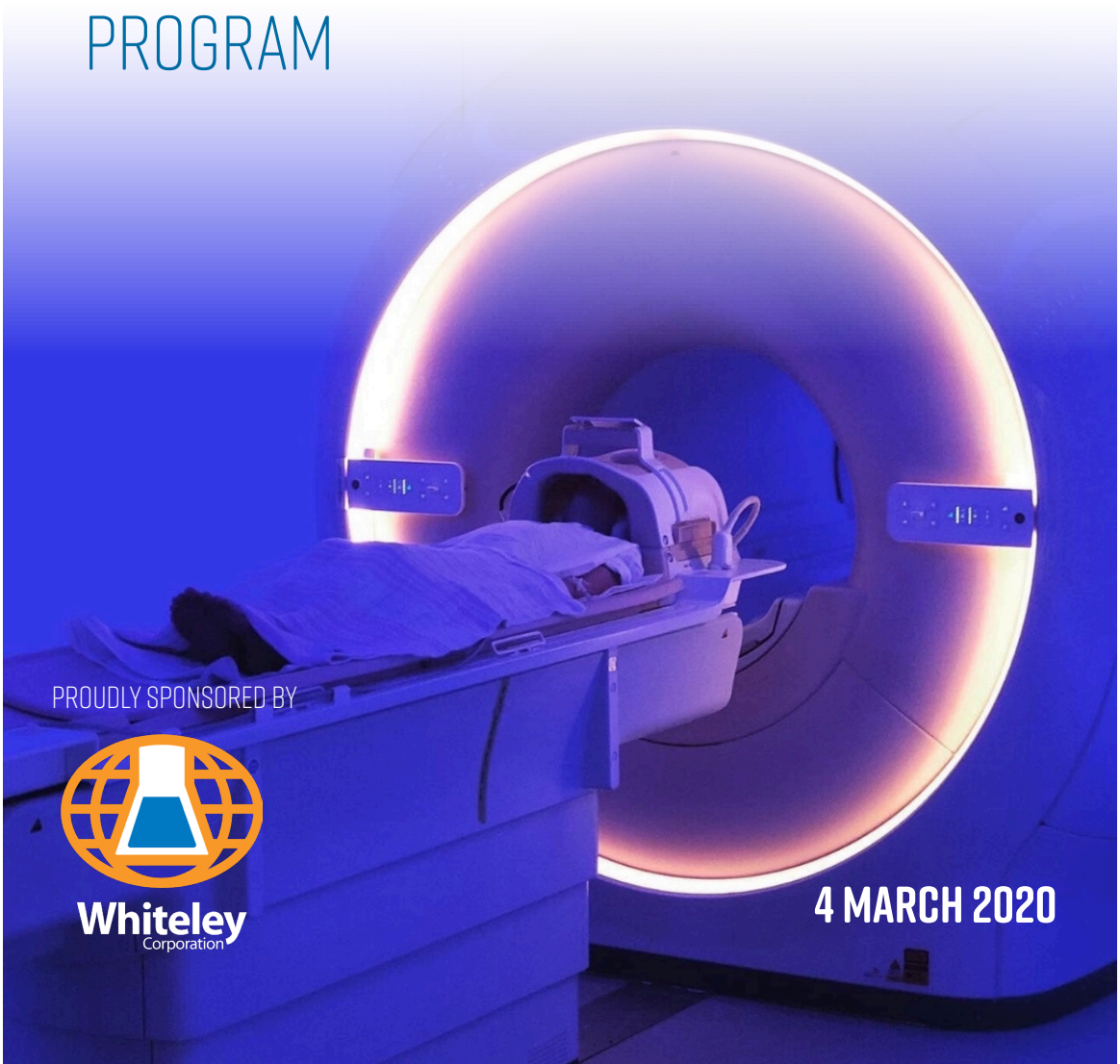
Children's Tumour Foundation
CONQUERING NF

THE CHILDREN'S TUMOUR FOUNDATION PRESENTS

2020 NF CLINICAL SYMPOSIUM

PROGRAM



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4 MARCH 2020

THANKYOU TO OUR

2020 SYMPOSIUM ORGANISING COMMITTEE

The 2020 Neurofibromatosis Symposium would not have been possible without the generous support of our sponsor Whiteley Corporation and the efforts of our Conference Committee members.

Committee Names:
Belinda Barton | Mimi Berman | Jonathan Payne | Louise Skilbeck |
Meredith Fannelli | Ruth Lindsay | Jessica Burgess | Jean Delany

We would like to acknowledge and thank all of our speakers who have generously given their time and expertise to present today and we look forward to an informative and enjoyable session.

We hope you will join us again in 2021/2022.

Our Symposium is being held on the traditional lands of the Gadigal people of the Eora Nation. We would like to pay our respects to the Elders past, present and emerging, as well as the Elders from other communities who may be represented here today.

Dear NF Symposium attendees,

On behalf of the Children's Tumour Foundation (CTF), I welcome you to the 2020 Neurofibromatosis Clinical Symposium.

The CTF is the leading and only national charity in Australia supporting those living with Neurofibromatosis (NF). We provide practical support and information for those impacted by NF at every stage of their journey. In addition to creating meaningful community connections and funding promising research, the CTF funds support coordinators in dedicated NF Clinics in Sydney and Melbourne with a goal to expand these services to every major hospital in the country.

Today, we are pleased to be able to gather Australia's best NF researchers and clinicians alongside some of their international counterparts to enable sharing of information and insights, while also providing networking opportunities that may ultimately help facilitate greater learning and development of treatment solutions.

We are thrilled to have two international speakers, Prof Michael Fisher and his colleague Dr Matthew Hocking, who are visiting from the US and appreciate the distance they've travelled to be here.

Today's program has two key areas of focus: updates on new and exciting research discoveries, and treatment options that seek to improve the health and wellbeing of people impacted by NF.

We thank you for your attendance and interest in enhancing your knowledge and treatment practices of NF. You can keep informed of the CTF news and events by subscribing to our newsletter or visiting our website at www.ctf.org.au.

Ensuring the NF community knows there is a safe and supportive space for them to ask questions, gather information and make connections is just as important as the treatment itself, so we would like to encourage you to share our details with your patients and their families. We can be reached at support@ctf.org.au or via our social media channels at @ctfaustralia.

Yours sincerely,

L Skilbeck

Louise Skilbeck
CEO, Children's Tumour Foundation

Children's Tumour Foundation
CONQUERING NF

NF SYMPOSIUM PROGRAM

Master of Ceremonies: Associate Professor Mimi Berman

TIME	SESSION	TOPIC	SPEAKER
9:00AM	**REGISTRATION & COFFEE**		
9:30am	Welcome	Welcome by CEO of the Children's Tumour Foundation and Symposium Sponsor	Louise Skilbeck Dr Greg Whiteley
		Welcome to Country	Dr Belinda Barton (SCHW)
9:45am	Keynote	Optic Pathway Gliomas in NF1	Prof Michael Fisher (CHOP)
10:40am	Plenary 1	Stemcell research applications towards NF	Dr Gautam Wali (USYD)
11:10AM	**MORNING TEA**		
11:30am	Plenary 2	MEK inhibitors in NF1 related tumours: update and future research trials	Dr Geoff McCowage (SCHW)
12:00pm	Plenary 3	Updates on L-Carnitine Therapy for NF1 and the Development of Gene Therapy Tools of NF treatment	A/Prof Aaron Schindeler (SCHW)
12:30pm	Plenary 4	Towards a vector-based universal genome editing approach for the treatment of NF1	Dr Samantha Ginn (MCRI)
1:00PM	**LUNCH**		
1:40pm	Plenary 5	The cognitive and behavioural development of children with NF1 from toddlerhood to school age	Dr Belinda Barton (SCHW)
2:10pm	Plenary 6	Social Skills and Autism Research	Dr Matthew Hocking (CHOP)
2:40pm	Plenary 7	Non-surgical NF2 treatment options	Dr Katrina Morris (RNS)

NF SYMPOSIUM PROGRAM

TIME	SESSION	TOPIC	SPEAKER
3:10pm	Plenary 8	Treatment of disfigurement in NF	A/Prof Mimi Berman with Dr Rebecca Saunderson (RNS)
3.40pm	Plenary 9	Insights into the implementation of a breast screening program for young women with NF1	Dr Mathilda Wilding (RNSH), Dr Katrina Morris (RNSH)
4.10pm	Plenary 10	NF2 Treatment Options	A/Prof Nigel Biggs (St Vincents)
4.50pm	Plenary 11	Auditory Processing Deficits in children with NF1	Alice Maier (MCRI)
5.20pm	Close	Symposium closed by the CTF Chair	Kerrie Kelly (CTF Chair)

5:30PM DRINKS & NETWORKING

Networking until 6:30pm

KEYNOTE SPEAKERS

PROF MICHAEL J FISHER, MD

Chief of Neuro Oncology, Children's Hospital of Philadelphia (CHOP) | Director of the Oncology Fellowship Program | Director of the Neurofibromatosis Program at CHOP

Dr Michael Fisher is Professor of Paediatrics (Perelman School of Medicine at the University of Pennsylvania), Chief of the Neuro-Oncology Section (Division of Oncology) at The Children's Hospital of Philadelphia (CHOP), and the Director of the NF Program at CHOP. His research focusses on identifying new treatments and novel biomarkers (particularly using new imaging modalities) and exploring functional outcomes for children with tumours associated with NF1. Dr Fisher is Chair of the Steering Committee of the Department of Defence NF Clinical Trial Consortium. He is a member of the Steering Committee and former Chair of the Visual Outcomes Committee for REiNS (Response Evaluation in Neurofibromatosis and Schwannomatosis), an international effort to develop standardised outcome measures for clinical trials. In addition, he serves as a co-leader of a Children's Tumor Foundation funded, international, multi-institutional, prospective longitudinal study of patients with newly diagnosed NF1 associated optic pathway glioma, and is co-PI of the NF1 Low Grade Glioma Synodos Project. Dr Fisher also leads the Michael Fisher Research Program, where researchers are using clinical outcomes analysis and molecular profiling to improve existing cancer treatment protocols. They are also evaluating new, targeted cancer therapies that can be used to treat paediatric solid tumours, including plexiform neurofibromas, optic pathway gliomas and medulloblastomas. The long-term goal of the research is to improve risk classification, better define treatment selection and evaluate new, targeted therapies that are more effective and less toxic than current treatment options for these tumours.

PRESENTATION: OPTIC PATHWAY GLIOMAS IN NF1

Optic Pathway Gliomas (OPGs) occur in 15-20% of children with NF1, leading to visual deficits in up to half of these individuals. Determining which tumours can be observed without treatment is as important as determining which require treatment. In most cases, the goal of treatment is to preserve vision, but predicting which tumours will cause vision loss is challenging and chemotherapy continues to be the main treatment option.

The ability to predict impending vision loss could potentially revolutionise care of these patients and improve overall visual outcomes. The management of these tumours including treatment indications, therapeutic approaches, visual assessments, and potential biomarkers of vision will be discussed.

DR MATTHEW C.HOCKING, PHD

Paediatric Psychologist | Division of Oncology | The Children's Hospital of Philadelphia | Assistant Professor of Clinical Psychology | Perelman School of Medicine | University of Pennsylvania

Dr Matthew Hocking, PhD, is a paediatric psychologist in the Center for Childhood Cancer Research and Neurofibromatosis Program at the Children's Hospital of Philadelphia. Dr Hocking provides consultation and intervention services to youth and families treated in the Cancer Center with a focus on those receiving care within the Neuro-Oncology and Neurofibromatosis Programs. His research program bridges the fields of neuropsychology, developmental, pediatric and family psychology and examines risk and resilience factors associated with the neurodevelopmental outcomes of youth diagnosed with neurofibromatosis and brain tumours.

PRESENTATION: IDENTIFYING TARGETS FOR SOCIAL INTERVENTIONS IN NF1

This presentation will provide a brief overview of the social competence difficulties of youth with NF1 and highlight what is known and not known about factors contributing to these difficulties. Data from ongoing research at the Children's Hospital of Philadelphia that seeks to identify neurobiological and psychological targets for interventions to improve the social competence of youth with NF1 will be reviewed along with potential future directions for clinical research.

DR GEOFFREY MCCOWAGE MB, BS, FRACP

Senior Paediatric Oncologist | Children's Hospital at Westmead

Dr Geoff McCowage is a senior paediatric oncologist and is CEO of Australasian Children's Cancer Trials. He has a particular interest in the use of MEK inhibitor drugs in the treatment of children with neurofibromatosis-related tumours.

PRESENTATION: MEK INHIBITORS IN NF1 RELATED TUMOURS: UPDATE AND FUTURE RESEARCH TRIALS

Patients with NF1 are at risk of developing low grade gliomas and plexiform neurofibromas. The MEK inhibitor drugs, trametinib and selumetinib, have been evaluated as treatments of these tumours. Results are very encouraging, and the drugs are being used increasingly often. Dr McCowage will present data on their use and describe some ongoing and future research studies.

SPEAKERS

SPEAKERS

DR REBECCA SAUNDERSON BMEDSCI (HONI) MBBS (HON) MPHIL (CANTAB) FACD

Dermatologist | NSW Faculty Honorary Secretary ACD

Dr Rebecca Saunderson is a dermatologist with a special interest in the treatment of NF. Dr Saunderson is a consultant dermatologist at Royal North Shore Hospital and has a special interest in the treatment of the cutaneous manifestations of Neurofibromatosis. Together with Dr Mimi Berman, Dr Saunderson is pioneering treatment of NF cutaneous neurofibromas in Australia.

PRESENTATION: TREATMENT OF DISFIGUREMENT IN NF

Her talk will address current evidence and therapies for the treatment of the cutaneous manifestations of NF.

ASSOCIATE PROFESSOR NIGEL BIGGS MBBS (HONS) FRACS

Chairman, Department of Otolaryngology, Head and Neck and Skull Base Surgery, St Vincent's Hospital, Sydney

Associate Professor Nigel Biggs is Chairman of the Department of Otolaryngology, Head and Neck and Skull Base Surgery at St Vincent's Public and Private Hospitals, Sydney. His career has focussed on otology, neuro-otology and skull base surgery. He has obtained extensive experience in the management of vestibular schwannoma and NF2. He is an Associate Professor at the University of Notre Dame and Senior Lecturer at the University of New South Wales and has recently been appointed deputy senior examiner in Otolaryngology, Head & Neck Surgery with the College of Surgeons.

PRESENTATION: NF2 TREATMENT OPTIONS

Advances in surgery, radiotherapy and imaging combined with a better understanding of the natural history of NF2 has improved the decision making of the medical teams and overall quality of life of patients. Conservative management and interventions with rationale and outcomes will be presented in detail along with discussion of new concepts and paradigms in treatment.

CLINICAL ASSOCIATE PROFESSOR MIMI BERMAN BMBS FRACP BSC HONS PHD

Clinical Geneticist (HGSA) | Head, Department of Clinical Genetics | Royal North Shore Hospital | President Australasian Association of Clinical Geneticists | MD Research Coordinator, University of Sydney Northern Clinical School

A/Prof Mimi Berman is the Head of the Genetics Department at Royal North Shore Hospital. Her special interest is in the care of adults and children with NF1, NF2, and Schwannomatosis. She has a PhD in Neurogenetics in the area of muscle performance and metabolism. A/Prof Berman is the Co-Chair of the ACI Clinical Genetics network, the current president of the Australian Association of Clinical Geneticists (AACG) and is the Medical Advisor to the Children's Tumour Foundation of Australia.

PRESENTATION: TREATMENT OF DISFIGUREMENT IN NF

The disfiguring effects of cutaneous neurofibromas are the most concerning complication of NF1 among young adults with NF1. Dr Berman and Dr Saunderson present on the treatment of neurofibromas and the impact of the burden of skin disease on people with NF1.

DR GAUTAM WALI, MSC, PHD STEMCELL AND NEUROSCIENCE

Department of Neurogenetics | Kolling Institute | Royal North Shore Hospital | Northern Clinical School, USYD

Dr Gautam Wali is a neuroscience stemcell scientist at the University of Sydney. His research is focussed on using patient-derived stemcell models: adult stem cells and induced pluripotent stem cells for disease modelling and drug screening for neurodegenerative diseases. Dr Wali and colleagues have identified a potential drug treatment candidate for Hereditary Spastic Paraplegia, a neurological disorder and they are now headed towards a clinical trial. Dr Wali specialises in using patient-derived stem cell models to understand disease mechanism and develop drug treatments for neurological disorders.

PRESENTATION: STEMCELL RESEARCH APPLICATIONS TOWARDS NF

His talk will address the possible application of stemcell technology to NF research.

SPEAKERS

ASSOCIATE PROFESSOR AARON SCHINDELER BSC (HONS), PHD

Bioengineering & Molecular Medicine (BAMM) Laboratory | Orthopaedic Research & Biotechnology Unit | The Children's Hospital at Westmead | A/Prof, School of Chemical and Biomolecular Engineering, Faculty of Engineering & IT (USYD) | Conjoint A/Professor, Sydney Medical School (USYD)

A/Prof Schindeler heads the Bioengineering & Molecular Medicine laboratory at The Children's Hospital at Westmead and holds academic positions in the faculties of Medicine & Health and Engineering & Computer Science at the University of Sydney. He leads a multidisciplinary team of scientists, engineers, and medical and allied health professionals that tackle major clinical issues affecting children's bone health.

PRESENTATION: UPDATES ON L-CARNITINE THERAPY FOR NF1 AND THE DEVELOPMENT OF GENE THERAPY TOOLS FOR NF TREATMENT

A/Prof Schindeler's preclinical research indicates that NF1 patients experiencing muscle myopathy may be treatable by nutritional supplementation. Results from a 2019 trial using L-carnitine supplementation in six children with NF1 aged 8-12 years will be discussed along with the updates on the appearance of other CRISPR gene therapy trials, including the emergent funding opportunities for NF gene therapy research and timelines for implementing gene therapy treatments for NF.

DR MATHILDA WILDING MB BS

Cancer Geneticist, Familial Cancer Service, RNSH

Dr Mathilda Wilding is trained as a Clinical Geneticist and Medical Oncologist and works as a Cancer Geneticist at the Familial Cancer Service at the Royal North Shore Hospital. Mathilda was involved in the development and co-ordination of the NF1 breast screening study currently being conducted at Royal North Shore Hospital.

PRESENTATION: INSIGHTS INTO THE IMPLEMENTATION OF A BREAST SCREENING PROGRAM FOR YOUNG WOMEN WITH NF1

SPEAKERS

DR BELINDA BARTON PHD, BA (HONS.PSYCH)

Department Head & Psychologist | Children's Hospital Education Research Institute (CHERI) | NF1 Neurocognitive Research Team Leader | Kids Neuroscience Centre | Conjoint Senior Lecturer | Sydney Medical School | USYD

Dr Barton is a paediatric psychologist whose major research interest is in understanding the cognitive and psychosocial aspects of genetic and neurodevelopmental disorders, as well as interventions that aim to improve the quality of life of children and their families. She leads the NF1 cognitive research program along with the NF1 Learning Disorders Clinic at the SCHW. She's been involved in a number of NF1 studies including a longitudinal study examining the cognitive development of young children, as well as treatments for reading and cognitive difficulties in children with NF1.

PRESENTATION: THE COGNITIVE AND BEHAVIOURAL DEVELOPMENT OF CHILDREN WITH NF1 FROM TODDLERHOOD TO SCHOOL AGE

Cognitive and behavioural impairments are common complications of NF1 in school-aged children. Understanding the developmental profile of children with NF1 allows for the earlier identification of cognitive problems and intervention. This presentation will provide results from a study that followed the cognitive and behavioural development of children with NF1 and healthy children from 30 months of age to 7 years.

DR KATRINA MORRIS BMEDSCI MBBS (HONS) PHD FRACP

Neurologist, RNSH, Sydney Neurology

Dr Morris is a neurologist in Sydney with a particular interest in NF2. She completed her neurology training in Sydney before taking up the ANZAN fellowship at the John Radcliffe Hospital in Oxford, UK. She then undertook a clinical research fellowship in Neuro-Oncology in Oxford. Her research on the UK NF2 population treated with bevacizumab led to a PhD awarded in 2017. Dr Morris sees patients in the multidisciplinary NF clinic at Royal North Shore Hospital and at Sydney Neurology.

PRESENTATION: NON SURGICAL TREATMENT OF NF2 TUMOURS

SPEAKERS

DR SAMANTHA GINN BSC HONS PHD

Senior Research Officer in the Gene Therapy Research Unit of the Murdoch Children's Research Institute (MCRI)

Dr Ginn is a Senior Research Officer in the Gene Therapy Research Unit of the MCRI and a Senior Lecturer at the University of Sydney. Her current research uses cutting-edge technology to develop treatments for genetic metabolic liver disease and NF1. Dr Ginn was a member of the team involved in treating the first infant in Australia with gene therapy and has proven expertise in vector-mediated gene delivery in both primary human cells and small animal models.

PRESENTATION: TOWARDS A VECTOR-BASED UNIVERSAL GENOME EDITING APPROACH FOR THE TREATMENT OF NF1

Genome editing strategies designed to repair the pathogenic defect directly at the mutant locus will not only recover physiological levels of gene expression, but also provide durable therapeutic effects with an increased safety profile compared to contemporary gene addition strategies. The work presented here will describe the development of novel, safe and efficient therapeutic options for the treatment of NF1.

ALICE MAIER BA(HONS), MPSYCH (CLINICAL NEUROPSYCHOLOGY)

Senior Research Officer in the Gene Therapy Research Unit of the Murdoch Children's Research Institute (MCRI)

Alice Maier is a clinical neuropsychology registrar who trained at the University of Melbourne. She is an early career clinical researcher in the Genetics and Neurodevelopmental Disorders team at the MCRI and works clinically in the Children's Cancer Centre at the RCH. In 2018, Alice was awarded the Barney Fellowship by the CTF to investigate auditory processing deficits in NF1.

PRESENTATION : AUDITORY PROCESSING DEFICITS IN CHILDREN WITH NF1

Study into auditory processing deficits in children with NF1 has provided the groundwork for a larger, fully-powered study investigating the efficacy of remote listening devices in treating audiological deficits in NF1.

NOTES

NOTES

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*WHO guidelines on hand hygiene in healthcare, World Health Organisation, 2009
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