard Lock
- Dr Michelle Henderson
- Dr Amanda Philp
- Melinda Bresolin

ILP AWARDS ABSTRACTS & ORAL PRESENTATION JUDGES

- Conjoint A/Prof Gad Kainer
- Conjoint A/Prof Edwin Kirk
- Dr Sean Kennedy
- Dr Michelle Farrar
- Dr Steven Leach
- Dr Michael Cardamone
- Dr Rebecca Spicer
### DAY ONE - MONDAY 16TH NOVEMBER 2015

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<tr>
<th>Time</th>
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<tbody>
<tr>
<td>8:50 AM</td>
<td>Opening Address: <strong>Professor Adam Jaffe</strong>, Head, Discipline of Paediatrics, School of Women’s &amp; Children’s Health</td>
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<td><strong>SESSION 1</strong> Chair: <strong>Christine Gana</strong></td>
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| 9:00 AM | KEYNOTE ADDRESS: **Dr David Ziegler**, Kids Cancer Centre, Sydney Children’s Hospital & Children’s Cancer Institute.  
         | ‘Novel Therapies for Childhood Cancer.’ |
| 9:25 AM | BSC (MED) HONS PRESENTATIONS:  
         | **Genevieve Ho** (Supervisors: Dr Michelle Farrar & Dr Michael Cardamone)  
         | ‘Myotonic Dystrophy in Childhood: Health Outcomes.’  
         | **Mona Sajeev** (Supervisors: A/Prof Claire Wakefield & Ms Jennifer Cohen)  
         | ‘A decision aid for nutrition support in paediatric oncology.’ |
| 9:50 AM | HIGHER DEGREE RESEARCH PRESENTATIONS:  
         | **Santosh Valvi** MSc Candidate (Supervisors: Dr David Ziegler & Dr Maria Tsoli)  
         | ‘Novel therapies for diffuse intrinsic pontine glioma (DIPG).’  
         | **Chi Yan Ooi** PhD Candidate (Supervisors: Dr Belamy Cheung & Dr Daniel Carter)  
         | ‘Identification and characterisation of microRNAs that interact with MYCN & its functional roles in neuroblastoma.’ |
| 10:40 AM | Morning Tea |
|       | **SESSION 2** Chair: **Walter Muskovic** |
| 11:00 AM | KEYNOTE ADDRESS:  
         | **Dr Michelle Farrar**, Neurology Department, Sydney Children’s Hospital & School of Women’s & Children’s Health, UNSW Medicine.  
         | ‘Spinal muscular atrophy: from genes to therapy. Approaches to translation.’ |
| 11:25 AM | **Dr Meredith Ward** PhD Candidate (Supervisor: Prof Anne Cunningham & Dr Nicole Jones)  
         | ‘Developmental neuroscience - brain stem cells in immature brain.’ |
| 11:50 AM | **Jixuan Gao** PhD Candidate (Supervisors: Dr Michelle Henderson & Prof Murray Norris)  
         | ‘Investigating the biological roles of ABCE1 and ABCF2 proteins in neuroblastoma.’  
         | **Mawar Karsa** PhD Candidate (Supervisors: Dr Michelle Henderson & Prof Richard Lock)  
         | ‘Novel therapies for high-risk leukaemia in children.’ |
| 12:40 PM | Lunch |
|       | **SESSION 3** Chair: **Dr Meredith Ward** |
| 1:30 PM | KEYNOTE ADDRESS:  
         | **Dr Keith Ooi**, Gastroenterology Department, Sydney Children’s Hospital & School of Women’s & Children’s Health, UNSW Medicine.  
         | ‘Inconclusive Diagnosis of Cystic Fibrosis after Newborn Screening.’ |
| 1:55 PM | **Keith Sia** PhD Candidate (Supervisors: Prof Richard Lock & Dr Sibasish Dolai)  
         | ‘Targeting CRLF2 in high-risk paediatric acute lymphoblastic leukaemia.’ |
| 2:20 PM | **Christine Gana** PhD Candidate (Supervisors: Dr Jamie Fletcher & Prof Michelle Haber)  
         | ‘Targeted Inhibition of Multidrug Resistance Protein 1 in neuroblastoma.’ |
| 2:45 PM | Afternoon Tea |
| 3:00 PM | Close |

**CHANGES TO PROGRAMME:**  
Prof Adam Jaffe will open the Two-Day Symposium  
Jayne Murray will present at 10:15 am on Tuesday 17th November 2015  
Santosh Valvi will present at 9:10 am on Monday 16th November 2015
DAY TWO - TUESDAY 17TH NOVEMBER 2015

SESSION 4  
Chair: Alistair Lum  
9:00 AM KEYNOTE ADDRESS:  
**Dr Jennifer Lynch**, Children’s Cancer Institute.  
‘Therapeutic targeting of G protein signalling to inhibit leukaemic stem cell activity.’  
9:25 AM BSC (MED) HONS PRESENTATIONS:  
**Kevin Jiang** (Supervisors: Dr Steven Leach & Conjoint A/Prof Daniel Avi Lemberg)  
‘A randomised controlled trial to test method of vitamin D supplementation and effect on disease activity in paediatric inflammatory bowel disease.’  
9:35 AM  
**Sarah Marokakis** (Supervisors: Dr Sean Kennedy & A/Prof Nadine Kasparian)  
‘Parents Perceptions of Counselling for Congenital Anomalies of the Kidney and Urinary Tract (CAKUT).’  
9:50 AM HIGHER DEGREE RESEARCH PRESENTATIONS:  
**Zara Ali** PhD Candidate (Supervisors: Dr Karen Mackenzie & Dr Greg Arndt)  
‘Telomerase insufficiency in human hematopoietic cells.’  
10:15 AM  
**Jayne Murray** PhD Candidate (Supervisors: Prof Murray Norris & Prof Michelle Haber)  
‘Genetic Suppressors of Neuroblastoma.’  
10:40 AM Morning Tea

SESSION 5  
Chair: Dr Yuliya Makeyeva  
11:00 AM KEYNOTE ADDRESS:  
**Dr Tiina Jaaniste**, Pain Research Unit, Sydney Children’s Hospital & School of Women’s & Children’s Health, UNSW Medicine.  
‘Ouch’: The ability of young children to give a meaningful self-report of pain.’  
11:25 AM  
**Dr Lisa Amato** MMed Candidate (Supervisors: Dr Jan Walker & Dr Kristen Neville)  
‘Infantile hypercalcaemia following maternal vitamin D supplementation.’  
11:50 AM  
**Fathalla Ali** PhD Candidate (Supervisors: A/Prof Kei Lui & Dr Steven Leach)  
‘The Early development of microbiota in premature infants.’  
12:15 PM  
**Tejaswi Kandula** PhD Candidate (Supervisors: Dr Michelle Farrar, Dr Susanna Park & A/Prof Arun Krishnan)  
‘Chemotherapy-induced peripheral neuropathy in the paediatric population: risk factors, assessment strategies and functional outcomes.’  
12:40 PM Lunch

SESSION 6  
Chair: Laura Mitchell  
1:30 PM KEYNOTE ADDRESS:  
**Dr Ursula Sansom-Daly**, Behavioural Sciences Unit, Kids Cancer Centre, Sydney Children’s Hospital & School of Women’s & Children’s Health, UNSW Medicine.  
‘From bench to computer screen: Implementing online psychological support for adolescent and young adult cancer survivors.’  
1:55 PM  
**Louisa Carrol** PhD Candidate (Supervisors: Prof Nadia Badawi & Conjoint A/Prof Julee Oei)  
‘Educational Outcomes of ‘high risk’ newborn infants.’  
2:20 PM  
**Dr Nusrat Homaira** PhD Candidate (Supervisors: Prof Adam Jaffe & Dr Tom Snelling)  
‘Modifiable risk factors for RSV hospitalization in children aged<2 years of age: a data linkage study.’  
2:45 PM  
**Walter Muskovic** PhD Candidate (Supervisors: Prof Maria Kavallaris & A/Prof Kerrie Mcdonald)  
‘Exploring the Therapeutic Potential of MicroRNAs in Glioblastoma.’  
3:10 PM Closing Address: Prof Richard Lock, Postgraduate Coordinator, Discipline of Paediatrics, School of Women’s & Children’s Health & Children’s Cancer Institute
Cure rates for childhood cancer have steadily increased since the first child was cured of leukaemia in the 1960s. Overall cure rates for all cancers in children now exceed 80%. This success has been achieved via a series of clinical trials over several decades that have optimised the delivery of chemotherapy and radiation therapy and incorporated the use of biological markers to improve patient stratification. However several challenges remain to further improve outcomes for children with cancer.

The majority of drugs currently used to treat childhood cancer are cytotoxic chemotherapeutic agents that were developed 50 years ago and are associated with significant acute and long term toxicities. Further, there are many high risk cancers that remain incurable, despite trials of multiple different chemotherapeutic combinations, and novel strategies are urgently needed for these children.

The translational research program at Sydney Children’s Hospital and the Children’s Cancer Institute, brings preclinical drug discoveries to the bedside of children with relapsed and refractory cancer. The presentation will highlight several areas of drug discovery that have led to the development of early phase clinical trials. We have shown that high levels of the enzyme ornithine carboxylase (ODC1) correlate with poor clinical outcomes in children with high-risk neuroblastoma. Targeting this protein with the ODC1 inhibitor DFMO, led to a profound anti-tumour effect in both in vitro and in vivo neuroblastoma models. These results have now been translated to clinical trial for children with relapsed and refractory neuroblastoma.

Other specific examples of translational research include the development of conjugated antibody therapy for high risk leukaemias; a drug screening program for high risk brain tumours and the implementation of a national personalised medicine program for children with relapsed malignancies. This research offers a unique opportunity to improve outcomes for children with cancer through the development of novel targeted treatment strategies.

Funding:
NHMRC, CINSW, SCHF, CBC, TCSN, Lions Club, KCP.
Dr. Michelle Farrar's research is focused on clinical pediatric neurology, neuromuscular disorders, and neurophysiology, aiming to further understand disease pathophysiology and develop treatment strategies. She is currently investigating the mechanisms and the possible prevention of neurodegeneration in spinal muscular atrophy and other inherited neuropathies. In addition, through collaborations with geneticists, she is developing next generation sequencing to improve diagnostic approaches in neurological disorders.

Spinal muscular atrophy (SMA) is a devastating genetic neuromuscular disorder, producing significant disability and mortality. While the most common causative gene was identified 20 years ago, resulting from mutations involving the survival motor neuron 1 (SMN) gene, SMA remains without disease modifying therapy.

Understanding the unique genetic structure of the SMN region (an inverted duplication) has provided a strategy for therapeutic development, and extensive efforts are being undertaken to expedite treatments. SMA is characterised by considerable genetic and clinical heterogeneity, which presents challenges in measuring disease progression and developing outcome measures to facilitate clinical trials and novel therapeutics.

This presentation incorporates clinical, neurophysiological, and genomic research studies to provide an overview of current understandings of SMA, including pathogenesis, natural history, and the development of outcome measures. Furthermore, we are part of the first-ever Phase 3 clinical trial for a drug developed specifically to treat the underlying cause of SMA. Looking towards new disease modifying treatments for SMA prompts examination of regulatory, health economic, and ethical issues.

Taken together these studies are designed to translate advances in understanding the molecular and biological basis of SMA to reach the ultimate goal of finding an effective treatment in the clinic.

Funding:
Thyne Reid Foundation

Acknowledgments:
Dr. Hugo Sampaio, Dr. Tony Roscioli, Dr. Hooi-Ling Teoh, Dr. David Mowat, Prof. Matthew Kiernan, Prof. Steve Vucic, A/Prof. Arun Krishnan. SCH Neuromuscular Team. SCH Clinical Research Centre.
INCONCLUSIVE DIAGNOSIS OF CYSTIC FIBROSIS AFTER NEWBORN SCREENING

Co-Authors: Carlo Castellani; Katherine Keenan; Julie Avolio; Sonia Volpi; Margaret Boland; Tom Kovesi; Candice Bjornson; Mark A Chilvers; Lenna Morgan; Richard van Wylick; Steven Kent; April Price; Melinda Solomon; Karen Tam; Louise Taylor; Kylie Ann-Malitt; Felix Ratjen; Peter R. Durie; Tanja Gonska

Infants with an inconclusive diagnosis of cystic fibrosis (CF) after newborn screening may turn out to have CF. However, little is known about the incidence, characteristics (phenotype and genotype) and outcomes of these infants to guide investigations and follow-up. Different terminologies have also been given to describe this group of infants, including “CFTR-related metabolic syndrome (CRMS)” and “CF Screen Positive, Inconclusive Diagnosis” (CFSPID).

Hence a prospective, longitudinal, multicentre study was performed to identify and prospectively evaluate CFSPID infants. In this study, a proportion (11%) of infants with an initial inconclusive diagnosis were subsequently diagnosed with CF within the first 3 years, underscoring the need for follow-up of this population.

Funding:
Cystic Fibrosis Canada
Acute myeloid leukaemia (AML) is characterised by a high rate of disease relapse. While current treatments kill the bulk of tumour cells, they ultimately fail to induce durable clinical responses due to the persistence of leukaemic stem cells (LSCs) which possess self-renewal capacity.

We have previously identified the G protein coupled receptor, Lgr4, as a critical regulator of LSC activity. Interference with Lgr4 signalling can block the key stem cell regulator, β-catenin, and perturb leukaemia development. Here, we identify G protein alpha-q (Gnaq) as a major downstream component of the Lgr4 signalling network. Gnaq inhibition using an shRNA-induced gene silencing approach and a commercially available selective inhibitor significantly diminished LSC proliferation, self-renewal ability and induced apoptosis. Strikingly, LSCs that were pre-treated with Gnaq antagonist in culture revealed a significant reduction in short-term proliferation capacity in mice. Our data also showed that Gnaq inhibition perturbed β-catenin activity and significantly delayed leukaemia development in vivo.

Microarray analysis revealed that inhibiting Gnaq significantly reduced the expression of several genes responsible for maintenance of mitochondrial integrity and energy metabolism (e.g. mtND2 and mtCytB). A significant increase in reactive oxygen species production indicated that inhibiting Gnaq destabilises the mitochondrial membrane. Gnaq deficient LSCs had a significantly reduced rate of oxygen consumption and lower basal ATP levels compared to control LSCs. Gnaq inhibition, therefore, induces substantial oxidative stress which triggers the intrinsic apoptotic pathway. Our data suggest a novel LSC-eliminating treatment strategy targeting Lgr4/Gnaq/β-catenin signalling network.

Funding:
NHMRC, ARC, CCNSW and Balnaves Foundation
“OUCH”: THE ABILITY OF YOUNG CHILDREN TO GIVE A MEANINGFUL SELF-REPORT OF PAIN

Self-report is of crucial importance when assessing pain in children and adults. However, how young can we go? Clinicians often avoid asking for self-report in pre-school aged children because of frequent difficulties in understanding the scales: the responses often do not make sense to an adult.

Methods to obtain self-reports of pain intensity are highly developed for children aged 5 years and older, but research focusing on self-report in 3- and 4-year-olds is sparse. Most studies including 3- and 4-year-olds aggregate their data with those of older children, making it impossible to assess the suitability of the scales.

The self-report pain tools that are commonly used with older children, such as numbers, tokens, and pictures of faces, might not be the best way to ask about pain in children under 5. In particular they might offer too many alternatives, a concept that is supported by the frequently-reported observation that young children tend to use the top and bottom points of scales but not the middle points.

This presentation will consider the cognitive-developmental requirements for using self-report tools. The results of a recent systematic review of self-report measures used to assess pain intensity in 3- and 4-year-olds will be presented. Results from a study evaluating the use of simplified self-report tools with a community and clinical sample of pre-school aged children will also be presented.

Acknowledgments:
Collaboration with Prof. Carl von Baeyer (University of Manitoba, Canada) on various projects reported in this presentation is gratefully acknowledged. The work of Mathushinee Mohanachandran (Medical ILP Student, 2015) is also acknowledged.
Dr Ursula Sansom-Daly is a Cancer Institute NSW Post-Doctoral Research Fellow, at the Behavioural Sciences Unit, Kids Cancer Centre. She is also the Clinical Psychologist for Sydney Youth Cancer Service. Dr Sansom-Daly’s PhD at UNSW, was supported by the Leukaemia Foundation of Australia, and examined mechanisms of distress/adjustment among young cancer patients.

FROM BENCH TO COMPUTER SCREEN: IMPLEMENTING ONLINE PSYCHOLOGICAL SUPPORT FOR ADOLESCENT & YOUNG ADULT CANCER SURVIVORS

Cancer diagnosed during the adolescent and young adult (AYA) years complicates the peak time of mental-health risk. Recently, Internet-based psychological interventions have been hailed as a way to remove barriers to evidence-based psychological care. Assisting AYAs to develop helpful coping skills at the transition to survivorship may prevent later distress.

Our team developed an online intervention named ‘ReCaPTure LiFe’ (Resilience and Coping skills for young People To Live well Following Cancer) for AYAs aged 15-25 years in the first year post-treatment. This presentation will discuss the pathway to developing, evaluating, and implementing the Recapture Life program. From the identification of a gap in service delivery, the development of Recapture Life will be outlined.

Recapture Life is a manualised program that promotes resilience using evidence-based cognitive behavioural therapy techniques tailored to the AYA experience. It involves six, weekly, small-group sessions, delivered online by a psychologist using innovative video-conferencing technology. Preliminary results from the nation-wide, multisite, randomised controlled trial (RCT) evaluating the acceptability, feasibility, and efficacy of Recapture Life will then be discussed.

Recapture Life appears highly acceptable and feasible. Emerging quality of life data is also promising, indicating that Recapture Life assists AYAs to adjust well to life after cancer. The ethical and clinical challenges that emerged in delivering Recapture Life will also be discussed. Using a case-series methodology, ethical/clinical challenges will be analysed relative to international e-mental health guidelines. Finally, plans for implementing Recapture Life within community settings will be outlined, to transition this platform of evidence into wider practice.

Funding:
The Recapture Life study was co-funded by a beyond blue and Cancer Australia
project grant (ID: 1022868). Ursula Sansom-Daly is supported by an Early Career Fellowship from the Cancer Institute of New South Wales (ID: 14/ECF/1-11). Claire Wakefield is supported by a Career Development Fellowship from the National Health and Medical Research Council of Australia (APP1067501) and an Early Career Development fellowship from the Cancer Institute of NSW (ID: 11/ECF/3–43). The Behavioural Sciences Unit is supported by the Kids with Cancer Foundation.

**Acknowledgments:**

The authors wish to thank all the young people who have participated in the Recapture Life study. We acknowledge the contributions of Ms Sanaa Mathur, as well as the wider Recapture Life-AYA Working Party, including Ms Kate Thompson, Ms Lucy Holland, Dr Belinda Barton, Dr Robert Battisti, Ms Belinda Matigian, Ms Lyndal Gray, Dr Michael Osborn, Ms Meg Plaster, and Dr Marianne Phillips.
Objective: Myotonic Dystrophy (DM1) is an autosomal dominant neuromuscular disease that can manifest at any age with a wide range of outcomes and severity; however, current care guidelines may not address specific issues in childhood. The present study aims to profile the disease in surviving neonates and childhood-onset patients to provide recommendations for management specific to healthcare needs of this population. It hopes also to provide an overview of sleep disorders, particularly Excessive Daytime Sleepiness (EDS) in children with DM1 and its relationship to neuromuscular function and quality of life.

Methods: In a retrospective study, 40 patients with DM1 were studied over a mean of 12.8 years (Range: 2-19) in which 143 clinical parameters were recorded. These encompassed cognition, musculoskeletal, respiratory and gastrointestinal issues. Sleep disorders, Epworth Sleepiness Scale, quality of life and neuromuscular function were assessed cross-sectionally in 17 of the present DM1 cohort.

Results: Despite a “biphasic course” in congenital DM1 (CDM) whereby initial neonatal manifestations of significant hypotonia (n=19, 63.3%), feeding difficulties (n=17, 56.7%), bilateral talipes (n=17, 56.7%), polyhydramnios (n=13, 43.3%), ventilation assistance (n=13, 43.3%) improve over several weeks, CDM patients have persisting significant health impacts that are more marked than childhood/juvenile DM1 (JDM). There is a high incidence of cognitive impairment (86.7%), gross and fine motor delay (80%, 76.6%). Long-term medical issues arise from muscle weakness, GIT dysfunction and urinary incontinence. Similar concerns are reported for JDM patients but with lesser severity, and the main issue is cognitive and learning difficulties (90%). Assessment of sleep disorders in 17 current patients revealed 35.5% had abnormal scores on the modified Epworth Sleepiness scale, but there was no relationship between EDS and sleep disorders detected on polysomnography. We also observed that there is impairment of neuromuscular function (r=0.77, p<0.001) and quality of life with increased ESS (r=0.78, p<0.001).

Conclusions: There are specific health issues in children with DM1, distinct from adults, although adult symptoms arise later. We recommend management guidelines specific to childhood DM1 focused in six care areas: Cognition and development, educational support, respiratory care, gastrointestinal, sleep and musculoskeletal issues. Additionally, sleep is a common under-reported problem in children with DM1, with a substantial impact on function. Further studies to determine the effect of stimulant treatment are necessary to determine their benefit.
A DECISION AID FOR NUTRITION SUPPORT IN PAEDIATRIC ONCOLOGY

Background: Nutrition is critically important in paediatric oncology patients who can become malnourished as a result of their disease and treatment. However the decision to begin nutrition support and to decide which method of nutrition support can be very difficult for parents.

Objective: To develop and pilot test a decision aid (patient information booklet with an emphasis on decision making) for parents about nutrition support in paediatric oncology.

Design: A single-centre prospective pilot study. Participants were given the decision aid and a self-report questionnaire, which was collected later that day or mailed in.

Participants: Parents and clinicians of children who had been treated for cancer or a haematological condition in the past five years and had faced the decision to begin nutrition support were eligible for the study. Thirty-two parents (92% response rate) and ten clinicians (100% response rate) opted into the study.

Outcomes and Analysis: In parents, we investigated acceptability (7 items), usability (2 items) and comprehensibility (4 items) of the booklet. In clinicians, we investigated different aspects of the development of the booklet (4 items), as well as the booklet’s content and format (9 items), and its perceived usefulness to parents (11 items) and clinicians (10 items). Descriptive analysis was then conducted.

Results: Nineteen parent questionnaires (60%) and seven clinician questionnaires (70%) were returned. Parents’ responses to the booklet endorsed more than 75% positive responses, with the exception of length (68% of parents said the booklet was ‘just right’ while 32% said the booklet was ‘too long’). Similarly, all clinician items received more than 75% positive responses, with the exception of the item, ‘This booklet is not influenced by vested interests (71% agreed or strongly agreed, while 29% neither agreed nor disagreed) and ‘Using this booklet will save me time’ (66% agreed or strongly agreed, while 17% neither agreed nor disagreed and 17% disagreed). All parents and clinicians reported that they would recommend the booklet to others.

Conclusions: The response to the decision aid was highly positive, suggesting that the decision aid is acceptable, usable and comprehensible to our target population. With changes suggested by participants – removing repetition and increasing conciseness – we can move to testing effectiveness and feasibility with a randomised controlled trial.
A RANDOMISED CONTROLLED TRIAL TO TEST METHOD OF VITAMIN D SUPPLEMENTATION AND EFFECT ON DISEASE ACTIVITY IN PAEDIATRIC INFLAMMATORY BOWEL DISEASE

Background: Vitamin D is important for bone health in paediatric inflammatory bowel disease (IBD). It is not known whether daily low dose oral supplementation or one-off high dose stoss therapy is better at treating vitamin D deficiency in paediatric IBD patients. Vitamin D has also demonstrated anti-inflammatory actions in experimental colitis but human studies to date remain inconclusive.

Aims: Arm 1 - to assess the efficacy of daily dosing vs stoss therapy in raising and maintaining serum 25OHD levels in vitamin D deficient paediatric IBD patients. Arm 2 – to determine whether vitamin D supplementation improves disease activity in vitamin D sufficient paediatric IBD patients.

Methods: A two-armed parallel randomised controlled trial was performed to assess the above aims. 20 vitamin D deficient patients were included in arm 1 and 72 vitamin D sufficient patients were included in arm 2. Arm 1 recruitment is ongoing. Patients in arm 1 were randomised to receive either 2000IU vitamin D3 daily or stoss therapy. Patients in arm 2 were randomised to receive either no treatment or 2000IU vitamin D3 daily. The primary outcome measure for both arms was serum 25OHD levels. Secondary outcomes included serum inflammatory markers and disease activity index.

Results: Preliminary analysis for arm 1 shows that the stoss group had insignificantly higher serum 25OHD levels at 3 months (mean(\( \bar{x} \))= 99.33, standard deviation (s)=54.28) compared to the daily dosing group (\( \bar{x} = 68, s = 29.72 \)) (P= 0.43, n=6). Preliminary analysis for arm 2 shows that the daily dosing group had a significantly higher increase in serum 25OHD at 3 months (\( \bar{x} = +17.2\text{nmol/L} \)) (n=15) compared to the no treatment group (\( \bar{x} = -11.6\text{nmol/L} \))(n=16) (P= 0.008). No significant difference in the change in inflammatory markers and disease activity index at 3 months was found between the no treatment and daily vitamin D3 groups.

Conclusion: Preliminarily, it can be concluded that stoss therapy insignificantly maintains higher serum 25OHD levels at 3 months compared to daily vitamin D supplementation in vitamin D deficient paediatric IBD patients. Vitamin D sufficient patients supplemented with 2000IU vitamin D3 daily show significantly increased serum 25OHD levels compared to controls at 3 months but no significant difference in the change of their serum inflammatory markers and disease activity index.
OBJECTIVES: To explore the experiences and perceptions of parents of children with CAKUT regarding the counselling and information received following the diagnosis, and examine the potential influence of the severity of the anomaly on parental experiences and needs.

METHOD: Semi-structured telephone interviews were conducted with parents of children born with either posterior urethral valves (PUV) or multicystic dysplastic kidney (MCDK). Interviews were audio-taped and data analysed using a thematic approach. Following the interview, parents were asked to complete a demographic survey and the Depression, Anxiety and Stress Scale short version (DASS21).

RESULTS: A total of 8 parents of children with PUV and 9 parents of children with MCDK participated in the telephone interview. All parents received the diagnosis during the prenatal period (range = 12-38 weeks). Parents described the persistent uncertainty regarding their child’s condition and the need for early information. All parents received counselling either in the prenatal (n=14) or postnatal (n=3) period, most commonly from a nephrologist. Most were satisfied with the amount of information provided and the person conducting counselling, however wished to receive more detailed information on the prognosis/potential outcomes and treatment. A number parents suggested the way information is delivered should be improved, as well as the need for further emotional support through parent support groups, meeting with a social worker or counsellor, and online or written resources. The experiences and perceptions of parents were similar regardless of the severity of postnatal kidney disease.

CONCLUSION: Whilst most parents were satisfied with the counselling they received, there remains unmet information and emotional needs. Improvements to current counselling practice and the inclusion of further support services, such as support groups and allied health involvement, may address these gaps.