Research Report 2007

‘turning today’s research into tomorrow’s medicine’
CONTRIBUTIONS
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Neonatology and the Grace Centre for Newborn Care Research Unit
Department of Nuclear Medicine
Nursing Research and Practice Development Unit
Children’s Hospital at Westmead Clinical School
Snapshot: Treating Cystic Fibrosis

Our committees
Research committee
Grants
Publications
Staff and students
Our supporters
How you can help
Research is one of the key mechanisms by which The Children’s Hospital at Westmead provides the highest possible standards in care and treatment to our patients and their families.

Our research covers basic, clinical and population health and is driven by the needs of sick children who come to the Hospital every day. The ultimate measure of the success of our research is improved health and well-being for children and families.

Unlike laboratory–based research undertaken at most other institutions, the majority of research done at The Children’s Hospital at Westmead involves clinical researchers interacting one–on–one with children. As a direct result of our research, significant advances in the development of treatments, cures and prevention of diseases have benefited many children with conditions such as cancer, obesity, kidney, heart and respiratory problems.

The Australian Paediatric Clinical Trials Centre (APCTC) at The Children’s Hospital at Westmead is the leading paediatric research centre in New South Wales (NSW), with the vision of developing a world–class translation clinical research facility and associated programs. We have worked hard to build our clinical trial capacity since moving into The Kerry Packer Institute for Child Health Research building at the end of 2005.

Our purpose is to improve the health of children by directly linking what is discovered in the laboratory to clinical trials in the hospital setting. This will give us evidence we can use for new and improved treatment and therapies in our Hospital, which will eventually be used around the world.

The scope of the APCTC will cover all interventional research for children, including pharmaceutical and non–pharmaceutical studies, laboratory and clinical research and newly emerging technologies, such as gene and stem cell transfer. It will lead to significant health gains, not only for children in NSW but across Australia and around the world.

Our Paediatric Ethics Committee has reviewed and approved more than 100 paediatric trials over the last five years.

The Children’s Hospital at Westmead is committed to the development of a vigorous research culture, where scientific inquiry is intrinsic to the way scientists and clinicians approach their roles.

The imperative to conduct clinical trials in children arises from major advances in basic biomedical sciences, which require us to match a commitment to translational research if we are to benefit from this new knowledge.

This puts our researchers in a unique position, allowing us to take the best science out of the lab and share it with children in a powerful, personal and practical combination that effectively turns research into medicine.

Our research staff continue to be honoured and recognised for their work in paediatric research. On behalf of the Hospital I thank all involved in research for the contribution you make to better therapies and treatment for children at The Children’s Hospital at Westmead and within the wider international community.

We are well on our way to becoming a world leader in child health research. With hard work, determination and much–needed ongoing support from the community and major supporters, we will achieve our goals.

Healthy children mean a healthy future, and helping create that future is a goal we are committed to and are passionate about.
Research at The Children's Hospital at Westmead has undergone a number of significant developments since moving into its new building – the Kerry Packer Institute for Child Health Research – late in 2005.

Our growth and development since this time has in large part been due to the wonderful new facilities provided by the building, including the transgenic small animal facility commissioned in 2006–2007 and Australia's first Human Applications Laboratory (HAL), used to modify human cells for the purpose of gene therapy. The first use of this facility will be during a clinical trial of gene therapy for the bone marrow of children receiving chemotherapy for brain tumours, planned for late 2008. Also opened during this period was the Human Movement Laboratory, which facilitates research for children with ambulatory disorders, including those with neuromuscular disorders, those with fragile bones, children rehabilitating from injury and those with cerebral palsy.

Our imaging facilities have expanded and are proving to be an invaluable resource both for the Hospital and for the Westmead Hub (the Western Sydney research precinct for the University of Sydney, NSW Health and the University of Western Sydney, and encompassing the Children's Medical Research Institute, Millennium Research Institute and research at The Children's Hospital at Westmead). The development and commissioning of these new facilities positions us at the very forefront of organisations carrying out translational research.

Another arena in which there has been much activity is that of population health research and policy. We now have three research groups who have secured major national funding for new activities, including the expansion of surveillance of vaccine preventable disorders and of rare childhood diseases and longitudinal studies of kidney disease in indigenous children. In addition, our research in the area of adolescent medicine has received National Health and Medical Research Council (NHMRC) funding to commence studies of best practice in the management of children and adolescents with anorexia nervosa, those with attention deficit hyperactive disorder and a community-based weight program looking at the management of obesity.

Research highlights during the past two years include Prof Kathryn North’s findings relating to muscle function in elite athletes and the identification of a new form of muscle disease through genetic mutation analysis and A/Prof David Little’s unique observations of bone repair, which are now being applied to improve the healing of bones in children. Clinical trials, always a key component of our work, were carried out on children with neuromuscular disorders, diabetes, bone disease, cystic fibrosis and cancer.

May 2007 saw a ‘changing of the guard’ in Research, with Prof Peter Gunning leaving the post of Director. Prof Gunning was our first Director, appointed in 1997, and has made an enormous contribution with his leadership, enthusiasm and scientific expertise. During his time at the helm, there was a four-fold growth in our research community and more than doubling of our laboratory and office space. He developed strong relationships with our Westmead Hub partners, promoted the commercialisation of our biomedical research and successfully lobbied government to establish a technology transfer office (Bio–Link) in NSW. We wish Prof Gunning well in leading his research program at the University of New South Wales.

The ultimate measure of the success of our research activity is improved health for children and we are proud of the significant advances made by our staff for the cures and prevention of diseases that have benefited so many children. Over the coming months and years, we will continue adding to these achievements as we strive to achieve our vision: to be a leading global centre for clinical translational research in children.
Our organisation

Our Vision
To be a leading global centre for clinical translational research in children

Our Mission
To improve outcomes and drive excellence in health care by
- enhancing our understanding of the basis of childhood disease
- developing cures for and preventions of childhood disease
- building clinical trials capacity
- incorporating evidence-based health care into clinical practice and health outcomes for children.

Who we are and what we do
Located near Parramatta, about 30km from the Sydney central business district, The Children’s Hospital at Westmead is right in the centre of the greatest concentration of children found anywhere in Australia.

Research has been going on at the Hospital since the 1950s when Australia’s first-ever televised public appeal raised enough money to set up a laboratory in a cottage on the Hospital grounds at Camperdown.

Our Research Division has grown to include more than 250 staff working across 31 research groups and is expected to grow by another 100 staff over the next five years.

While some research institutions have a single focus, like cancer or heart disease, our research is broad-ranging and we look at the impact of illness on the whole child. Focusing on the links between illness, symptoms, treatments, side effects and individual experiences, our researchers share their knowledge in a multidisciplinary approach to improve child health.

A critical part of our work is clinical trials, allowing us to take new research findings and test them directly in the clinic. Our ‘bench to bedside’ strategy gives children access to new methods and treatments as quickly as possible and allows new discoveries to be translated into advanced clinical practices.

The quality of our research and the number and mix of patients we treat makes us a collaborator of choice for many prestigious medical and educational organisations. Our partners include the University of Sydney, Children’s Medical Research Institute, Millennium Institute, Garvan Institute and many other quality national and international institutions. These collaborations make our research stronger, because by working as a team we are able to achieve more.

We have made many significant discoveries, a number of these world-firsts, and will continue to strive to achieve outcomes that directly translate to better health in children.

‘Your child: the centre of your universe, the centre of our research.’
Our research

Research at The Children’s Hospital at Westmead is undertaken by 31 research groups comprising more than 250 staff and students. Our research groups are listed below in alphabetical order.

Academic Surgery
Anaesthetic Research
Australian Paediatric Surveillance Unit
Centre for Kidney Research
Centre for Perinatal Infection Research
Centre for Trauma Care, Prevention, Education and Research
Children’s Chest Research Centre / Respiratory Medicine
Children’s Hospital Burns Research Institute
Children’s Hospital Education Research Institute
Children’s Hospital Institute of Sports Medicine
Children’s Hospital at Westmead Clinical School
Department of Adolescent Medicine and Eating Disorders
Department of Allergy and Immunology
Department of Infectious Diseases and Microbiology
Department of Nuclear Medicine
Developmental Cognitive Neuropsychology Research Unit
Division of Allied Health
Gene Therapy Research Unit
Institute of Endocrinology and Diabetes
Institute for Neuromuscular Research
James Fairfax Institute of Paediatric Nutrition
Kids Heart Research
National Centre for Immunisation Research and Surveillance
Neonatology and the Grace Centre for Newborn Care Research Unit
Nursing Research and Practice Development Unit
Obesity Research Group
Oncology Research Unit
Orthopaedic Research and Biotechnology Unit
Pathology
Sudden Infant Death Syndrome
Western Sydney Genetic Research Program
Overview of research
The Institute for Neuromuscular Research (INMR) has a major focus on the diagnosis and treatment of children with muscle diseases, such as muscular dystrophy. The institute has strong collaborative links with the Children’s Medical Research Institute, as well as with other national and international research groups.

In addition to conducting a broad range of laboratory studies and clinical trials, the INMR provides a diagnostic service for inherited myopathies and muscular dystrophies to clinicians throughout Australia and the Asia Pacific region.

Laboratory research
Over the past few years, one of the major areas of research undertaken in our laboratory has been the study of muscle performance in elite athletes. If we are able to understand the factors that result in exceptional muscle performance in athletes, this is likely to reveal targets for therapy to improve muscle function in children with muscle disease.

We have so far been able to identify an important gene polymorphism (ACTN3) associated with skeletal muscle performance and are now using a mouse model to investigate the effect of this genotype on response to exercise and ageing and the onset and severity of muscle disease phenotype. Our recent findings that loss of function of the ACTN3 gene alters muscle metabolism were published in Nature Genetics in October 2007.

Another of our research projects relates to congenital myopathies. We have made major contributions to understanding the natural history and predictors of survival in inherited myopathies, and have discovered new genes responsible for several forms of, often lethal, muscle disorders.

Our laboratory also focuses on developing new therapeutic approaches to inherited muscular disorders. In particular, we are studying a form of muscular dystrophy caused by mutations in the gene dysferlin, which has been shown to play a role in repairing muscle membrane damage. We are currently focussing on the pathways involved in repair, with a view to identifying the genes involved and developing new therapeutic approaches.

Our clinical studies
The INMR is involved in several studies, including critical illness polyneuropathy in children, assessment of outcome measures for very young children with neuromuscular disorders and improving respiratory function in children with neuromuscular disorders. We are also studying various aspects of Charcot-Marie-Tooth disease and Duchenne Muscular Dystrophy.

Over the past few years we have established a strong and active clinical trials team and have joined international clinical trials consortia so that the latest advances in therapy become immediately available for our patients. Clinical trials underway include:

• high dose steroids in Duchenne Muscular Dystrophy (DMD) – as part of the Collaborative International Neuromuscular Research Group
• PTC124 in DMD – as part of an international consortium organised through PTC Therapeutics
• deflazacort in DMD, tyrosine in nemaline myopathy and Vitamin C and botulinum toxin in hereditary neuropathies.

We are also involved in preclinical trials of antisense oligonucleotides in DMD.

Major achievements
• Our discovery of the association between the ACTN3 genotype and athletic performance was cited by Discovery Magazine as one of the ‘Top 100 Science Stories of 2003’.
• We conducted the first human clinical trial in nemaline myopathy.
• We identified all four of the known genetic causes of congenital fibre type disproportion (CFTD) and a novel locus on the X chromosome, resulting in genetic diagnosis in 40 per cent of our patient cohort.
• We identified a novel form of inherited myopathy, associated with deficiency of syntrophin and dystrobrevin, and identified a novel gene associated with a lethal congenital myopathy.
• We established a diagnostic service for inherited myopathies and congenital and limb girdle muscular dystrophies.
• We have developed novel techniques for improving diagnostic accuracy and reducing the amount of tissue required to make a diagnosis, some of which have been adopted worldwide.
Overview of research
The Kids Heart Research group is a team of scientists and doctors working together to address clinical problems in the area of cardiac health. Congenital (inherited) heart diseases affect one in every 100 children, with problems ranging from ‘holes in the heart’ to more complex conditions.

Our research efforts are focused on two main areas: improving our understanding of the genetic basis of congenital heart disease and finding ways of making heart surgery safer and more effective.

Genetics and heart disease
In most cases, a diagnosis of a heart condition in a foetus or child comes as very unexpected news for the family. Part of our research is aimed at improving our understanding of the genetic basis of congenital heart disease, so we are better able to predict who will be affected and determine the best timing for interventions to address the problem.

We know that most heart conditions in children are caused by variations in multiple genes, as well as changes in the foetal environment. To further investigate the role of genes, we are using new techniques to evaluate the role of seemingly minor genetic abnormalities that may combine to affect the normal development of the heart, leading to heart disease. Key to this work is the Kids Heart Research DNA Bank at The Children’s Hospital at Westmead. Using this resource, we can match genetic information from a child with detailed clinical information about his/her heart, allowing us to study the association between the two.

Improving recovery after surgery
In our research, we are always striving to improve therapies and treatments for heart disease in children. In particular, we are working on improving heart function after surgery. An important factor that can slow recovery after surgery is water accumulation in the body’s tissues, including the heart. To investigate how fluid retention can be reduced, we are studying the role of specific proteins involved in water transport, the aquaporins. Using a range of experimental models, we are manipulating aquaporin function to try to prevent severe water accumulation, speeding recovery and ultimately making surgery safer.

Optimising health outcomes
There is a need to know more about how having a heart problem can affect children in the long term. For example, while the majority of children with heart disease do not experience any problems with the brain, in some cases complications in brain development can occur. We are therefore working closely with our colleagues in neonatology to study the development of children who underwent heart surgery in the first 90 days of life. This study involves detailed follow-up of children to preschool age, including intricate brain imaging.

Major achievements
• Our research findings on water retention after heart surgery were presented at the 2007 meeting of the Cardiac Society of Australia and New Zealand.
• On the basis of our work, the traditional understanding of how water accumulation affects heart function has been reinterpreted. It is likely that the conduct of infant cardiac surgery will be altered as a result of these findings, including the application of novel therapeutic agents.
• Our research and collaborations culminated in a high profile publication in the American Journal of Human Genetics detailing the role of a molecular intermediary involved in the development of congenital heart disease. Together with our national and international collaborators, we established the role of the transcription factor TBX20 in human disease.
Snapshot: Treating muscle disease

Prof Kathryn North
Neurogenetics Research Unit (NRU) and the Institute for Neuromuscular Research (INMR)

“What we learn by studying elite athletes may help us come up with therapies for those at the other end of the muscle performance spectrum, children with inherited muscle disorders.”

Prof Kathryn North heads a research team at The Children’s Hospital at Westmead that studies debilitating inherited neuromuscular disorders in children, such as muscular dystrophy.

“Children with these disorders often have progressive, degenerative muscle weakness. This not only means they may never walk, but may die young because of the inability of muscles in vital organs like the heart and lungs to work properly,” she explains.

As part of her research, aimed at finding new treatments for muscle diseases, Prof North has been investigating muscle function in elite athletes. “If we can learn what makes muscles perform very well, this may be applied to children with muscle diseases and guide us towards potential new therapies.”

Prof North’s team gained international attention when they made a major discovery about one of the genes known to influence muscle function, ACTN3, responsible for making a protein (alpha-actinin-3) found in ‘fast-twitch’ skeletal muscle fibres – those required for rapid, forceful movement, such as sprinting.

“What we found was that there’s a common version of this gene that doesn’t actually make any protein, and people who inherit two copies of this gene – which turns out to be almost 20 per cent of the general population – are completely deficient in the protein,” says Prof North. Studies also show that this deficiency is extremely rare in sprint athletes, suggesting that the protein plays a crucial role in the function of fast-twitch muscle fibres.

Over the past few years, the team has focused on how the loss of alpha-actinin-3 influences the function of muscle. “By studying mice that completely lack the ACTN3 gene, we have found that loss of function of this gene alters muscle metabolism. We are now examining the precise biochemical pathways involved in this process.”

“The study of elite athletes is looking at one end of the spectrum, where skeletal muscle works at its peak,” says Prof North. “Through these studies we are finding out more about the functioning of muscles. Our aim is to apply this knowledge to develop treatments for people at the other end of the spectrum – kids with debilitating muscle disease.”

Case study

Adam was diagnosed with Duchenne muscular dystrophy at age four, after being unable to run and keep up with his playmates and having frequent falls. From the age of five, he was treated with steroids, with the aim of slowing the deterioration of his muscle strength, but treatment had to be stopped due to side effects. At the Hospital, we began treating Adam with the experimental drug Deflazacort, as part of an Australia-first clinical trial. Adam is now almost ten years old, has had no side effects from the Deflazacort and has greatly improved muscle strength. His muscle performance is almost normal, at a time when he might otherwise have needed a wheelchair.

Adam provides a wonderful example of the advances that are being made in the treatment of muscular dystrophy and the importance of clinical trials.
Overview of research
Our Department has a strong and growing research program that informs clinical practice at The Children’s Hospital at Westmead. Our team is multidisciplinary and we collaborate with several other groups, including Hospital departments and universities. Key areas of eating disorder research include family therapy, the impact of eating disorders on patients and their families, physiological measures of health and recovery and neuropsychiatry and neuro-imaging. Our adolescent medicine projects are focused on attention deficit hyperactivity disorder, complex and chronic illness, substance use and weight management.

Eating disorders
We carry out an extensive range of research projects on the causes, diagnosis and management of eating disorders. Much of this work involves collaboration with other research groups, such as the Centre for Research into Adolescent Health. A particularly exciting project underway is a large, National Health and Medical Research Council (NHMRC) funded randomised controlled trial evaluating family therapy in the treatment of anorexia nervosa. This trial is a world-first and is one of only two NHMRC-funded projects on treatment of eating disorders.

Complex and chronic illness
Our team is involved in a world-first trial to find new, cost-effective solutions to the serious eating disorder, anorexia nervosa

We are well known for our work on complex and chronic illness and have conducted several multidisciplinary research projects in chronic illness and transition, particularly cystic fibrosis. We are currently involved in a large-scale project on the treatment of complex medico-psycho-social conditions, including pain disorder and post-viral fatigue. This research, which involves staff working in youth health, is being conducted in collaboration with the Centre for the Advancement of Adolescent Health.

Drug and alcohol use
Common adolescent risk-taking behaviours, such as drug, alcohol and tobacco use is another area of interest to our team. Our focus at present is a study on resilience building in high-risk youths. This work is being undertaken by Teen-Link, an outreach service of our Department, with support from the Centre for Research into Adolescent Health.

Attention deficit hyperactivity disorder (ADHD)
ADHD is a common behavioural disorder in adolescents. Despite its prevalence, there is a lack of understanding of the biological factors related to this condition and diagnosis and treatment continue to be based on the subjective rating of behaviour.

We are involved in a series of projects aimed at improving our understanding of neuropsychological and neurobiological aspects of ADHD, ascertaining objectively measured markers for this disorder and assessing the impact of stimulant and other medications. This work is being done in close collaboration with the Centre for Research into Adolescent Health and the Brain Dynamic Centre. We have also had success in obtaining major funding through NHMRC and ARC grants to investigate biological markers for this condition.

Weight management
Adolescent obesity is a serious epidemic in Australia, with severe consequences for the individual and high costs for society as a whole. Our team is undertaking several multidisciplinary projects that seek to find new, cost-effective solutions to this problem. We are evaluating a number of different treatment approaches and have recently completed a randomised control trial examining a martial arts intervention for obesity, in collaboration with the University of Sydney. A further randomised control trial in dietary management has been recently funded.

Major achievements
• Through our clinical research program, we have become recognised as the leading Australian institution for the treatment of child and adolescent eating disorders.
• We were the first in the world to describe reversibility of structural brain changes associated with anorexia nervosa.
• Our research into transition to adult care of adolescents with cystic fibrosis has played a major role in informing the chronic illness transition program at The Children’s Hospital at Westmead and beyond.
• Our four-year study of adolescents with somatoform disorders and chronic fatigue syndrome is the largest ever follow-up study of this population and is providing valuable insights into the mechanisms underlying successful treatment for these disorders.
Overview of research
Our research group incorporates both the Children’s Hospital Education Research Institute (CHERI) and the Developmental Cognitive Neuropsychology Research Unit (DECOG).

CHERI is a highly regarded and nationally recognised institute that conducts research into the educational and psychosocial aspects of children with learning problems. Current research particularly focuses on children with genetic disorders who also have cognitive impairments and/or learning problems.

DECOG is an internationally recognised neuropsychology research facility, established in 2000 as a joint initiative of The Children’s Hospital at Westmead and the Macquarie Centre for Cognitive Science. Current research in the Unit includes cognitive neuropsychological and neuropsychological outcomes studies.

The Developmental Cognitive Neuropsychology Research Unit (DECOG)
DECOG is the only developmental cognitive neuropsychological research unit in Australia and offers high calibre research into cognitive disorders with direct clinical application. A large part of DECOG’s current research program involves group studies investigating neuropsychological outcomes in children with developmental or acquired disorders. The knowledge we gain from these studies is likely to have a significant impact on outcomes in children with quite varied conditions, such as acquired cerebellar disease, brain tumours, diabetes and sleep disorders.

DECOG is the only unit of its kind in Australia, conducting clinically-useful research into cognitive disorders

We also carry out cognitive neuropsychological studies. These typically include case studies of children with disorders such as, dyslexia or face processing deficits and involve the use of theoretical models to explain cognitive processes and aid our understanding. In particular, we are focused on developing treatment and intervention programs, aimed at improving the outcome for children with these disorders.

Major achievements

- CHERI’s study on educational services for students with chronic illness has led to a NSW Department of Education training module for teacher aides.
- In conjunction with the Neurogenetics Research Unit, CHERI has made a number of novel and clinically relevant observations regarding the cognitive functioning of school aged children with NF1.
- DECOG’s studies have made a significant contribution to our understanding of the impact of childhood diseases (such as brain tumours) on cognitive development and have resulted in new methods for assessing and treating cognitive disorders.
- DECOG has an outstanding track record in cognitive neuropsychological case studies of reading, spelling and visual processing disorders and has developed a range of treatment programs for children with these disorders.
As well as working as a cardiac surgeon at the Hospital, A/Prof David Winlaw heads a research team that carries out studies aimed at solving some of the clinical problems seen in children with heart disease. “One of the major problems we come across in children who have had surgery for heart problems is water retention,” explains A/Prof Winlaw. “Water tends to accumulate in the body’s tissues after heart surgery. This prevents the normal functioning of the heart and significantly slows the recovery of these children.”

“We were looking at problems associated with the heart being stopped during surgery, versus keeping it beating using a heart-lung machine. We found that it was actually the length of time the heart has been stopped for that determined how well it functioned after surgery, and this is largely because of damage sustained to the heart’s membranes. We thought that if it was possible to somehow repair the membranes, the heart should work better after surgery.”

Using models in the laboratory, ranging from individual heart cells to experiments that mimic the conditions of heart surgery, A/Prof Winlaw and his team were able to show that a pharmaceutical agent (which had not previously been used for this application) could be used to prevent damage to the heart’s membranes and potentially facilitate membrane repair. In turn, this was associated with better heart function and a reduction in the amount of swelling associated with the operation.

Following further studies in experimental models over the next few years, the heart research team hope to reach a stage when this treatment can be tested in a clinical trial of children undergoing heart surgery. “My hope is that, through the use of this drug, children will be able to bounce back better from heart surgery and that their heart function will be much improved.”

Case study
Benjamin’s journey highlights the plight of many children and families affected by congenital heart disease. Diagnosed while still in his mother’s womb, he was born with a set of problems affecting his heart and lungs. Without medical treatment and surgery in the first few days of life, Benjamin’s condition – called aortic atresia and ventricular septal defect – would certainly have been fatal. After birth, Benjamin underwent a seven-hour operation and spent six weeks in the Hospital. He has been in and out of hospital over the last few months and will need another operation before starting school. He is now gaining strength and his family looks forward to the future.

*Through our research, we seek to understand the causes of congenital heart disease and improve our treatments for children who are affected.*
Overview of research

The mission of our Unit is to advance orthopaedic care through improved understanding of bone diseases, bone healing and pharmaceutical therapies.

An emerging theme of our research is that, in bone healing, it is the levels of anabolism (bone formation) and catabolism (bone resorption) that determine the outcome. Much of our work is therefore centred on finding effective means of treating catabolic and anabolic deficiencies.

Treating bone disease

In many clinical situations, our research has demonstrated that the underlying defect causing the bone pathology is excessive catabolism. We have found that this can be effectively treated using bisphosphonates, a class of anti-resorptive drugs originally developed for the treatment of osteoporosis. Bisphosphonates are now routinely used at the Hospital to treat Perthes disease of the hip (osteonecrosis), as well as complications that can occur during distraction osteogenesis.

In other research, we have been investigating the role of bone-resorbing cells, known as osteoclasts, in fracture repair. The majority of fractures heal via a process known as endochondral ossification, where a soft cartilage callus is progressively removed and replaced by a bony hard callus. The conventional dogma holds that osteoclasts are the cells responsible for this removal of cartilage. However, we have demonstrated that when osteoclasts are specifically inhibited using bisphosphonates, removal of the soft callus can occur normally. This research has direct relevance to the clinical use of anti-resorptive drugs in fracture management.

Bone defects in Neurofibromatosis type 1

Neurofibromatosis type 1 (NF1) is a genetic disease affecting one in every 3000 children. These children can develop severe orthopaedic problems, including scoliosis (curvature of the spine) and a tibial non-union, known as congenital pseudarthrosis of the tibia. Orthopaedic surgery on children with these conditions has a poor prognosis.

In our research, we are using genetic mouse models of NF1 deficiency as well as patients with NF1. These studies suggest that problems in both bone anabolism and catabolism resulting from multiple cellular defects may contribute to the NF1 bone phenotype.

Emerging research directions

It is not uncommon for bone to form in abnormal locations and muscle is the most common site for this to occur. This has led us to speculate that muscle stem cells have a strong innate capacity to form bone. We are therefore investigating the potential contribution of muscle cells to bone formation and fracture repair. We are hopeful that this project may result in new cell-based therapies for bone repair.

We are also looking to utilise muscle stem cells together with pro-anabolic and anti-catabolic therapies for bone tissue engineering and have recently elucidated a new class of drugs that may be helpful for augmenting orthopaedic treatments.

Finally, in collaboration with a number of other groups at The Children’s Hospital at Westmead, we are working towards adapting a selective bone marrow transplant therapy to treat genetic bone diseases, such as osteogenesis imperfecta (brittle bone disease).

Our mission is to advance orthopaedic care through improved understanding of bone diseases, bone healing and pharmaceutical therapies.

In some clinical situations, anabolic deficiencies can be overcome using the bone-forming agents, bone morphogenetic proteins (BMPs). However, we are increasingly finding that the effect of these anabolic drugs can be optimised with co-treatment with anti-catabolic drugs, such as bisphosphonates. We are continuing to explore drug combination therapy in laboratory models, as well as applying it to the clinical situation.

Major achievements

- Our team developed the first models of bisphosphonate treatment of osteonecrosis.
- We were the first research group to form the concept of manipulation of anabolism and catabolism in bone repair.
- We have successfully developed the first models of fracture repair in Neurofibromatosis type 1.
Overview of research
At The Children's Hospital at Westmead, 1000 children with burns are treated each year. The Children's Hospital Burns Research Institute (CHBRI) was established in 2005 to conduct laboratory and clinical research, aimed at improving the treatment of burns and burn wound outcomes in children.

More than 1000 children with burns are treated at The Children's Hospital at Westmead every year.

Laboratory research
Immediate cooling following a burn limits the extent of the burn wound and decreases the need for skin grafting. Using a porcine burn model, we have found that cool running water for an optimum period of 20 minutes consistently improves wound recovery, compared with using wet towels or water spray. We are now comparing the effectiveness of running water with that of Burnaid.

We are also using our in vivo burn wound model to determine the rate of hypertrophic scarring, resulting from the application of skin grafts to a deep partial thickness burn wound at different time intervals following a contact burn.

In collaboration with the Vascular Biology Research Centre at Westmead Hospital, we have been investigating the role of the fibrocyte, a novel leucocyte, in burn wound healing. We have confirmed the presence of this cell in paediatric burn wounds and have identified an association with hypertrophic scarring. Further work may allow us to manipulate the fibrocyte to alleviate scarring in some patients.

Wound Healing Laboratory
The healing of skin is a complex process and is usually accompanied by a robust inflammatory response. Excess and ongoing inflammation tends to lengthen healing time, resulting in increased scarring and contracture. The extent of inflammation is largely determined by the influx into the wound of macrophages (cells that form part of the immune system).

Research has shown that mice that lack macrophages heal faster, with reduced scarring. Using debrided skin samples from patients in the Burns Unit, we are purifying primary macrophages and using cell lines to investigate how and when the number of macrophages in a wound might be reduced, to limit scarring without affecting the over healing process.

Clinical research
We are conducting a prospective study to determine the relationship of time of healing to the development of hypertrophic scars and the effect of early skin grafting in children with scald burns. We are also researching the relationship of positive burn wound culture to graft failure.

In conjunction with the Burns Unit, we have been instrumental in setting up and running a Laser Doppler Imaging Service that allows us to predict whether a burn wound will heal with dressings or require skin grafting. This allows clinicians to plan optimal care for the wound, as well as to prepare the child and family for any surgery required. Further research will investigate the ability of this imaging to predict hypertrophic scarring.

Major achievements
• We were the first to prove the effectiveness of Laser Doppler Imaging prediction of burn wound outcome in children.
• We were the first to identify fibrocytes in burn wounds in children and to have correlated their presence with the development of hypertrophic scarring.
• We have proven the effectiveness of 20 minutes of cold running water as optimal first aid for scald burns, even when first aid is delayed for up to an hour.
• We have established a method of purifying macrophages from debrided skin samples and have identified the specific integrins upregulated in the macrophages.
Our group has been one of the first to study this problem in Australia.

Research into a debilitating genetic disease called Neurofibromatosis type 1 (NF1) is leading to new approaches to aid bone healing in children. "Children with NF1 can develop many medical problems, and one of these is that their bones tend not to heal when they break," says A/Prof Little. "In Australia, our group has been one of the first to study this phenomenon and try to work out why it happens and what can be done about it."

"Research by our group, particularly postdoctoral scientist Aaron Schindeler, as well as research teams elsewhere, has confirmed that defects in the NF1 gene lead to problems both with bone formation and bone healing. As far as bone healing goes, it's likely that there are several issues at play. The bone-forming cells don't make enough new bone and, instead, the injured site is invaded by fibrous tissue that prevents bone repair. We also see that any bone that is made can be prematurely removed by bone-resorbing cells."

A/Prof Little's team has been working on recreating this type of non-healing bone in a mouse model of NF1 and, using this genetically modified mouse, they have been able to learn much about the problem. "We now have a good understanding of what is going wrong in the bone healing process in mice and are looking to translate these findings to children with NF1."

"We have found we can obtain a good effect in bone repair when we use agents that increase bone formation, such as bone morphogenetic proteins or BMPs, together with other drugs that decrease bone resorption, such as bisphosphonates," explains A/Prof Little. "Although this works quite well, we are always looking for an improved result and what we now want to do is model this condition more accurately and test other drugs that may have an even stronger therapeutic effect."

While this research is initially focusing on children with NF1, there are much broader implications for its application. "We are using this condition as an example, but we expect our findings will be applicable to several other conditions," says A/Prof Little.
Overview of research
The Oncology Research Unit was established in mid-1997 with the aim of increasing our understanding of cancer biology and translating this knowledge into clinical practice, leading to the improved diagnosis and treatment of cancer.

Cellular oncology
Our cellular oncology research is aimed at understanding cancer at the cellular level. Specifically, we are investigating the changes in cell structure that accompany cancer, focusing on the role of one of the major building blocks, tropomyosin, in the cancer cell skeleton. We are developing drugs that target tropomyosin and disable the cell skeleton, thus preventing both cancer cell growth and movement. Our first drugs show high efficacy in killing childhood cancer cells derived from neuroblastoma and brain tumours.

Cancer gene therapy project
The Children's Hospital at Westmead's Oncology Department and Gene Therapy Research Unit are working to address the problem of toxic side effects of chemotherapy on the bone marrow of children with brain tumours. Our aim here is to apply a new gene therapy strategy using a DNA repair protein, methyl-guanine-methyl-transferase (MGMT) to provide protection to the bone marrow of these children. A clinical trial of this strategy is planned for the near future.

Molecular oncology
In our investigation into the molecular basis of cancer, we are studying genes that are over-expressed in different cancers, with the aim of determining the functional significance of this. For example, we are investigating how over-expression of key chromosome 8q amplified genes promotes tumourigenesis. We also aim to develop critical genes as diagnostic markers for the early detection of cancer, such as the tumour protein D52 gene at chromosome 8q21.

Focal adhesion biology
These studies are aimed at identifying new targets for cancer treatments. While it is well known that metastasis (the migration of cancer cells from the primary tumour to other sites) is the most common cause of death from cancer, this process is still not well understood. Our goal is to work out how cell migration is regulated, so new therapies may be designed to stop metastasis. Specifically, we are investigating the mechanisms that control cellular adhesion, including the role of the molecule HEF1.

Tumour Bank
The Tumour Bank was established to facilitate childhood cancer research both in The Children's Hospital at Westmead and the broader scientific community in Australia and internationally. The Bank contains over 20,000 specimens from more than 2,000 patients. Currently, cancer diagnosis and treatment decisions are largely based on the pathological examination of tumour tissue and patient assessment. We are using our collection of tumour samples to conduct high throughput genomic and gene expression assays with novel computer analytics, with a view to developing new point-of-care tools that can be applied to cancer biology, diagnosis, prognosis and clinical management.

Our Tumour Bank was the first childhood cancer bank to be established in Australia.

Major achievements
- We identified that the expression of ribosomal protein L38 was able to distinguish childhood leukaemia patients at risk of relapse.
- We determined that vesicle transport is a potential drug resistance mechanism in rhabdomyosarcoma tumours.
- Our team was the first to identify the role of different tropomyosins in normal cell function and how their dysfunction contributes to cancer progression.
- We successfully generated anti-tropomyosin drugs able to cause death in cancer cells, while sparing the heart.
- We generated a producer Master Cell Bank for the upcoming cancer gene therapy clinical trial and this has been tested and qualified for clinical use.
- We identified freezer storage as a technical factor influencing gene expression results obtained from Tumour Bank samples.
“Tumour bank samples are precious, so it’s important that when they’re used for experiments, the best results possible are obtained.”

Although relatively rare, cancer in children can be difficult to treat and, among diseases, it is the biggest cause of death in children. Improving our understanding of childhood cancer is an essential step to further improving cure rates. This is precisely the aim of The Children’s Hospital at Westmead’s Oncology Research Unit.

“We need to study the genes in cancer cells to understand cancer better and develop improved diagnosis and treatment,” says A/Prof Byrne.

“To carry out many of these gene studies, we need samples of tissue or marrow taken from children with cancer.”

Once a patient and/or family have given permission for samples to be used for research, the samples are stored frozen in a facility called a Tumour Bank. The Children’s Hospital at Westmead’s own tumour bank, established in 1997 within the Oncology Research Unit, now contains specimens from more than 2000 patients.

“In 2006, our group published some research that involved extracting a molecule called RNA from bone marrow samples taken from children with leukaemia,” explains A/Prof Byrne. “During the course of this work, we found that certain factors seemed to affect how useful those samples were for this type of molecular analysis. One of these factors was the length of time that the samples had been frozen in the Tumour Bank. We found that we were less likely to successfully detect a particular type of RNA in samples that had been frozen for longer periods of time.”

“Before we found this link between freezer storage time and molecular analysis results, no one had really understood just how important the freezing time for samples was. It turns out that freezer storage time could in fact lead to false results in some cases.”

These findings have implications for a range of research involving the use of frozen samples. “Tumour bank samples are hard to come by and quite precious, so it is very important that when they are used for experiments the best results possible are obtained,” explains A/Prof Byrne. “We hope our findings will help others doing molecular analysis to make the best use of tumour bank resources in the future. We also hope these results will encourage hospitals and other research institutions to collaborate, so rare tissue resources can be shared.”

**Case study**

Richard is a 16 year old boy who came to the Hospital with a tumour, called rhabdomyosarcoma, which had spread to his bones and lungs. In the past, very few patients with this type of diagnosis have been able to be cured. Richard enrolled in a research study that involved a new drug, Irinotican, being added to the other drugs usually used to treat this disease. Following the first course of irinotican, Richard’s scan showed that the tumour had resolved almost completely. He then went on to receive more chemotherapy, then radiation therapy. Richard is now in remission and is soon due to complete treatment.

*Research studies, can be of great benefit to children like Richard, who in the past would have had a very poor outlook.*
Overview of research
The Western Sydney Genetics Program is focused on translating research findings into clinical practice and examining fundamental research questions. The diversity of our research reflects the broad impact that medical genetics has on paediatric practice.

NSW Centre for Rett Syndrome Research
Rett Syndrome (RTT) is a neurodevelopmental disorder leading to severe loss of intellectual and physical abilities. In most cases, the disorder is caused by mutations of a gene called the methyl CpG-binding protein 2 (MECP2) gene. We have carried out extensive genotype-phenotype studies on RTT and were key collaborators in the discovery of a second gene associated with this syndrome, CDKL5. We are working to identify target genes for MeCP2 and are also using proteomics to identify important proteins in RTT.

The diversity of our research reflects the broad impact that medical genetics has on paediatric practice.

Genetic Metabolic Disorders Research Group
Phenylketonuria (PKU) is a rare disorder, caused by an inherited enzyme deficiency. We are investigating the use of co-factor tetrahydrobiopterin (BH4), a medication that increases the activity of the defective enzyme. We have identified that at least a third of our patients have mutations that should respond to BH4 and are now aiming to find the lowest effective dose. We are also exploring the use of genetically modified probiotics as treatment for PKU.

Our group is also researching mitochondrial respiratory chain (RC) disorders in which there is a defect in ATP production. Prenatal testing is available, however in about 50 per cent of cases the underlying molecular defect is not known, thereby preventing diagnosis. We have been exploring new ways in which we can unmask RC defects.

In addition, we have encountered a number of families who have a Mendelian disorder of unknown cause and are using linkage or autozygosity testing to identify the causative gene. Through this work, we recently identified the gene responsible for Arts Syndrome.

Biochemical Genetics and Newborn Screening Research Group
As the first publicly-funded Newborn Screening Program to adopt tandem mass spectrometry technology, our major research focus is evaluation of the clinical and economic outcomes of this new technique. We were the first to clearly demonstrate the reduced morbidity and mortality in medium-chain acyl-CoA dehydrogenase deficiency (MCADD) in screened populations and subsequently have shown improved clinical outcomes for one other disorder.

In other research, we are evaluating screening for cystic fibrosis and fragile X syndrome, and are collaborating with the Gene Therapy Research Unit to investigate the use of gene therapy to treat ornithine transcarbamylase (OTC) deficiency.

Eye and Developmental Genetics Research Group
Our purpose is to gain a fundamental understanding of critical factors involved in development, especially eye development. We are focusing on children with abnormalities from birth, including congenital eye abnormalities such as glaucoma and cataract, to improve our understanding of the underlying genetic causes. We have identified a number of relevant genes and are undertaking in vitro and in vivo studies to understand the functional consequences of abnormality of these genes. Other projects include a detailed investigation of a novel candidate glaucoma gene (Twist2) and a study of WNT signalling in eye development. Outcomes of these studies offer genetic diagnosis and potential novel therapeutic and management approaches.

Marfan Syndrome Research Group
The Marfan Research Group provides a research and diagnostic facility for the genetic screening of patients referred with a possible diagnosis of Marfan Syndrome, or a Marfan-related condition. Marfan Syndrome (MFS) belongs to the most common group of connective tissue disorders, the fibrillinopathies. A second group of more recently described disorders are the TGFß signallopathies. Common to these groups are abnormalities of the blood vessel walls, including the aorta, which can be life-threatening.

Several lines of evidence suggest that abnormalities in the TGFß signalling pathway may represent a final common pathway for the development of MFS and related disorders. A critical first step is the study of aortic dilatation and dissection.
of patients and their families to better understand the effect of specific mutations of genes, including TGFBR1, TGFBR2 and FBN1. It is our hope that these studies may lead to the development of new, highly effective treatment strategies that revolutionise the care of affected patients and make emergency surgery obsolete.

Skeletal and Lysosomal Disorders Research Group

Our research into the genetics and treatment of bone disorders in children began with brittle bone disorders, collectively known as osteogenesis imperfecta. We are now investigating the normal ranges of bone density and skeletal metabolites in children, the natural history of various skeletal disorders and the treatment of these disorders with bisphosphonates.

We are regarded as a world authority in the histopathology and radiological delineation of skeletal birth defects. We have major collaborations underway to investigate the natural history of achondroplasia and to search for molecular genetic defects in Desbuquois and 3M syndromes. We are also working to define the human homology of mutations in the SMDP3 gene, impaired function of which can lead to excessive bone resorption.

In other research, we have investigated the frequency of consanguinity over a two year period and demonstrated in nearly 60 per cent of our Middle Eastern and central Asian clientele that the parents were consanguineous. Autosomal recessive disorders were 1.7 times more frequent in the offspring of consanguineous couples. We are working towards developing gene chip technologies to offer population-specific testing in the future.

Clinical Genetics Department

Clinicians from our group head formal research groups within the Western Sydney Genetics Program. Medical and counselling staff collaborate in additional clinical research related to other areas of interest. Current research is focused on delineation of the features or underlying causes of known or new syndromes or genetic disorders and psychosocial issues in predictive testing for Huntington Disease.

Major achievements

• Our team was among the first to undertake large scale phenotype-genotype correlations in RTT patients, providing clinicians with firmer data for disease prognostication. We were also the first to report mutations in CDKL5 in RTT patients.
• We identified the gene responsible for Arts Syndrome (PRPS1) and found S-adenosylmethionine to be of therapeutic benefit, leading to significant improvements in patients and a decrease in hospitalisation.
• We were the first publicly-funded Newborn Screening Program in the world to adopt tandem mass spectrometry technology. Our early adoption of this technology has placed us at the forefront in recognising different mutations in cases of MCAD deficiency and in establishing new methods for analysis of methylmalonate and total homocysteine.
• Our eye research led to the discovery of MAF as a novel disease gene in cataract, microcornea and eye development, discovery of TMEM114 as a novel candidate gene in cataract formation and discovery of a novel SOX2 mutation in a family with marked ocular phenotypic variability.
• We were the first to describe and delineate the clinical features of Mowat-Wilson Syndrome, which led to the diagnosis of many children with this condition and were one of two groups to identify the gene involved (ZFHX1B).
• We have conducted a detailed investigation into predictive testing for Huntington Disease, an untreatable adult onset genetic disorder, and have made a strong case against predictive testing in minors.

22 Stream 4 – Genetics, gene therapy and genomics | the children’s hospital at Westmead
Overview of research

The Gene Therapy Research Unit, a joint initiative between The Children’s Hospital at Westmead and the Children’s Medical Research Institute, aims to develop gene-based strategies for the treatment of genetic and acquired diseases in children. This is an exciting and challenging area of research, requiring both laboratory and clinical expertise.

Our research program is focused primarily on the safe and efficient correction of genetic errors in the liver and bone marrow, since success in these organ systems has the greatest potential to impact on child health. The gene transfer systems we are using are virus-based, including recombinant adeno-associated virus and HIV-1-derived lentiviral vectors. We are also working to understand the body’s immunological responses to these gene transfer vectors, as well as vector-encoded transgene products.

Our unit has led the way in establishing gene therapy in Australia.

Laboratory-based research

Many of our studies involve mouse models, which allow us to carry out extensive experimental research in a living system before progressing to clinical trials involving children. In one such study, we have successfully treated mice with ornithine transcarbamylase (OTC) deficiency – a genetic condition that prevents ammonia (which is toxic to the body) being converted to urea so that it can be removed in the urine. This involves gene delivery to the liver and we are currently tackling the challenge of translating this therapeutic success in mice through to human therapy.

We are also using mice to improve the safety and efficiency of gene transfer to the bone marrow. This is especially important for our ongoing involvement in world-first clinical trials to treat infants with an immune deficiency disorder called SCID-X1.

Another of our research projects focuses on the unique immunobiology of adeno-associated virus (AAV). In particular, we are using this virus to define strategies that have the potential to be used to induce antigen-specific immune tolerance. Success with this project has broad implications for circumventing unwanted immune responses to gene therapy.

Clinical trials

A number of clinical trials are either underway or are in the process of being initiated. In a planned clinical trial for children with brain tumours, we intend to use gene transfer to increase the resistance of the patient’s bone marrow cells to the toxic effects of chemotherapy. This will hopefully allow more intense chemotherapy to be used, leading to improved tumour control. A clinical trial of gene therapy to treat OTC deficiency is also planned for the next few years and our involvement in clinical trials to treat SCID-X1 is ongoing.

Establishing the infrastructure needed to support these translational activities has been a major area of effort, involving the construction of a human application laboratory (HAL) in which human cells can be gene-modified for clinical trial use. This facility is the first of its kind in Australia and will allow us to be of service to other Australian research groups wishing to participate in gene therapy trials.

Major achievements

- We have led the way in establishing gene therapy in Australia. We were the first in Australian medical history to treat a patient with genetic disease using gene therapy.
- We were responsible for introducing both AAV and HIV-1-derived lentivirus vectors to the Australian research community.
- We have successfully used gene therapy to treat mice with the immune condition SCID X-1 and to repair liver cells in OTC-deficient mice.
- We designed, constructed and commissioned Australia’s first Human Applications Laboratory, allowing human cells to be genetically altered prior to being used as gene therapy.
“Gene therapy has huge potential to treat conditions caused by faulty genes.”

Gene therapy, the use of genes as medicine, is a new and rapidly expanding area of research. “Gene therapy is a radically different approach to treatment and has huge and, as yet, unrealised potential,” says Dr Alexander, Head of The Children’s Hospital at Westmead’s Gene Therapy Research Unit.

One research project showing great promise is on the use of gene therapy for an inherited defect that can occur in newborn boys, called OTC (ornithine transcarbamylase) deficiency, a defect of the urea cycle. Newborns with this condition have faulty liver cells that are missing a gene needed for the conversion of ammonia – a chemical that is highly toxic to the body – to urea, which is excreted in the urine. “Little boys with this urea cycle defect can die very quickly if it is not detected very soon after birth,” explains Dr Alexander. “If detected soon enough, the child may survive, but will need a liver transplant and immunosuppressive therapy for the rest of his life.”

Dr Alexander and his team have recently obtained some very exciting results in mice, successfully using gene therapy to repair the faulty liver cells responsible for OTC deficiency. “By transferring a copy of the missing gene into the liver, we have managed to cure mice of this condition. We do this by injecting a viral vector into the abdomen – a virus that carries the gene into the cells of the liver, correcting the deficiency and rendering them able to function normally.”

Having successfully used gene therapy in mice, the challenge is to translate this work to humans. “We now need to take the many necessary steps to move this experimental therapy on to the stage where it can be applied in children in a clinical setting,” says Dr Alexander.

“What is particularly exciting about this therapy is that, apart from being able to cure children with urea cycle defects, there is the potential to treat a wide range of other metabolic defects that affect the liver, such as haemophilia and PKU (phenylketonuria). What we are seeing now may just be the tip of the iceberg.”

Case study

When he was born, Abdalrahman had a rare liver enzyme deficiency that put him at risk of developing lethal ammonia levels in his blood. Luckily, the condition was quickly diagnosed and action taken. To have any chance of a healthy life however, Abdalrahman needed a liver transplant that could not be performed until he reached a safe weight at about ten months of age. Fortunately he made it through to this age and was lucky to receive the new liver he so desperately needed. To prevent rejection of the liver, Abdalrahman will need to take immunosuppressive medicines for the rest of his life.

Our hope is to be able to treat infants like Abdalrahman using gene therapy to repair the enzyme defect in the liver that causes this condition.
Overview of research
The Institute of Endocrinology and Diabetes is the leading clinical paediatric endocrinology research centre in Australia. The key theme of our research is prevention. Our main aims are to prevent the onset of diabetes in children, to prevent the development of diabetes complications and to prevent bone pain and fractures in children with osteopenia.

Type 1 diabetes and its complications
The incidence of type 1 diabetes in Australia is increasing by approximately 2.7 per cent per year and more than 1000 children each year are now diagnosed with this disease. We are leading a number of multicentre collaborative studies to improve our understanding of the causes of type 1 diabetes. These studies cover:

- risk factors that may trigger diabetes including viral infections (eg. enterovirus), vitamin D deficiency and dietary factors
- the effect of interventions (eg. intranasal insulin) on preventing diabetes
- the contribution of genetics to diabetes
- the effect of gene variations on the frequency and severity of diabetes complications
- vascular changes and soft tissue thickening in early diabetes complications and their relationship to more severe subsequent complications
- ways to reduce the development of kidney abnormalities
- changes in lung function and heart rate variability.

We are the leading clinical paediatric endocrinology research centre in Australia.

Type 2 diabetes and insulin resistance
Our research into type 2 diabetes includes population-based epidemiological studies, aimed at determining the incidence and risk factors associated with this disease in children in NSW and Australia. We are also leading a number of intervention studies in young people with insulin resistance syndrome, to investigate the effect of modified carbohydrate intake and whole body vibration on insulin sensitivity.

In related research, we are investigating environmental factors that influence obesity, metabolic syndrome and cardiovascular risk, the evolution of insulin resistance in daughters of mothers with polycystic ovary syndrome and the inflammatory role of visceral and subcutaneous fat in obesity.

Bone health
Osteoporosis can be a major problem in childhood. Children who have inherited osteoporosis or who have developed it due to a medical condition, such as cancer, are at risk of recurrent fractures. We are currently studying children who play sport to investigate the link between exercise and bone health and are studying the effect of whole body vibration training on bone mass and muscle function. We are also conducting research into osteopenia (decreased calcification or density of bone) and the prevention and treatment of vitamin D deficiency (a risk factor for osteopenia).

Clinical studies
In current clinical studies we are focussing on the impacts of new diabetes therapies, including insulin pump therapy, continuous glucose monitoring and multiple daily injection therapies. We are also conducting innovative studies on cortisol secretion in children at risk of adrenal insufficiency.

Major achievements
- We were the first to report a rising incidence of diabetes in NSW and have led collaborative studies of diabetes incidence throughout Australia.
- Our research into type 1 diabetes has laid the groundwork for vaccine development and randomised controlled studies to prevent diabetes.
- Our findings have changed the way paediatricians manage diabetes. In particular, we have contributed to the understanding of diabetes complications.
- Identifying risk factors for metabolic complications in young people has formed the basis of our current randomised control trials on the management of insulin resistance and pre-diabetes.
- We have led the development of databases for bone mass and body composition using dual energy X-ray absorptiometry. Our expertise has enabled us to address key issues in secondary osteoporosis in children and we are leaders in the use of bisphosphonate therapy.
Helping health service professionals
One of our aims is to build the capacity of area health service professionals to promote nutrition and physical activity, in an effort to address the problem of obesity in children. This is the subject of a study funded by the NSW Health Western Child Health Network, ‘Promoting healthy kids – everyday, everywhere’. This is the first study to develop and evaluate a health professional training package in simple paediatric obesity assessment and management.

Major achievements
- Our team was the first to show that muscle metabolic abnormalities, characteristic of insulin resistance (pre-diabetes), are present in apparently healthy children at familial risk for the development of disorders associated with obesity and diabetes.
- We have shown that abdominal fat is inversely associated with birth size and positively associated with risk factors for diabetes and heart disease – findings that highlight the very early onset of abnormalities that predispose to these diseases.
- We have identified a range of factors in mid-childhood that predict the development of obesity (especially central obesity) in adolescence.
- As a result of work we undertook in collaboration with the International Obesity Taskforce, the World Health Organisation established an Expert Consultation on Obesity.
- We have been instrumental in raising awareness in Australian State and Federal Health departments of the need for a coordinated model of care for the treatment of childhood obesity and the importance of obesity prevention.
Case study

Charmain came to the Hospital at age 13. She had a strong family history of type 2 diabetes and was carrying excess weight around the abdomen – a condition linked to type 2 diabetes in adolescents. Tests showed that Charmain had very high insulin and blood sugar levels as well as other complications associated with type 2 diabetes. Significant lifestyle changes were made and Charmain managed to lose eight kilos and reduce her waist circumference by seven centimetres during the first year of treatment. These changes led to the resolution of her diabetes and other medical complications.

Our research has shown that children and adolescents can reduce their risk of type 2 diabetes by keeping their waist circumference to less than half their height.

"Our simple health message is: ‘Keep your waist less than half your height.’"

Obesity is a major public health problem in Australia. In the mid 1990s, about one in five Australian children and adolescents were overweight or obese and it seems this number is ever increasing.

"It’s difficult to treat obesity, so prevention is the key," says A/Prof Chris Cowell, Head of the Institute of Endocrinology and Diabetes. “To do this, we need to identify the factors that lead to obesity.”

In 1996, a collaborative study began at The Children's Hospital at Westmead to explore the factors that influenced obesity in childhood. During the study, children born at Nepean hospital in Penrith between August 1989 and April 1990 were followed up at age eight, 13, 15 and 17 years. At each visit, body composition, levels of physical activity and food intake in the children was looked at, as well as the general physical activity and food environment of their families.

Factors most likely to lead to obesity identified by the study included lack of activity, early puberty, consumption of soft drinks and maternal adiposity (overweight mother). In terms of the home food environment, the impact of mothers as role models for eating and as the primary gatekeepers for food, appeared to have a particularly strong influence on the dietary behaviours of the children.

"We found that, by eight years of age, over 15 per cent of boys and 23 per cent of girls were already overweight or obese. Five years later, this had increased to 28 per cent for boys and 29 per cent for girls,” says A/Prof Cowell. “Of most concern was the rise we saw in waist circumference over this time, because we know that excess weight around the waist area is related to metabolic complications, like increased blood pressure and insulin resistance. And over time, these can lead to major health problems like type 2 diabetes.”

“A simple prevention message we can give the community is ‘keep your waist circumference half your height.’ If parents and other care givers can keep this in mind for their children, it will help prevent health problems, like diabetes, later in life.”

Snapshot: Obesity in children

Clinical A/Prof Chris Cowell
Institute of Endocrinology and Diabetes
Centre for Kidney Research
Improving the understanding, treatment and prevention of kidney diseases

Group Leader (Laboratory research): Dr Stephen Alexander
Group Leader (Clinical research): Prof Jonathan Craig

Overview of research
The Centre for Kidney Research is dedicated to studying the causes, treatment and prevention of diseases of the kidneys and urinary tract. There are a number of research groups within the Centre, many of whom have national and international standing. The Centre is also the editorial base of the Cochrane Renal Group, which plays a major role in incorporating clinical epidemiology and evidence-based medicine into mainstream nephrology worldwide.

Our laboratory research
Laboratory-based research at the Centre is organised into three groupings. The Renal Genetics Group is investigating genetic renal diseases in patients, such as renal failure caused by mutations in the WT-1 gene. The Glomerulonephritis Research Group studies the causes and treatment of nephritis, the most common cause of renal failure in children in Australia, with a particular focus on T-cell directed forms of immunotherapy.

In the Transplantation Research Group, important studies are underway to better understand rejection in renal transplantation, particularly related donor transplants (parent to child), which are performed regularly at The Children’s Hospital at Westmead. This group forms part of the larger transplantation research group at the Western Hub, with whom there are close collaborations.

We are the NHMRC Centre of Clinical Research Excellence in Kidney Disease.

Clinical studies
The Centre’s clinical studies cover a wide range of kidney health issues. In the area of Aboriginal health, we are conducting studies with more than 2000 children to assess whether Aboriginal children in the southern states are more likely to have early markers of kidney disease, compared with non-Aboriginal children. We are also collaborating with a number of organisations to investigate health and illness in Aboriginal children in urban and large regional centres.

In our research into cancer and renal disease we are studying various aspects of cancer risk, such as screening, prognosis and treatment effectiveness, so that policy makers and health professionals will be informed about appropriate management strategies.

The CARI (Caring for Australasians with Renal Impairment) Guidelines, a project aimed at improving the health of patients with kidney disease by helping health care professionals adhere to evidence-based medical practice, is also based at our Centre. Most recently, we have undertaken an investigation into the use of central venous catheters and fistulas in new cases of end-stage kidney disease.

In our Incontinence Research Group, a population-based study is underway to determine the natural history and frequency of daytime wetting and risk factors for persistent daytime wetting and urinary tract infections.

In other research, we are conducting a study in children presenting to the Emergency Department with fever. Our aim is to measure the accuracy with which clinicians are able to diagnose serious bacterial illness, and to assess whether an algorithm-based decision support tool could be used to improve health outcomes in these children.

Finally, a series of projects is underway using qualitative research methods to improve the quality of care delivered to children with chronic disease. These include patient perspectives on living with chronic kidney disease and parental perspectives on caring for a child with chronic kidney disease.

Major achievements
- As the NHMRC Centre of Clinical Research Excellence in Kidney Disease, we have provided valuable infrastructure to young clinical researchers in Australia and developed a national network of clinician-scientists in kidney disease.
- The Cochrane Renal Group, through the publication of a large number of meta-analyses of kidney disease and the training of researchers in meta-analytic methods, has directly impacted clinical practice worldwide.
- Our laboratory-based groups have made a major contribution to international kidney research, including discoveries on the role of regulatory T cells in kidney disease and transplantation, the development of novel therapeutic strategies for T-cell mediated disease and the identification of genetic causes of kidney disease.
Overview of research
The Centre for Perinatal Infection Research was established in 1999 to address the significant clinical problem of infections in foetuses and newborn babies. These infections contribute to a large burden of disease and disability, as well as the burden of miscarriages, stillbirths and premature births.

Neonatal infections
Through our research, we aim to determine why the immune system of a newborn infant does not protect against certain viral infections, like herpes simplex virus (HSV), which can cause devastating disease.

To develop strategies to prevent infection and disease, we need to first understand the body’s immune defences at the site of entry of the virus. To do this, we are performing studies of immune cell (dendritic cells or DC) responses to HSV in mice. This will lead to a better understanding of innate defences against viruses across all age groups and should facilitate the development of new therapies.

Other immune cells, regulatory T cells (T regs), play a vital role in controlling autoimmunity and excessive immune responses to infection. We have recently shown that the neonatal mouse CD8+ T cell effector response to HSV is of slow onset, reduced intensity and short duration compared to that of adults. We have also shown that there is a relative dominance of T reg activity in neonatal mice after HSV infection. These and further studies are providing critical information for the development of therapeutics that provide life-long protective immunity without inducing autoimmunity.

National surveillance of infections
The incidence, presentation and management of many perinatal infections in Australia are largely unknown. We are running three collaborative studies of national surveillance (HSV, Hepatitis C virus and rubella) through the Australian Paediatric Surveillance Unit and are collaborating on a study of atypical mycobacterium. These studies are providing unique information on the burden of disease and epidemiology of infections and will be vital in the implementation of preventative strategies. We are also collaborating with investigators overseas to compare the incidence of these infections in other countries (InOPSU).

The incidence, presentation and management of many perinatal infections in Australia are largely unknown.

Antenatal and newborn screening
There is currently no national routine antenatal screening programme for infectious diseases in Australia. In particular, there is much debate as to the most appropriate antenatal screening policy for human immunodeficiency virus (HIV) and hepatitis C virus (HCV). It may also be appropriate to screen for other infectious diseases, such as toxoplasmosis or past chickenpox exposure. We are currently collaborating with the Macfarlane Burnet Institute to assess the current antenatal testing practice used by general practitioners and obstetricians throughout Victoria and NSW.

Major achievements
- We were the first to demonstrate that vaccinating mothers against herpes simplex virus (HSV) prevents the spread of HSV to the organs of newborn mice, but not to the brain. This has important implications for the design of vaccines.
- Our studies of newborn regulatory T cell (T reg) response to viruses demonstrated marked differences in the kinetics, magnitude and longevity of antiviral newborn CD8+ T cell responses that were previously unknown.
- We were the first to demonstrate that HSV causes programmed cell death of dendritic cells in a species and serotype dependant manner, important for the design of immunotherapeutics and vaccines.
- Our systematic review of antiviral agents for the treatment of cytomegalovirus (CMV) diseases in transplant recipients has revised clinical practice for this condition worldwide.
- We made the novel observation that a HSV type 2 strain, deficient in essential genes for replication, was unable to maintain stable latency in the nervous system. This provided a paradigm for important safety features of human HSV vaccines.
Overview of research
The National Centre for Immunisation Research and Surveillance of Vaccine Preventable Diseases (NCIRS) was established in 1997 after success in a federal government tender. With this funding, the Centre produces national reports on vaccine preventable diseases (VPDs), immunisation rates and adverse events following vaccination (AEFI) and conducts national sero-surveys. Vaccine trials in children and adults are also an important aspect of our work.

National reports and surveys
Our Centre produces national reports on VPDs and immunisation rates every two years, reports on indigenous people every three years and reports on AEFI twice a year. National serosurveys are carried out every four years to measure population immunity by age group for a wide range of VPDs. NCIRS also plays a major role in the writing and compilation of the Australian Immunisation Handbook.

Vaccine trials
Our vaccine trials group, led by Prof Robert Booy, regularly takes part in trials to determine best practice in vaccination for persons of all ages.

In a world-first, we recently collaborated with the Women’s and Children’s Hospital in Adelaide to evaluate giving acellular pertussis vaccine at birth, in addition to routine vaccination commencing at two months of age. Our data indicated that vaccination at birth led to early antibody production with no reduction in later immunological responses, compared with those vaccinated at two months.

In the first study to examine this issue in Australia, we are collaborating with the Menzies School of Health Research to measure the long-term persistence of immunity from hepatitis B vaccine in young indigenous adults immunised as babies in the Northern Territory.

We also conduct trials in frail elderly people, in collaboration with researchers at Westmead Hospital. We are conducting a world-first trial of conjugate versus polysaccharide pneumococcal vaccine and are examining how antiviral drugs should be best used when influenza outbreaks occur in nursing homes.

Trials in children with impaired immunity have included a study of response to pneumococcal vaccine in children with nephrotic syndrome and to human papilloma virus (HPV) vaccine in immunosuppressed children aged five to 15 years.

Trials sponsored by vaccine manufacturers have included evaluation of combined Haemophilus influenzae type b (Hib) – Meningococcal type C (MenC) vaccine and trials of influenza vaccines. Another important trial involved the long-term follow-up of adults who were among the first in the world to receive an adult-formulated pertussis vaccine in 1998/99.

We regularly take part in trials to determine best practice in vaccination.

Health and economic modelling
Mathematical modelling is needed to examine important vaccine policy issues where it would not be ethical or feasible to conduct clinical trials. Examples of modelling to inform immunisation policies and programs include projects looking at measles, varicella zoster virus and seasonal and pandemic influenza.

Economic evaluation is an important tool for health policy in vaccines. Recently we examined the cost-effectiveness of lowering the age threshold for the universal influenza vaccination program and of pharmaceutical-based strategies for the prevention/mitigation of pandemic influenza. We also reviewed economic evaluations of infant rotavirus vaccination.

Major achievements
- Our work with key government bodies and national reports has had a major impact on the availability of data to inform policy in infectious diseases control.
- We were awarded a $2.5 million National Health and Medical Research Council (NHMRC) Capacity-Building Grant, leading to the creation of a network of Infectious Diseases Modellers in Australia.
- Our NHMRC and Australian Research Council (ARC) funded vaccine trials are relevant to policy development both for pneumococcal disease and influenza.
Overview of research
The Department of Allergy and Immunology conducts two streams of research, one on immunology and one on allergy. Our allergy research, outlined below, primarily involves looking at the clinical features of childhood allergic disease, together with the consequences for the child and family. We also carry out long-term epidemiological studies of allergic disease in collaboration with the Woolcock Institute (Sydney) and the Murdoch Institute (Melbourne).

Egg allergy in children
Our research into egg allergy in children commenced in 2006. This research has focused on egg avoidance in children with egg allergy and examined the adherence of parents with egg allergy to egg-free diets and the outcomes of this.

Food protein intolerance
A clinical study documenting the Department’s 16-year experience of protein intolerance has recently been completed, in which rice was found to be a common and severe cause of food protein induced enterocolitis syndrome (FPIES), a gastrointestinal food hypersensitivity. This is the largest published series of cases of FPIES due to rice and, for the first time, has documented the increased severity of rice-induced cases.

Prevention and evolution of allergic disease
In collaboration with the Woolcock Institute, we are conducting a National Health and Medical Research Council (NHMRC) funded project to follow and examine the data generated by the Childhood Asthma Prevention Study. The follow-up of allergic and respiratory features of this cohort at eight years is due for completion in 2008. The association between infant feeding practices and subsequent atopy has been examined and we have also documented adherence with allergy prevention recommendations in this cohort.

Early life influences on allergic disease
Experiences early in life can have a strong influence on the allergies developed in later life. We are conducting a prospective 16-year follow-up birth cohort study of Tasmanian children followed since 1988–89, one of the longest allergic disease follow-up cohorts in existence. This research has been the first to show an interaction between early life upper respiratory tract infection and birth during the pollen season, with hay fever and rye grass sensitisation in later life. We are also examining the effect of peanut exposure in pregnancy on peanut sensitisation at age 16 years.

Experiences early in life can have a strong influence on the allergies developed in later life.

Psychosocial aspects
We have studied a number of aspects of the impact of allergic disease, particularly with relation to family stress and anxiety. This work has documented increased stress levels in the parents of children with atopic dermatitis. We have also studied the impact of food challenges on the stress levels of the parents of egg allergic children and found that the challenges reduce parental stress levels, irrespective of the challenge outcome. A study of the medical decision-making process in dealing with food allergy has also recently been completed.

HPV vaccine anaphylaxis
In conjunction with The Children’s Hospital at Westmead’s National Centre for Immunisation Research and Surveillance and the NSW Department of Health, we are participating in a study of anaphylaxis following human papilloma virus vaccination.

Major achievements
- We pioneered qualitative research looking at doctor/patient interactions with respect to allergic disease.
- Our food protein intolerance study showed, for the first time, that rice intolerance is more severe than cows’ milk or soy food protein intolerance.
- Our childhood asthma prevention study found omega 3, fatty acids and house dust mite elimination measures undertaken in early life do not prevent later allergic disease.
- Our study into egg allergy showed that resolution of egg allergy is not related either to dietary adherence, the stringency of the diet or the occurrence of accidental egg exposure.
- Our 16-year follow-up study on allergy was the first to demonstrate an interaction between birth in the pollen season, exposure to viral respiratory infection and the development of rhinitis and rye grass sensitisation in later life.
Overview of research
The Department of Infectious Diseases and Microbiology conducts research into basic viral immunopathological mechanisms, as well as clinical research into infectious diseases in children and diagnostic testing. The key theme of our research is pathogenesis and diagnosis. Our main aims are to understand mechanisms of disease, clinical features and laboratory diagnosis.

Viral immunopathological mechanisms
We are investigating a group of viruses called flaviruses, specifically the West Nile virus, which can cause encephalitis and is an emerging virus worldwide. Our research is aimed at elucidating the mechanism by which the virus induces a host response, with a view to developing ways to minimise the immune response.

It is so far known that flaviruses cause up-regulation of cell surface expression of molecules critical for immune response. This up-regulation of immune-recognition molecules, which is very rare in viral-host interactions, is paradoxical as the virus signals for an enhanced immune response against the invading virus. The interaction between the virus and the host is a direct effect on gene expression and is not mediated by soluble products, such as cytokines.

Our research aims to determine which regions of the genes encoding these immune recognition molecules are responsible for this up-regulation. We expect this research to provide new information about the mechanisms of expression of these genes, as well as about the interaction of flaviruses with the host.

Basic research underpins our understanding of the pathogenesis of diseases

Clinical research
Our clinical research addresses important issues in infectious diseases and has the ultimate aim of providing better outcomes and quality of life for young people. Our research into clinical paediatric infectious diseases covers many areas. Recent research topics include:

- influenza
- norovirus
- infections in lawnmower injuries
- community acquired methicillin resistant Staphylococcus aureus infections
- urinary tract infections
- neonatal infections
- antimicrobial use and resistance in microorganisms in intensive care units
- implementation and outcomes of a hospital-wide antimicrobial stewardship program.

Diagnostic methods
The research we carry out into diagnostic methods aims to improve our laboratory function, enhance our understanding of paediatric infections and improve patient care. Recent research has involved:

- adenovirus genotypes
- human metapneumovirus
- detection of beta-lactamases
- detection of plasmid-mediated AmpC resistance in gram negative rods
- detection of H5 influenza.

Major achievements
- Our diagnostic research has improved clinicians’ capacity to identify pathogens in children with a wide range of infections.
- Our clinical research has led to the publication of manuscripts describing the management and outcome of infectious diseases in children.
- Our basic research has led to the identification of the intra-cellular signalling pathway used by flaviruses to alter expression of immune recognition molecules.
Case study

Chau came to the Hospital when she was five weeks old after developing a bad cough. Her parents noticed she had “stopped breathing” during her sleep the day before. Tests at the Hospital showed that Chau had a pertussis (whooping cough) infection and treatment began. However, while antibiotics are effective in reducing spread of the disease, they are not as effective in treating an established infection. Chau went on to have many more coughing fits and episodes where she stopped breathing – a very stressful time for both parents and staff. After almost three weeks in hospital, Chau was able to be sent home and has since recovered fully.

Our research has shown that infants like Chau could gain protection from a pertussis vaccine given at birth, instead of at two months of age.
Overview of research
The Australian Paediatric Surveillance Unit (APSU) is a vital component of the national network of agencies that monitors the health of children in Australia. Our Unit conducts monthly national surveillance of, and research into, a large number of rare childhood diseases. While these conditions may be rare, they are often chronic, have a significant impact on health resources and are of national public health importance. In many cases, APSU provides the only source of national data.

The APSU is an active member of the International Network of Paediatric Surveillance Units, which facilitates communication and cooperation among national surveillance units worldwide.

Diseases studied
In 2006/2007, a total of 12 communicable or vaccine preventable conditions were studied.

- Acute Flaccid Paralysis (AFP)
- Acute Intussusception (IS)
- Congenital cytomegalovirus infection
- Congenital rubella infection
- Perinatal exposure to human immunodeficiency virus (HIV), HIV infection and AIDS
- Neonatal herpes simplex virus infection
- Hepatitis C virus infection
- Non-tuberculous Mycobacterium infection
- Neonatal Group B Streptococcus Infection
- Varicella: neonatal, congenital and severe complications requiring hospitalisation
- Severe complications of influenza (including during a severe epidemic)
- Acute rheumatic fever

Other health conditions we studied during this period included:

- serious seatbelt injuries
- simple Vitamin D deficiency
- hyperinsulinaemic hypoglycaemia of infancy
- haemoglobinopathies
- Rett syndrome
- Vitamin K deficiency bleeding
- neuromuscular disorders.

Epidemiological data
The main aim of our Unit is to document the epidemiology, geographical distribution, clinical features, current management and short-term outcomes of specific rare childhood diseases. We strive to provide information that is truly nationally representative. Our mailing list includes 93 per cent of paediatricians listed by the Royal Australasian College of Physicians (RACP) as in active clinical practice in Australia and we obtain very high response rates from these clinicians (92.3 per cent for 2007). Even so, complete case ascertainment is unlikely, particularly from remote communities where children have limited access to pediatricians.

In 2007, we undertook a number of new collaborative projects. These included a project to standardise the classification of congenital anomalies in Australia, in collaboration with the National Perinatal Statistics Unit. We also developed and are piloting a hospital-based surveillance system in collaboration with the National Centre for Immunisation Research and Surveillance. In addition, we initiated a collaboration with the National Heart Foundation and the Menzies School of Health Research in Darwin to try to improve disease surveillance among Aboriginal communities.

APSU is an integral part of Australia’s national disease surveillance effort.

Our educational role
A key part of our role is to document and disseminate our data. To keep clinicians informed and to guide best practice, we regularly issue updated clinical and diagnostic information and publish our results in the medical literature. We also inform the broader community through the media and publications, such as our annual report. These educational activities help raise awareness of rare conditions in children, contributing to the early detection and timely management of these conditions.

Major achievements

- Over the past 15 years, we have undertaken 45 research studies involving over 300 clinician researchers, resulting in over 200 publications which have informed clinical practice, service development and policy.
- More than 1200 clinicians report clinical cases to the APSU monthly from a population of 4.1 million children aged <15 years.
- APSU has secured the support of the Department of Health and Ageing, NHMRC, Faculty of Medicine of the University of Sydney and the RACP Division of Paediatrics and Child Health.
Overview of research
The Centre for Trauma Care, Prevention, Education and Research (CTCPR) is the core centre for trauma services at The Children’s Hospital at Westmead. Our mission is to provide high quality services to children and adolescents injured due to traumatic events, to play a leading role in the prevention of injury among children and adolescents and to conduct groundbreaking research in trauma care, prevention and education.

The Centre also plays an important role in training health professionals in trauma-related clinical skills and in providing safety information and education to the public.

Preventing injuries in children and adolescents
There are many reasons that children and adolescents get injured. At different ages, young people are exposed to various kinds of environments and activities putting them at risk of different types of injuries. Our research aims to identify, as much as possible, the causes of different types of injuries so that future injuries to children and young people can be prevented.

For example, CTCPR is a member of a large research consortium investigating the causes of car crash-related injuries among young drivers in NSW. A large-scale study, in which more than 20,000 young drivers were followed for three years after obtaining their provisional licences, has just been completed.

In other research, we are investigating the causes of accidents in which young children drown in backyard pools, fall out of house windows, or are driven over in driveways. We are collaborating with The Children’s Hospital at Westmead’s Institute of Sports Medicine to investigate the causes of sports injuries in young people.

By identifying the causes of injuries in young people, we hope to be able to prevent some injuries in the future.

Managing trauma
One of our research projects is examining ways to improve the care and management of traumatically injured children admitted to hospital. Our aim is to evaluate the effectiveness of a case management approach using senior trauma nurses in providing care to moderately or severely injured children.

Recovery from injury
Children who sustain severe injury to their bodies require lengthy rehabilitation to regain normal function. This is particularly true for those who sustain a severe traumatic brain injury. One aspect of our research is focused on finding ways to help these children recover better. We are currently investigating the effectiveness of an individually tailored exercise program for children who have sustained moderate and severe traumatic brain injuries.

Major achievements
- Our research into factors that put young people at risk of motor vehicle crash injuries identified the number of passengers carried by drivers under the age of 25 as a significant risk factor. These results, together with those of subsequent research, led to road and traffic authorities in New Zealand and Australia implementing legislation to restrict the number of passengers carried by these drivers. Recent road statistics indicate a decline in motor vehicle-related crashes and injuries.
- Results obtained from our school bus-related injuries study were instrumental in the implementation of 40km/h zones around school areas during the time of day when most school children arrive at and leave from school.
- Our study on the transfer of paediatric trauma patients provided evidence for the Statewide Trauma Management Committee as a base for changing the statewide trauma transfer policy.
Division of Allied Health
Optimising the clinical care we provide

**Group Leader:** Alison Jones

**Overview of research**
The Division of Allied Health consists of several departments including Physiotherapy, Occupational Therapy, Orthotics, Orthoptics, Psychological Medicine, Pharmacy and Speech Therapy. Clinicians are involved in a range of research projects, most of which focus on developing and evaluating assessment methods and/or treatments.

**Occupational therapy**
A number of studies are being performed on cerebral palsy, including a randomised controlled trial of modified constraint-induced therapy for children with hemiplegic cerebral palsy. Other research topics include psychometric properties of the Paediatric Motor Activity Log and a comparison of assessments used in identifying fine motor outcomes of infants. A randomised trial of parent education versus individual occupational therapy on play outcomes is also underway.

**Pharmacy**
Research projects in the Pharmacy Department include the prevention of medication errors, the assessment of antiviral medications for preventing cytomegalovirus disease and the evaluation of certain drugs for the treatment of cystic fibrosis. In addition, we are participating in trials examining the efficacy of Vitamin C treatment for Charcot-Marie-Tooth disease type 1A, and the safety and efficacy of stoss therapy for Vitamin D deficiency.

**Physiotherapy**
In musculoskeletal research, we are conducting a comparison of knee exercise programs for the management of joint hypermobility, evaluation of an eccentric hamstring test in hypermobile children and vibration training for osteogenesis imperfecta. Research into cystic fibrosis includes an investigation of the effect of weight-bearing vibration and a randomised trial of the use of Mannitol. In rehabilitation research, we are investigating fitness training following traumatic brain injury.

**Psychological medicine**
Studies within the Developmental Psychiatry Research Group are furthering our understanding of children with developmental disability and mental health problems.

*A number of clinics at the Hospital are conducting research to optimise the care they provide.*

In the Family Therapy Research Clinic, trials are underway to investigate reflecting teams in family therapy and the effect of family therapy internships on the lives of interns. The Anxiety Clinic is researching young people living with a sibling with obsessive-compulsive disorder and/or another anxiety disorder and the Deafness Clinic is exploring the psychosocial needs of families of children diagnosed with congenital hearing loss.

The Telepsychiatry Research Team continues to evaluate the Child and Adolescent Psychiatry Telemedicine Outreach Service, while the Child Protection Research Team conducts collaborative research into Shaken Baby Syndrome and an investigation of Social Workers as educators.

**Speech pathology**
A number of studies into children with cleft palate are underway including investigations of obstructive sleep apnoea and feeding difficulties, the use of a computerised analysis module to facilitate speech study and a computerised system for the simultaneous collection of multiple cleft classifications. Research is also underway into velopharyngeal dysfunction and infants with Pierre Robin sequence.

**Orthoptics**
A retrospective study has been conducted of the previous ten years of paediatric eye injuries, focussing on the cause and location of the injury and final visual outcome.

**Major achievements**
- We completed a trial and systematic review of the use of Botox, with and without occupational therapy, in treating upper limb function in children with cerebral palsy.
- We developed a national competency framework for paediatric clinical pharmacists, in conjunction with the SHPA Paediatric COSP.
- We conducted a randomised trial comparing PEP and Acapella therapy for acute exacerbation in cystic fibrosis.
- Our Crux Cleft Palate Database continues to be used by centres in New Zealand, USA, The Netherlands and Canada.
- We published our work showing the effect of a hospital-based IV antibiotic course on the modified shuttle test in children with cystic fibrosis and completed a comparison of home based IV with hospital based IV antibiotic therapy.
Overview of research
Our group carries out clinical research into respiratory diseases, such as asthma and cystic fibrosis. We also study abnormalities in the control of breathing in children, in particular, Sudden Infant Death Syndrome (SIDS) and sleep apnoea.

Cystic fibrosis
In children with the genetic disease cystic fibrosis (CF), problems with water transport in the body result in a build up of mucous on the lungs and this often leads to bacterial infections. We are participating in a number of collaborative studies focusing on infections in CF, including the Australasian Bronchoalveolar Lavage (BAL) study on long-term outcomes of treatment against the bacterium Pseudomonas aeruginosa (PsA) and an Australia-wide study of the prevalence and influence of different clonal strains of PsA.

In our research into novel therapies for CF, we are involved in multicentre studies of aztreonam lysinate, a new inhaled anti-PsA antibiotic and are testing dry powder mannitol, a product that acts as thin mucous in the lungs. We are also assessing the effect of vibration plate therapy on lung function and are investigating the relative efficacy of different means to airway clearance.

Asthma
Asthma remains a common reason for hospital admission in children. In collaboration with groups in Perth and London, we are examining the normal growth and development of the airways and are investigating the development of asthma in early childhood. We are also assessing the influence of viral infections on the severity and duration of asthma exacerbations and evaluating a new technique for measuring lung function in young children.

We are always seeking to improve management approaches for asthma. This includes evaluating existing therapies to see if they may be used in new ways, such as oral anti-leukotriene as a rescue medication for asthma exacerbations.

Sudden Infant Death Syndrome (SIDS) and sleep apnoea
The causes of SIDS (also known as cot death) are still largely unknown. We are carrying out laboratory-based research to evaluate brain abnormalities found in infants who die from SIDS.

Major achievements
As a result of our work, the way respiratory disease is investigated and managed has improved. Services provided by The Children’s Hospital at Westmead have been enhanced and new therapies have become available.

- We have established exercise testing as an important tool in the assessment of children with chronic diseases that impact on cardiopulmonary functioning.
- Our finding that intravenous antibiotics administered in the home are as effective as those administered in hospital, has meant that some children can now stay at home during this therapy.
- Our research has shown that exposure to certain noxious stimuli, even if only in the post-natal period, can produce similar brain abnormalities to those seen in infants who die from SIDS. This indicates that vulnerability to SIDS need not necessarily be present before birth.
- We have identified brain abnormalities that are associated with exposure to SIDS risk factors, like prone sleeping and cigarette smoke.
- We have shown that the incidence of obstructive apnoea is much higher in children with cleft palate than in infants with normal airway structure.
Overview of research

Our Unit cares for newborn babies with cardiac and surgical problems or complex medical conditions. While the mortality rate in such infants has been declining steadily over the years, surprisingly little is known about their long-term outcomes.

The research we undertake is mainly focused on evaluating the best methods of treatment and assessing long-term outcomes that are important to babies and their parents. While we carry out a diverse range of projects, they have in common the ultimate aim of improving the care provided to babies and their families at The Children’s Hospital at Westmead.

Our Unit cares for newborns with cardiac and surgical problems or complex medical conditions.

Long term outcomes of infants

Every year, approximately 250 infants in NSW and ACT undergo major surgery. Such surgery, including cardiac surgery, is being undertaken in infants at a younger age than ever before and at smaller birth weights. To investigate long-term outcomes in these babies, we are conducting a long-term follow-up study in collaboration with the departments of Surgery, Cardiology and Cardiac Surgery at The Children’s Hospital at Westmead, as well as in four other tertiary centres in NSW.

Our research findings to date suggest that a significant proportion of children undergoing early cardiac surgery are left with permanent neurodevelopmental disabilities that impact substantially on their quality of life as well as that of their families.

In addition to behavioural and developmental problems, these babies often experience feeding and sleeping difficulties when taken home from hospital. These concerns are now the focus of an additional study being undertaken in collaboration with the Royal Children’s Hospital in Melbourne, examined the eating and sleeping behaviours of infants following cardiac surgery. The confidence of parents was high, however the fall in breastfeeding rates over nine months indicated a need for support strategies for these families.

Hospital outcomes are largely dependent on quality improvement in terms of critical incident monitoring and decision-making. We are exploring organisational culture and decision-making in a collaborative study highlighting how critical incidents managed at a local level can influence quality outcomes.

Other studies

Encephalopathy and cerebral palsy in newborn infants is another major area of study. Our Unit has been carrying out population-based research into the risk factors and outcomes of newborn encephalopathy and cerebral palsy for over ten years and our ongoing follow-up in this area of research continues to provide information that is currently available from no other source.

In research involving collaboration with the Royal Hospital for Women and University of NSW, we are studying the role of an inflammatory protein, Clara Cell Secretory Protein (CCSP), in newborn lung protection in premature babies. By examining the behaviour of CCSP in newborns, we hope to contribute to the improved understanding and future prevention of neonatal lung disease.

Another collaborative study, in conjunction with the Royal Children’s Hospital in Melbourne, examined the eating and sleeping behaviours of infants following cardiac surgery. The confidence of parents was high, however the fall in breastfeeding rates over nine months indicated a need for support strategies for these families.

Hospital outcomes are largely dependent on quality improvement in terms of critical incident monitoring and decision-making. We are exploring organisational culture and decision-making in a collaborative study highlighting how critical incidents managed at a local level can influence quality outcomes.

Major achievements

• Our team tested and validated a clinically reliable pain assessment tool, which has been recommended internationally for use in postoperative neonates.
• Our research into the risk factors and outcomes of newborn encephalopathy and cerebral palsy has led to numerous publications in international and national journals. A recent joint publication from the American College of Obstetricians and Gynecologists and the American Academy of Pediatrics cited our research papers as the “best available evidence” on neonatal encephalopathy.
• We were awarded funding from the March of Dimes Birth Defects Foundation to conduct a population-based study on the outcomes of surgery in early infancy.
Overview of research
The Department of Nuclear Medicine is the leading clinical paediatric nuclear medicine centre in Australia. Our main areas of research are clinically orientated, although considerable basic science research is undertaken in our bone mineral density and radiopharmacy sections.

We are the leading clinical paediatric nuclear medicine centre in Australia.

PET imaging in paediatrics
Since July 2006, Positron Emission Tomography (PET) has been available at Westmead Hospital and paediatric patients are scanned by The Children’s Hospital at Westmead staff at Westmead Hospital. We are currently conducting research to compare conventional radiological imaging and PET in staging paediatric solid tumours, to investigate the application of PET in assessing response to treatment and in detecting residual disease and recurrence in paediatric solid tumours. The study also examines the impact of PET on the management of solid tumours, especially lymphoma, bone and soft tissue sarcomas. In addition, we are evaluating the use of PET in assessing malignant transformation of neurofibromas in patients with Neurofibromatosis type 1 and paediatric epilepsy.

Bone mineral density research
The bone mineral density (BMD) section is involved in many clinical research projects on bone mineralisation and body composition, in conjunction with the Departments of Endocrinology, Orthopaedics, Genetics, Gastroenterology and Adolescent Medicine. Our research projects include assessment of BMD and body composition in adolescent patients with anorexia nervosa, various endocrine disorders such as growth hormone deficiency, nutritional disorders such as cerebral palsy, genetic disorders such as osteogenesis imperfecta and orthopaedic disorders such as avascular necrosis and osteoporosis. We are also conducting a new program assessing peripheral computed tomography (pQCT) in various bone disorders.

Radiopharmacy research
The Radiopharmacy Research Program includes the physiochemical aspects of bisphosphonates and the biodistribution of therapeutic and diagnostic bisphosphonate agents, such as pamidronate and zolendronic acid. New radiopharmaceuticals are being developed, in particular, technetium 99m-labelled bisphosphonates, labelled apoptosis agents to assess cell death after chemotherapy and radiotherapy and technetium 99m-labelled glucose agents to assess metabolic function of cancer cells.

Clinical studies
Our clinical research addresses important issues in paediatric oncology, renal disease (such as renal scarring and obstructive renal disease), paediatric bone conditions (including osteoporosis and body composition) and paediatric biliary diseases, as well as the development of new nuclear medicine techniques and radiopharmaceuticals.

Major achievements
- The Children’s Hospital at Westmead is the only paediatric Hospital in Australia that has a formal PET program for paediatric patients, performed and reported by paediatric nuclear medicine scientists and physicians. Over 1000 paediatric cases have been studied in the first two year period.
- Our research into applications of PET in paediatric solid tumours has laid the groundwork for appropriate application of this diagnostic test in the various forms of paediatric solid tumours, in the evaluation of malignant transformation of tumours and in the evaluation of intractable paediatric epilepsy.
- Our PET findings have changed the way paediatric oncologists manage paediatric solid tumours. In particular, the major contribution is in changing the stage of the cancer with the consequence of better prognostic indications and treatment change, in assessing response to treatment programs and in the earliest detection of recurrence of tumour.
- Our research in radiopharmacy has been important in understanding the biodistribution of bisphosphonates, which will impact on bisphosphonate treatment in paediatric bone disorders and in using the labelling of radiotracers to visualise apoptosis to enable imaging of cell death from chemotherapy and radiotherapy.
- In conjunction with the Departments of Endocrinology and Genetics, we have internationally led the development of databases for bone mass and body composition using dual energy X-ray absorptiometry.
Overview of research
The Nursing Research and Practice Development Unit has been in operation since 2005. We are currently undertaking a five-year practice development program, the broad aim of which is to ensure that care delivered at The Children’s Hospital at Westmead meets the needs of patients and their families. Practice development work is progressively being introduced to clinical units throughout the Hospital. Activities in the program encourage staff to reflect on current practice, identify the need for change and challenge themselves and each other to do better where possible. It is envisaged that engagement in these types of activities will lead to improved teamwork and staff satisfaction, sustainable changes in practice and, ultimately, improved family-centred care.

Action research
Activities offered through the program are many and varied. Action Research, a qualitative critical research methodology, is being used by several units and individual clinicians to collectively ‘plan, act, observe and reflect’ on specific areas of clinical practice. An international research collaboration between Australian organisations and those in Northern Ireland is offering a pathway to lifelong learning through an effective appraisal process, framework, personal development contract, reflective practice, and the development of a portfolio and formal and work-based learning opportunities.

In another collaborative project, we worked with the Nursing Faculty at the University of Sydney to conduct interviews with nurses, children and parents and carers to explore each group’s experience of caring in partnership with one another. This is forming the basis of an action plan to improve the care delivered to children and families.

Action learning and workshops
Action learning is a well-established strategy for reflective inquiry at an individual, collective and organisational level. There have been several action learning sets established at The Children’s Hospital at Westmead, based on the connection between reflection and action. The sets provide the opportunity for members to use high challenge/high support to work on real issues and take the time to reflect and learn from their own experiences. In a number of departments within the Hospital, staff development days have used creative techniques to help staff explore their role as care-givers and evidence-based practice workshops have been run to provide staff with baseline skills in formulating clinical questions, searching literature, identifying levels of evidence, critically appraising papers and implementing evidence into practice.

Action learning can help staff reflect and learn from their experiences.

Critical companionship
Several members of our team act as critical companions to nurses, both within and outside The Children’s Hospital at Westmead, to help them become truly person-centred in their work. This involves critical reflection and dialogue, through which the clinician learns what they need to change and how they can make those changes to transform their practice.

Major achievements
• We are leading an international project reviewing the theory underpinning the evaluation of practice development, as part of an International Practice Development Colloquium.
• We are collaborating internationally to investigate critical creativity as an integral element of practice development and the development of facilitators.
• Publication of our investigations into a negotiated care model in paediatric nursing has led to discussions with international experts in the field of family-centred care on further opportunities for collaboration between The Children’s Hospital at Westmead and Ireland.
Overview of research
Located at The Children's Hospital at Westmead, the Clinical School is a department of the Faculty of Medicine of The University of Sydney. There are more than 370 medical students at the School, together with 30 staff and a large team of volunteer teachers, tutors and examiners comprising medical and allied health staff both within and external to the Hospital. The School is a leader in medical education and medical education research and has a broad education and research role.

Clinical School activities
Our activities include investigating evidence-based methods of information delivery to students and medical professionals, participating in curriculum development for medical students, coordinating placements for elective students and junior medical staff, the local management and coordination of postgraduate students and their supervisors and the online delivery of a Masters in Medicine (Paediatrics) course.

The School offers clinical and research staff an opportunity to apply for conjoint and clinical titles and provides valuable teaching and professional development opportunities. A number of translational research programs within The Children's Hospital at Westmead are led by Clinical School Academic Staff.

The Clinical School is a leader in medical education and medical education research.

Partnership with The Children's Hospital at Westmead
The Clinical School operates in partnership with The Children's Hospital at Westmead to set the direction of teaching and education for medical and allied health staff. In keeping with this role, the School is actively involved in both the recruitment and professional development of a number of educators and researchers at the Hospital.

The School also participates in the overall strategic direction of research at The Children's Hospital at Westmead and works closely with the Hospital's Research Office, particularly in managing grants, scholarships and fellowships.

Major achievements
- Seven NHMRC grants, totalling over $4 million, were awarded in 2007 to Clinical School researchers based at The Children's Hospital at Westmead.
- The number of conjoint appointments at the School has almost tripled over the last few years.
- The successful partnership between the School and The Children's Hospital at Westmead has led to an increased number of clinicians participating in research and education. It has also created a vibrant and productive environment capable of attracting new research groups.
- A Masters Program in Paediatrics has been established, available online to national and international students.
- In partnership with The Children's Hospital at Westmead, we established the Kim Oates Australian Paediatric Simulation Centre to provide hands-on clinical skills training to students and health professionals at all levels of training.
Snapshot: Treating Cystic Fibrosis

Prof Peter van Asperen
Respiratory Medicine Research

“One of the best things about this treatment is that patients can treat themselves at home.”

Cystic fibrosis (CF) is the most common life-threatening genetic disorder affecting Australians. A hereditary disease affecting the mucus glands of the lungs, liver, pancreas and intestines, CF causes progressive disability and often a premature death. Prof Peter van Asperen and his research group are studying CF at The Children’s Hospital at Westmead to try to improve the treatment of this disease.

“In children with CF, a build up of mucous on the lungs often leads to bacterial infections, commonly *Pseudomonas*,” explains Prof van Asperen. “There are treatments available for these infections, but they often need to be given intravenously (IV).”

“A colleague of mine, Dr Peter Cooper, had previously tried treating *Pseudomonas* infection using an antibiotic administered through a nebuliser instead of IV, as it would normally be given. He had some success with this IV version of the antibiotic given via the inhaled route and a company in the US subsequently developed a nebulised version of the drug. We are now testing this nebulised antibiotic as part of a multicentre clinical trial in children with CF. So the trial has come directly from Peter’s clinical observation.”

The clinical trial, which began late in 2006, involves two stages. In the first instance, children received either the active medication (aztreonam lysinate) or a placebo over a period of 28 days. “We found that children who received the nebulised drug experienced significant improvement in symptoms, such as the frequency of cough, as well as improved lung function,” says Prof van Asperen. “Based on these results, the US drug company has applied to make this product available to all patients with CF.”

Stage two of the clinical trial is now under way, in which patients who choose to do so are continuing to take the drug for a further 18 months, at the end of which the results will again be analysed. Results are expected late in 2008.

“One of the best things about this treatment is that, because it is inhaled, patients can treat themselves at home, so the need for hospital visits is reduced. It also gives us another option for treatment when a patient is not getting much benefit from the antibiotic they are already on or has developed antibiotic resistance, which can happen if the same antibiotic is used over and over.”

Case study

James is a 16 year old boy who has cystic fibrosis (CF), an inherited condition which results in the build up of mucus in the lungs and other organs of the body. He repeatedly suffers infections in his airways, including that caused by the bacteria *Pseudomonas aeruginosa*. James’s cough interfered with his ability to exercise and go to school and his lung function had been deteriorating, so he agreed to take part in the Hospital’s study of a new nebulised antibiotic, Aztreonam. After treatment, James’s cough improved, he was able to exercise more and his lung function is now the best it has been for several years.

*This is one example of how the development of new therapies can benefit children involved in clinical trials, as well as those treated in the future.*
Research Committee

Chair
John Dunlop AM

Secretary
Anne O’Neill

Members
Dr Antonio Penna
A/Prof Chris Cowell
Daniel Petre AO
Dr Ian Alexander
Prof Kathryn North
Prof Peter McIntyre
Wendy Haigh

Research Executive

Chair
A/Prof Chris Cowell

Members
Anne O’Neill
A/Prof Chris Cowell
Dr Ian Alexander

Prof Kathryn North
Dr Leanne Mills
Prof Peter McIntyre
Trish van Leeuwen

Human Research Ethics Committee

Chair
Prof Kevin Gaskin

Secretary
Carolyn Casey

Community Members
Ruth Burleigh
Matthew Campbell
Paul Masson (to December 2007)

Executive Representative
Dr Antonio Penna (to May 2007)
Dr Stuart Dorney

Lawyer
Ian Butcher
The Hon. James Wood
Our committees

Minister of Religion
Margaret Bassett (to April 2007)
Sister Patricia Bolster

Professional Care Member
Dr Peter Cooper

Research Members

Allied Health Research
Helen Slatyer

Basic/Laboratory Research
Dr Bruce Benettts
Dr Nicole Graf

Anaesthetic/Surgical Research
Dr John Harvey

Nursing Research
Peter Lewis (to December 2007)
Margaret Kelly

Oncology Advisor
Dr Luce Dalla-Pozza

Clinical Trials Pharmacist
Pathma Moodley

Scientific Advisory Committee

Chair
Dr Ted Loughlin (to August 2006)
A/Prof Chris Cowell

Secretary
Carolyn Casey

Members
Dr Belinda Barton
Dr Bruce Lord (to January 2007)
Donna Rose
Dr Geraldine O’Neill (to February 2007)
Dr Gabrielle Williams
Dr Ian Alexander (to August 2006)
Dr Jonathan De Lima (to January 2006)
Dr Jonathan Egan
Prof Kathryn North (to October 2006)
Prof Kevin Gaskin
Dr Luce Dalla-Pozza
Prof Val Wilson
Dr Patricia Caldwell
Dr Stephen Alexander

Radiation Safety
Mark Hanlon (to December 2007)

Clinical Trials
Pathma Moodley

Form Review Committee

Secretary
Carolyn Casey

Members
Helen Slatyer (to December 2007)
Prof Kevin Gaskin (to December 2007)
Margaret Kelly
Peter Lewis (to December 2007)
Paul Masson (to December 2007)
Ruth Burleigh (to December 2007)

Amelia Hill – Secretary (to February 2007)
Margaret Athans – Secretary (to November 2007)

Intellectual Property Committee

Chair
Dr Antonio Penna

Secretary
Anne O’Neill

Members
A/Prof Chris Cowell
A/Prof David Little
Dr Ian Alexander
Dr James McCauley
Prof Kathryn North
Dr Ralph Hanson
Prof Valerie Wilson
Wendy Haigh

In Attendance
Paul Field, Biolink

Research Salary Review and Grading Committee

Chair
Anne O’Neill

Members
A/Prof Chris Cowell
Dr Ian Alexander
Dr Rachael Murray
Sharon Bau
John Dunlop AM (Chair)
Mr Dunlop became a member of the board of The Children’s Hospital at Westmead in 1973, going on to become President from 1983 to 2005. He also served as Honorary Treasurer (1978–1981) and Vice President (1981–1983). He is an external member of a number of The Children’s Hospital at Westmead’s committees as well as a Director of the Children’s Medical Research Institute and the Hospitals’ Contribution Fund of Australia Limited. Formerly Managing Director of Edwards, Dunlop & Company Limited, Mr Dunlop was a director of Health Super Pty Ltd and Health Super Financial Services Pty Ltd between 2000 and 2007. In 1987, Mr Dunlop was appointed a Member of the Order of Australia in recognition of his work for child health.

Anne O’Neill (Secretary)
Ms O’Neill has been Head of Research Administration and Operations at The Children’s Hospital at Westmead since 1998 and is currently the co-convener of the ACT/NSW Chapter of the Australasian Research Management Society (ARMS). At The Children’s Hospital at Westmead, Ms O’Neill is responsible for building high performance research support teams, supporting research of high quality and impact, enabling translational research and facilitating strategic planning for paediatric research. Her objective is to be nationally recognised as having played a leading role in the growth and development of health and medical research programs that produce world-class paediatric research outcomes. She holds an Honours degree in Science and is currently enrolled in a Master of Clinical Research at the University of Melbourne.

Dr Ian Alexander
Dr Alexander is Head of the Gene Therapy Research Unit at The Children’s Hospital at Westmead. His specific expertise and interests include virus-mediated gene transfer with a focus on target organs including the liver and bone marrow, both of which show great promise for the treatment of genetic disease in children. Dr Alexander’s team became the first in Australia to treat a genetic disease (SCID-X1) by gene therapy and he is recognised as a leader in the establishment of this exciting field in Australia. This is evidenced by his election as the inaugural president of the Australasian Gene Therapy Society in 2001 and more recently his appointment as Chair of the National Health and Medical Research Council’s Cellular Therapies Advisory Committee in 2007.

A/Prof Chris Cowell
A/Prof Cowell is Acting Director of the Research Division at The Children’s Hospital at Westmead as well as Head of the Institute of Endocrinology and Diabetes. He has also been Director of Clinical Research at The Children’s Hospital at Westmead since 2005, a part-time position created to help advocate and develop clinical research and its infrastructure. A Clinical Associate Professor of the University of Sydney, A/Prof Cowell trained as a paediatric endocrinologist in Toronto and Sydney and has extensive clinical experience in diabetes, growth, obesity-related metabolic syndrome and disorders of bone metabolism. His major research interests are the prevention of metabolic complications of obesity in teenagers, and the effects of disease states on bone mass and bone geometry.
Prof Peter McIntyre
Prof McIntyre is Director of the National Centre for Immunisation Research and Surveillance of Vaccine Preventable Diseases and is a Senior Staff Specialist in Infectious Diseases at The Children’s Hospital at Westmead. He has broad interests in vaccines and vaccine-preventable diseases (VPDs), particularly Haemophilus influenzae type b, Streptococcus pneumoniae and pertussis and VPDs in Aboriginal and Torres Strait Islander people. He has been Chief Investigator for a number of vaccine trials, most recently a trial of the first dose of pertussis vaccine at birth rather than two months of age. He is a member of the Australian Technical Advisory Group on Immunisation, the Communicable Diseases Network of Australia and the National Immunisation Committee. He has been an invited speaker at many international and national meetings and is the author of over 150 scientific papers and book chapters.

Prof Kathryn North
Prof North is the Douglas Burrows Professor of Paediatrics and Associate Dean of The Children’s Hospital at Westmead Clinical School, part of the Faculty of Medicine at the University of Sydney. Trained as a paediatric physician, neurologist and clinical geneticist, she completed a postdoctoral fellowship at Harvard Medical School before returning to Australia in 1995 as the recipient of the Children’s Hospital Research Career Development Award. At The Children’s Hospital at Westmead, Prof North runs the clinical Neurogenetics and Neuromuscular Service, and is Head of the Neurogenetics Research Unit and Deputy Head of the Institute for Neuromuscular Research. Her research interests include the molecular basis of inherited muscle disorders, genes that influence skeletal muscle function and new therapies for muscular dystrophy, neurogenetic disorders and learning disabilities.

Dr Antonio Penna
Dr Penna was appointed to the position of Chief Executive of The Children’s Hospital at Westmead following Prof Kim Oates’ retirement in 2006. For one year prior to this he was Director of Clinical Services – Medical. Before joining The Children’s Hospital at Westmead, Dr Penna was the Director of Medical Services at Royal North Shore Hospital, a position held since 1997. He completed his paediatric training at the Adelaide Children’s Hospital and was an NHMRC Postgraduate Fellow at the University of Melbourne, where he completed his doctorate in pharmacokinetics. In 1992, he became Clinical Superintendent in the Department of Paediatrics at Westmead Hospital, where he was subsequently promoted through a range of administration positions while maintaining a clinical role.

Daniel Petre AO
Mr Petre has been at the forefront of the technology industry in Australia for more than 20 years. Prior to founding Netus (a technology investment company), he spent nine years at Microsoft including three years as Managing Director, Australia. He went on to found Ecorp, a subsidiary of Publishing and Broadcasting Limited (PBL), which became Australia’s leading internet company. Mr Petre’s contribution to the not-for-profit sector has been extensive, with positions held on the Area Health Advisory Council for The Children’s Hospital at Westmead, the Advisory Board of HealthInsite and The UNSW Foundation, among many others. Mr Petre and wife Carolyn set up the Petre Foundation in 2000, which has funded research chairs both at The Children’s Hospital at Westmead and the Garvan Institute as well as a scholarship for university medallists at UNSW.
### 2007 grants

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### 2006 grants

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Our 2007 publications are listed below. For details of our 2006 publications please refer to our website, www.chw.edu.au/research.


Almqvist C, Li Q, Britton W, Kemp A, Xuan W, Tovey E, Marks G (2007). Early predictors for developing allergic disease and asthma: examining separate steps in the ‘allergic march’. Clinical and Experimental Allergy: Journal of the British Society for Allergy and Clinical Immunology 37(9): 1296-302.


Donaghue KC, Clarke SL, Cowell CT (2007). Increased Adiposity at Diagnosis in Younger Children With Type 1 Diabetes Does Not Persist. Diabetes Care 30(3) e10


In this document, the following publications are listed:


Publications


Tovey E, Kemp A, Almqvist C, Sharland A, Marks G (2007). Do immune responses to inhaled skin flakes modulate the expression of allergic disease? Clinical and Experimental Allergy: Journal of the British Society for Allergy and Clinical Immunology 37(8): 1199-203.


Weber-Chrysochoou C, Crisafulli D, Almqvist C, Li Q, Kemp A, Britton W, Marks G (2007). IL-5 T-cell responses to house dust mite are associated with the development of allergen-specific IgE responses and asthma in the first five years of life. The Journal of Allergy and Clinical Immunology 120(2): 286-92.


RESEARCH OFFICE

Division Chair
Prof Peter Gunning (to May 2007)
A/Prof Chris Cowell (Acting)

Research and Development Manager
Anne O’Neill

Operations Manager
Dr Leanne Mills (to September 2007)

Fundraising and Revenue Manager
Robin Dougherty (to August 2007)
Trish van Leeuwen (from October 2007)

Ethics Manager
Carolyn Casey

Clinical Trials Pharmacist
Pathma Moodley

Grants and Scholarships Officer
Amelia Hill

Executive Support Officer
Margaret Athans

Administrative Officer
Nicole Jacobs

Facility Support Officer
Lyndsey Bray

Statistician
A/Prof Jenny Peat (to December 2006)
Dr Cornelis Biesheuvel (from January 2007)

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Imran Hussain

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Rachel White (to June 2007)
Matthew Laver (from October 2007)

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Patricia McGregor
Deidre Shiel

Advanced Microscopist & Imaging Specialist
Laurence Cantrill

Microscopist
Jacqui Mills (to December 2006)

Engineer
Baptist Soo

Transgenic Facility Manager
Karen Brennan

Senior Technical Officers
Rebecca-Lee Rielly
Karen Knight
John Fisher (to April 2007)

Animal Technicians
Arturo Samcam (to November 2006)
Michelle Fuller (to December 2007)
Heidi Lecshmann
Michelle Sorrenson

ADOLESCENT MEDICINE AND EATING DISORDERS
Dr Susan Towns – Head, Adolescent Medicine
Dr Sloane Madden – Senior Staff Specialist
Dr Michael Kohn – Senior Staff Specialist
Prof David Bennett – Senior Staff Specialist
Helen Bibby – Clinical Psychologist
Dr Paul Rhodes – Senior Lecturer, Clinical Psychologist
Andrew Wallis – Senior Social Worker
Jane Miskovic – Research Psychologist
Natasha Cormarin – Research Psychologist
Susan Sampson – Clinical Nurse Consultant
Ronalda Hoffman – Senior Social Worker
Dr Basiliki Lampropoulos – Staff Specialist, Teen-Link
Popi Zappia – Psychologist, TeenLink
Natasha Coumerin – Psychologist
Colleen Alford – Psychologist
Bin Moore CNC – Transition
Michelle Casey – ChiPS Co-ordinator
Dr Simon Clarke – Head, Adolescent Medicine
Westmead Hospital, SSS CHW
Gail Anderson – Clinical Nurse Consultant
Fiona Robards – CAAH Co-ordinator
Linda Ramsbottom – CAAH Project Officer

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Alison Jones – Chair, Division of Allied Health

Audiology
Rosemary Douglas – Head Audiologist
Batoul Khalife – Audiologist
Sarah Love – Audiologist
Lindsey Mak – Audiologist
Latha Ramesh – Audiologist
Romayne Shakespeare – Audiologist
Rebecca Summons – Audiologist
Jean Tsembis – Audiologist
Sovan Taing – Secretary

Kids Health
Candace Douglass – Department Head
Lauren Jones – Health Promotional Officer
Dushyanhi Vimalachandra – Health Promotional Officer
Marea Planner – Administration Officer
Moira Tampoe-Mahendern – Administration Officer

Dietetics
Sheridan Collins – Department Head
Susie Burell – Senior Dietitian
Kerryn Chi sholm – Senior Dietitian
Fiona Deady – Senior Dietitian
Barbara Dennison – Specialist Allergy Dietitian
Daniela Gerlach – Dietitian
Susan Thompson – Senior Dietitian, Genetic Metabolic Disorders Service

Occupational Therapy
Alison Jones – Department Head
Margaret Wallen – Senior Occupational Therapist
Belinda Swain – Senior Occupational Therapist
Prof Anita Bundy – Honorary Appointment
Paula Bray – Occupational Therapist
Rachel Rolinson – Occupational Therapist

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Peter Barclay – Deputy Director of Pharmacy
Pathma Moodley – Clinical Trials and Drug information
Senior Pharmacist
Rachael Worthington – Senior Pharmacist, Clinical Services

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Kerry West – Deputy Head
Verity Pacey – Senior Physiotherapist
Kelly Evans – Senior Physiotherapist, Orthopaedics
Bronwyn Thomas – Senior Physiotherapist, Rehabilitation
Jan Hancock – Senior Physiotherapist, Rehabilitation

Psychological Medicine Psychologists
Dr Michelle Wong – Clinical Psychologist
Dr David Dossetor – Child Psychiatrist
Dr Sandra Heriot – Clinical Psychologist
Sandy Vickerstaff – Psychologist
Dr Victoria Grahame – Clinical Psychologist
Belinda Ratcliffe – Psychologist
Dr Paul Rhodes – Senior Clinical Psychologist
Dr Angela Dixon – Senior Clinical Psychologist
Karen Munro – Senior Clinical Psychologist
Siew Koo – Clinical Psychologist

Adolescent Medicine Psychologists
Helen Bibby – Clinical Psychologist/Reseacher
Jane Miskovic – Eating Disorder Research Co-ordinator
Popi Zappia – Individual and Family Therapist Psychologist TeenLink
Natasha Comarin – Intern Psychologist

Students
Ainslie Hatch – Doctoral Psychology

Social Work
Psychological Medicine
Sue Foley – Senior Social Worker
Robyn Lamb – Senior Social Worker

Adolescent Medicine
Ronald Hoffman – Senior Social Worker Head
Andrew Wallis – Senior Social Worker

Diabetes
Simone Kelly – Social Worker
Melissa Loos – Social Worker
Lynda Dunstan – Social Worker

ANAESTHETICS
Dr David Baines – Department Head
Dr Neil Street – Duty Head
Dr Stephanie Aplin – Anaesthetics Consultant
Dr Jenny Chien – Anaesthetics Consultant
Dr Michael Cooper – Anaesthetics Consultant
Dr Jonathan De Lima – Anaesthetics Consultant
Dr Peter Gibson – Anaesthetics Consultant
Dr Sue Hale – Anaesthetics Consultant
Dr Mary Hegarty – Anaesthetics Consultant
Dr Donald Innes – Anaesthetics Consultant
Staff and students

Dr Ramanie Jayaweera – Anaesthetics Consultant
Dr Sarah Johnston – Anaesthetics Consultant
Dr David Kinchington – Anaesthetics Consultant
Dr Mark Lovell – Anaesthetics Consultant
Dr Ian Miles – Anaesthetics Consultant
Dr David Murrell – Anaesthetics Consultant
Dr Michele O’Brien – Anaesthetics Consultant
Dr Lian Pfitzner – Anaesthetics Consultant
Dr Kristen Schwager – Anaesthetics Consultant
Dr Ian Sherratt – Anaesthetics Consultant
Dr Rasa Venclovas – Anaesthetics Consultant
Dr Sue Ann Wan – Anaesthetics Consultant
Dr Harry Wark – Anaesthetics Consultant
Dr Sally Wharton – Anaesthetics Consultant
Dr John Donnelly – Anaesthetics Consultant
Dr James MacDonald – Anaesthetics Consultant
Dr Jane McDonald – Anaesthetics Consultant
Dr Margaret Perry – Anaesthetics Consultant

Pamela Lopez-Vargas – Project Officer
Alison Lowe – Research Assistant
Ruth Mitchell – Trials Search Co-ordinator
A/Prof Paul Roy – Senior Clinical Researcher
Dr Giovanni Strippoli – Honorary Research Fellow
Allison Tong – Research Officer
Dr Angela Webster – Senior Research Fellow
Dr Gabrielle Williams – Senior Research Officer
Narelle Williams – Research Nurse
Rita Williams – Senior Aboriginal Health Education Officer
Narelle Willis – Cochrane Renal Group Co-ordinator
Sandra Puckeridge – Office Manager
Tamara Borysko – Administrative Officer
Leslee Edwards – Administrative Officer
Rachelle Samuels – Administrative Officer
Dr Yuan Min Wang – Senior Research Officer
Dr Debbie Watson – Research Assistant
Dr Geoff Zhang – Senior Hospital Scientist
Monique Macara – Research Assistant
Rebecca George – Research Assistant
Flavia Li – Research Nurse
Kate Rigney – Research Nurse
Effie Wong – Research Assistant

Students
Miriam Codarini – PhD student
Nick Cross – PhD student
Hasantha Gunasekera – PhD student
Leigh Haysom – PhD student
Angie Morrow – PhD student
Yashwant Sinha – PhD student
Germaine Wong – PhD student
Premala Sureshkumar – PhD student
Min Hu – PhD student
Tania Polhill – PhD student
Jeff Fletcher – PhD student

AUSTRALIAN PAEDIATRIC SURVEILLANCE UNIT

Prof Elizabeth Elliott AM – Department Head
Dr Yvonne Zurynski – Assistant Director
Karen Pattinson – Office Co-ordinator
Ingrid Charters – Administrative Officer
Nicole McKay – Data Manager
Dr Deepika Mahajan – Research Fellow
Elizabeth Peadon – Research Officer
Suwen He – Research Officer
Sarah Srikanthan – Publications Project Officer
Nicola Benwell – Research Officer

Students
Abbeye Evans – Masters Honours student
Franz Puttur – PhD student
Eddy Hasrul – PhD student

CENTRE FOR KIDNEY RESEARCH

Clinical and Laboratory Staff
Prof Jonathan Craig – Head of Clinical Research
Dr Stephen Alexander – Head of Laboratory Research
Dr Elisabeth Hodson – Head of Department of Nephrology/Centre for Kidney Research
Dr Patrina Caldwell – Senior Research Fellow
Denise Campbell – Senior Project Officer
Sonja Crampton – Data Manager
Sana Hamilton – Data Manager
Gail Higgins – Trials Search Co-ordinator
Michelle Irving – Senior Research Officer
Ashisha Kallukaran – Data Manager
Marianne Kerr – Data Manager
Anh Kieu – Research Assistant

Students
Abbeye Evans – Masters Honours student
Franz Puttur – PhD student
Eddy Hasrul – PhD student

CENTRE FOR PERINATAL INFECTION RESEARCH

A/Prof Cheryl Jones – Department Head
Dr Marian Fernandez – Senior Research Officer
Rose White – Senior Research Assistant
Maggie Brett – Senior Scientist
Meera Esvaran – Senior Research Assistant

Students
Abbeye Evans – Masters Honours student
Franz Puttur – PhD student
Eddy Hasrul – PhD student
Kenny Tang – BScMed Honours student
Wade Howden – Summer student
Enamul Hypaque – Summer student

CENTRE FOR TRAUMA CARE, PREVENTION, EDUCATION AND RESEARCH
Prof Danny Cass – Department Head
Dr Lawrence Lam – Deputy Director
Dr Soundappan Soundappan – General Surgery
Dr Gideon Sandler – Research Fellow
Frank Ross – Clinical Nurse Consultant
Fiona Fahy – Trauma Nurse Co-ordinator
Patricia Maglick – Data Manager
Glenda Portelli – Administration Assistant
Donna Green – Secretary

THE CHILDREN’S HOSPITAL AT WESTMEAD CLINICAL SCHOOL: DISCIPLINE OF PAEDIATRICS AND CHILD HEALTH
Prof Kathryn North – Douglas Burrows Professor of Paediatrics & Child Health; Associate Dean Clinical School; Head of Discipline
Prof Elizabeth Elliott AM – Consultant Paediatrician; Print Co-ordinator
Prof Louise Baur – Consultant Paediatrician
A/Prof Andrew Holland – Head, Academic Surgery; Associate Professor of Paediatric Surgery and Surgical Consultant
A/Prof Cheryl Jones – Postgraduate Co-ordinator; Paediatric Infectious Diseases Consultant
Dr Russell Dale – Senior Lecturer
Dr Soundappan Soundappan – Clinical teaching, Academic Surgery
Dr Meg Phelps – Lecturer
Dianne Campbell – Senior Lecturer; Sub-Dean (Education); Staff Specialist
Dr Shoma Dutt – Lecturer
Dr Patrina Caldwell – Senior Lecturer
Dr Anne Morris – Paediatrician Lecturer
Dr Joanna MacLean – Lecturer
Dr Shirley Alexander – Academic Fellow
Elizabeth Peardon – Staff Specialist
Wendy Oldmeadow – Lecturer Education Support
Leigh Smith – Executive Officer
Diane Hanlon – Office Manager
Vikki Cheetham – Education Support Officer
Fran Devasayaham – Administration Officer

Maureen Baker – Administration & Electives Officer
Lydia Beltran – Student Co-ordinator
Chris Perdu – Administration Officer
Sandra Harris – Postgrad Administration Officer
Jennifer Greer – Postgrad Administration Officer

DEPARTMENT OF ALLERGY AND IMMUNOLOGY
Dr Alyson Kakakios – Department Head
Prof Andrew Kemp – Professor of Paediatric Allergy
Dr David Isaacs – Staff Specialist
Dr Melanie Wong – Staff Specialist / Laboratory Head
Dr Preeti Joshi – Staff Specialist
Dr Dianne Campbell – Staff Specialist
Dr Wendy Allen – Fellow
Dr Genevieve Ostring – Fellow
Dr John Tan – Fellow
Dr Miriam Codarini – Fellow
Dr Sam Mehr – Research Fellow
Dr Wendy Hu – Research Fellow
Geraldine Dunne – Anaphylaxis Nurse Educator
Andrew Williams – Hospital Scientist
Lou Gacis – Hospital Scientist
Reta Nambiar – Hospital Scientist
Tina Hanlon – Technical Assistant
Alvin Benig – Technical Assistant
Francine Sanhard – Secretary
Jenny Cook – Administration Officer
Julie Bellenger – Administration Officer
Grace Cacino – Administration Officer

GENE THERAPY RESEARCH UNIT
Children’s Medical Research Institute and The Children’s Hospital at Westmead (Joint Unit)
Dr Ian Alexander – Department Head
Dr Sharon Cunningham – Research Fellow
Dr Julie Curtin – Clinical Researcher
Allison Dane – Research Assistant
Dr Samantha Ginn – Noel Dowling Research Fellow
Aiman Jajo – Honours student
Cindy Kok – PhD student
Margot Latham – Research Administrator
Dr Jerome Laurence – PhD student
Grant Logan – Senior Research Officer
Dr Christine Smyth – Senior Research Officer
Afrodit Spinoulias – Research Assistant
Maolin Zheng – Senior Research Assistant
Staff and students

CHILDREN’S HOSPITAL INSTITUTE SPORTS MEDICINE
Dr Robert Parker – Department Head
Dr Carolyn Broderick – CHISM Staff Specialist
A/Prof Gary Browne – CHISM Staff Specialist
Nancy van Doorn – CHISM Exercise Physiologist
Miriam Dawes – Administration Co-ordinator
Dr Damien McKay – Honorary Research Fellow
Rachel Turley – Physiotherapist
Kathrine McIntosh – Physiotherapist

A/Prof Veronica Wiley – Principal Scientist
Tiffany Wotton – Hospital Scientist

Eye and Developmental Genetics Research Group (Western Sydney Genetics Program, The Children’s Hospital at Westmead)
Dr Robyn Jamieson – Department Head
Dr Yongjuan Chen – Sir Norman Gregg Postdoctoral Fellow
Dr Linda Weaving – CJ Martin Postdoctoral Fellow
Rebecca Storen – Research Assistant

Students
Maja Popovic – Honours student
Luke St Heaps – Masters student
Marija Mihelec – PhD student
Peter Abraham – Honours student

Eye and Developmental Genetics Research Group (Embryology Unit, Children’s Medical Research Institute)

Eye Genetics Research Group
Prof John Christodoulou – Director, Western Sydney Genetics Program
Dr Hooshang Lahooti – Research Scientist
Andrew Grimm – Rett-base Co-ordinator
Ellen De Leon – Research Assistant
Dr Sarah Williamson – Postdoctoral Research Officer
Dr Rania Kairoz-Wahbe – Postdoctoral Research Officer
Dr Desiree Cloosterman – Postdoctoral Research Officer
Dr Xing Zhang Tong – Postdoctoral Research Officer
Gladys Ho – Senior Research Assistant
Joanne Perkins – Acting Business Manager
Danielle Pfitzner – Business Manager
Kerry Vallely – Personal Assistant
Brown Bale – Personal Assistant

Students
Vidya Nelaturi – PhD student
Roksana Armani – PhD student
Margaret Perry – PhD student
Abidali Mohamedali – PhD student
Katrina Slater – M Phil student

Biochemical Genetics and Newborn Screening Research Group
Prof Bridget Wilcken – Director, NSW Biochemical Genetics and Newborn Screening Services
Dr Kevin Carpenter – Principal Scientist Biochemical Genetics, Senior Lecturer

Skeletal and Lysosomal Disorders Research Group (collaborating with the Centres for Children’s Bone Health and Genetic Innovative Therapies)
Prof David Sillence – Department Head
Dr Lesley Adès – Department Head
A/Prof  Bruce Bennetts – Head, Department of Molecular Genetics
Dr Andrew Biggin – Honorary Research Fellow
Dr Maggie Brett – Hospital Scientist
Katherine J Holman – Hospital Scientist

Students
Kate Sullivan – PhD student
Sarah Bell – Honours student

Eye Genetics Research Group

Marfan Syndrome Research Group

Biochemical Genetics and Newborn Screening Research Group

Stall and students at the children’s hospital
Verity Pacey – Senior Physiotherapist
Dr Jenny Ault – Senior Staff Specialist – Department of Rehabilitation
Brenda D’Souza – Secretary
Kancy Ho – Administration Officer – Academic Department of Medical Genetics

Clinical Genetics Department
Dr Meredith Wilson – Department Head
Dr Felicity Collins – Staff Specialist
Dr Kristi Jones – Staff Specialist
Dr Janine Smith – Staff Specialist
Fiona Richards – Social Worker

INSTITUTE FOR NEUROMUSCULAR RESEARCH/NEUROGENETICS RESEARCH UNIT (incorporating Neuroinflammation, Neuropathology and CHERI [NF1 research])
Prof Kathryn North – Department Head
Dr Nan Yang – Senior Hospital Scientist
Dr Kate Quinlan – Postdoctoral Fellow
Dr Daniel MacArthur – Postdoctoral Fellow
Dr Peter Hoeving – Postdoctoral Fellow
Dr Biljana Ilkosvski – Postdoctoral Scientist
Dr Nigel Clarke – Postdoctoral Scientist/ Clinical Geneticist
Dr Aurelie Vandebrouck – Postdoctoral Scientist
Dr Sandra Cooper – Senior Hospital Scientist
Dr Rachel Peat – Postdoctoral Scientist
Michelle Moeskops – Unit Manager
Dr Russell Dale – Head of Team/Senior Lecturer Fellow
Dr Fabienne Brilot – Postdoctoral Scientist
Dr Brian Owler – Neurologist
Dr Lucy Wang – Postdoctoral Scientist
Dr Joshua Burns – NHMRC Fellow/ Postdoctoral Scientist
Kristy Rose – Clinical Trials Co-ordinator
Stephanie Wicks – Clinical Trials Co-ordinator
Jonathan Payne – Neuropsychologist – NF Research
Jennifer Lorenzo – Educational Psychologist – NF Research
Dr Rachel Susman – Neurogenetics Fellow

Students
Mimi Berman – PhD student/Clinical Geneticist
Jane Seto – PhD student
Monkol Lek – PhD student

Stephen Chan – PhD student
Fleur Garton – Masters student
Nancy Mokbel – PhD student
Angela Chen – PhD student
Frances Evesson – PhD student
Leigh Waddell – PhD student/Research Assistant
Paula Bray – PhD student
David Fitzsimons – PhD student
Esther Tantsis – PhD student

INSTITUTE OF ENDOCRINOLOGY AND DIABETES
A/Prof Chris Cowell – Department Head, Senior Staff Specialist
Dr Kim Donaghe – Senior Staff Specialist, Head of Diabetes and Diabetes Complications Assessments Service
Dr Geoffrey R Ambler – Senior Staff Specialist, Head of Clinical Information Systems and Clinical Technology
Dr Neville J Howard – Senior Staff Specialist, Principal Investigator, TRIGR (Australia)
Dr Maria Craig – Staff Specialist, Conjoint Senior Lecturer
Prof Martin Silink OA – Senior Staff Specialist
Dr Chris Poon – Senior Staff Specialist
Dr Craig Munns – Senior Staff Specialist
Dr Shubha Srinivasan – Staff Specialist
Dr Ann Maguire – Staff Specialist
Catherine Kay – Business Manager
Albert Chan – Clinical Infomation and Systems Manager/Statistician
Jenny Lee – Manager, Endocrine Laboratory
Dr Sarah Garnett – Clinical Research Fellow
Dr Kim Ramjan – Research Fellow
Dr Patricia Gallego – Research Fellow
Dr Jesper Johannesen – Research Fellow
Dr Xiong Feng – Clinical Observer
Dr Paul Benitez-Aguirre – Research Fellow
Dr Myra Poon – Research Fellow
Jim Minchenko – Research Scientist, Endocrine Laboratory
Charmaine Tam – PhD candidate
Alison Pryke – Research Assistant, Diabetes Complications Assessments Service
Ros Bongiorno – TRIGR Nutrition Co-ordinator
Glenda Fraser – TRIGR Nurse Co-ordinator
Staff and students

Janine Cusumano – CNS Diabetes Complications & Assessment Co-ordinator
Margaret Lloyd – Diabetes Prevention Research Nurse Co-ordinator
Lynne Foxall – Research Trial Co-ordinator
Lori Hopley – Research Assistant
Rachel Hayes – Senior Diabetes Dietitian
Samatha Clarke – Senior Diabetes Dietitian
Anna Pham – Dietitian
Simone Kelly – Social Worker
Melissa Loos – Social Worker
Lynda Dunstan – Social Worker
Oksana Markovych – Hospital Scientist
Darna Bradford – Senior Hospital Scientist
Mirjana Pupovac – Hospital Scientist
Rose Tesoriero – Hospital Scientist
Cathy Hassarati – Hospital Scientist
Catherine Loi – Hospital Scientist
Liz Lawrie – Clinical Nurse Consultant, Endo-testing Unit
Kelly Winning – Registered Nurse, Endo-testing Unit
Mary Maquade – Clinical Nurse Consultant, Bone Services Nurse Co-ordinator
Nuala Harkin – Nurse Practitioner
Carolyn Judge – Diabetes Educator
Nicole Matthews – Diabetes Educator
Rick Gray – Diabetes Educator
Janelle Collins – Diabetes Educator, Clinical Nurse Consultant
Anne Forgarty – Diabetes Clinical Services Manager, Clinical Nurse Consultant
Anne Craighead – Growth and Endocrine Nurse Co-ordinator, Clinical Nurse Consultant
Bin Moore – Growth and Endocrine Nurse Co-ordinator, Clinical Nurse Consultant
Tracey Jopling – Growth and Endocrine Nurse Co-ordinator, Clinical Nurse Consultant
Tina Willmore – Senior Administrative Officer
Kerrie Barker – Administrative Officer
Jane Haynes – Administrative Officer
Suzanne Forwood – Administrative Officer
Sandra Elliott – Administrative Officer
Lourdes Rontale – Information systems data entry and filing Administrative Officer
Jeanette Lock – Informations systems entry and filing Administrative Officer

JAMES FAIRFAX INSTITUTE OF PAEDIATRIC NUTRITION/ GASTROENTEROLOGY
Prof Kevin Gaskin – Department Head
Margie Gruca – Laboratory Manager
Jane Allen – Senior Research Dietitian
Caron Blumenthal – Senior Research Dietitian
Cheryl Frazer – Personal Assistant/Admin Officer

Students
Verena Haas – PhD student
Dorothea Stark – Master Nutrition Science
Melissa Hunter – Master Nutrition Dietitian
Fiona Deady – PhD student
Sharon Youde – Master Science Medicine student

KIDS HEART RESEARCH
A/Prof David Winlaw – Department Head / Paediatric Cardiac Surgeon
Dr Aniko Huizer-Pajkos – Manager
Dr Tanya Butler – Postdoctoral Scientist
Dr Vanita Lal – Genetic Counsellor
Yee Mun Tan – Research Assistant
Grace Lee – Research Assistant
Gina Walizada – Research Assistant
Joanne Hawkes – Technical Officer
Gillian Blue – Genetic Counsellor
Leigh Waddell – Data Co-ordinator
Jody Middlemiss – Administrative Support

Students
Carol Au – PhD student
Jonathan Egan – PhD student

NATIONAL CENTRE FOR IMMUNISATION RESEARCH AND SURVEILLANCE OF VACCINE PREVENTABLE DISEASES
Prof Peter McIntyre – Director
Prof Robert Booy – Head, Clinical Research
Prof Raina MacIntyre – Senior Principal Research Fellow
Dr Kristine Macartney – Deputy Director – Evidence Based Policy Support
Dr Shelley Deeks – Deputy Director – Surveillance
Dr Julia Brotherton – Senior Research Fellow
Dr Glenda Lawrence – Senior Research Fellow
Dr Nicholas Wood – Senior Research Fellow
Robert Menzies – Manager Indigenous/Migrant Health Projects and Program Evaluation
Dr Clayton Chiu – Clinical Research Fellow
Dr Leon Heron – Clinical Research Fellow
Dr Mary Iskander – Clinical Research Fellow
Dr Monica Lahra – Clinical Research Fellow
Dr Julie Leask – Research Fellow
Dr Helen Quinn – Research Fellow
Dr Amy Glasswell – Research Fellow
Cameron Moffatt – Research Fellow
Brynley Hull – Epidemiologist
Telphia Joseph – National Indigenous Immunisation Co-ordinator
Dr Jane Jelfs – Handbook & Policy Support Co-ordinator
Jo Backhouse – Scientific Officer
Han Wang – Statistician/Data Manager
Dr Zhanhai Gao – Clinical Trials Data Manager & Post-doctoral Modelling Fellow
Annemarie Egan – Nursing Co-ordinator of Clinical Trials
Lyn Barnes – Research Nurse
Elizabeth Clarke – Research Nurse
Kylene Gibbins – Research Nurse
Margaret Rose Joyce – Research Nurse
Jennifer Murphy – Research Nurse
Margaret Pym – Research Nurse
Laura Rost – Research Nurse
Carol Shineberg – Research Nurse
Pamela Cheung – Research Nurse
Dr Myra Parsons – Research Officer
Dr Padmasiri Aratchige – Research Officer
Dr Greta Ridely – Research Officer
Dr Iman Ridda – Research Assistant
Anita Heywood – Research Assistant
Andrea Schaffer – Research Assistant
Holly Seale – Project Officer
Ralf Itzwerth – Project Officer
Catherine King – Information Manager
Donna Armstrong – Communications Officer
Amanda Edkins – Information Management Assistant
Karyn Phillips – Business Manager
Lynda Beaumont – Personal Assistant
Danielle Marchant – Personal Assistant
Kirsty Whybrow – Administrative Officer
Joanne Perkins – Administrative Officer
Caroline Turner – NSW Health Public Health Officer
Alexander Rosewell – MAE scholar

Students
Michelle Cagney – PhD student
Holly Seale – PhD student
Mohamud Sheikh – PhD student
Nicholas Wood – PhD student
Robert Menzies – PhD student
Anita Heywood – PhD student
Iman Ridda – PhD student
Honglin Jiang – PhD student
Katherine Hale – PhD student
Anthony Newall – PhD student
Reena Gill – Masters of International Public Health
Dr Anna Dean – Masters of International Public Health
Emma Jan Inglis – Graduate Medical Program
Tania Moujaber – Graduate Medical Program
Jennifer Andersen – Graduate Medical Program
Cameron Armstrong – Graduate Medical Program
Michelle Nicholson – Graduate Medical Program
Hwee-Woon Lim – Graduate Medical Program,
USydSummer Research, Faculty of Medicine 2006/07 Scholarships
Abhijit Pal – Summer Scholarship
Craig Motbey – Summer Scholarship
Niccole George – Summer Scholarship
Shu Wang – Summer Scholarship
Mengzhi Zhung – Summer Scholarship
Meng Choo Leck – Summer Scholarship
Daniel Simpkins – Summer Scholarship
Wilson Chong – Summer Scholarship
Susanne Hansen – University of Copenhagen undergraduate Public Health Degree internship
Kader Kurt – Free University of Amsterdam, The Netherlands, Master of Science internship
Jennie Borg – University College PhD
Harun Rashid – Queen Mary MD
Haitham el Bashir – Queen Mary MD
Shamez Ladhani – Queen Mary PhD
Jo Tully – University College MD

CHILDREN'S HOSPITAL EDUCATIONAL RESEARCH INSTITUTE (CHERI) & DEVELOPMENTAL COGNITIVE NEUROPSYCHOLOGY RESEARCH UNIT (DECOG)

CHERI
Dr Belinda Barton – Department Head
Anneli Cassel – Research Assistant
Prof Max Coltheart – Academic Director and James Packer Chair of Educational Research
Sandra Crosson – Education Program Co-ordinator
Staff and students

Valerie De Fina – Education Program Co-ordinator
Susie Edmonds – Education Program Co-ordinator
Kirrilly Garvey – Administration Manager
Gerry Manuel – Administration Manager
Catherine Miskovich – Education Program Co-ordinator
Neil Nicoll – Psychologist
Erin Patten – Research Assistant
Katrina Sheraton-Yu – Education Program Co-ordinator
Dr Richard Webster – Paediatric Neurologist
Dr Belinda Barton – Head & Psychologist
Prof Max Coltheart – Academic Director
Janelle Levesque – Senior Research Assistant
Neil Nicoll – Developmental Psychologist

Students
Ajesh George – Doctoral student
Susan van den Berg – Postdoctoral student

NUCLEAR MEDICINE
Prof Robert Howman-Giles – Department Head
Prof Roger Uren – Physician
Dr David Chung – Physician
Dr Hamda Saleh – Physician
Dr Thomas Neil-Pascual – Registrar
Dr Kevin London – Research Registrar
Justine Trpezanovski – Chief NM Scientist
Theo Kitsos – Deputy Chief NM Scientist
Daniel Armao – NM Scientist
Suzannah Fuller – NM Scientist
Davina Hughes – NM Scientist
Nelly Keeka – NM Scientist
Julie Briody – BMD Scientific Officer
Madeleine Thompson – BMD
Mabel Harrison – Nurse
Natasha Soo – Secretary

Students
Peta Minton – D Psych student
Michelle Robison – D Psych student
Melissa Grogan – D Psych student
Joanna Ho – D Psych student
Georgia Phillips – D Psych student
Laura Schmalz – PhD student
Saskia Kohnen – PhD student

NURSING RESEARCH & PRACTICE DEVELOPMENT UNIT
Prof Valerie Wilson – Director
Margaret Kelly – Clinical Nurse Consultant
Vanita D’Souza – Administration Officer
Natalie Hooke – Registered Nurse
Peter Lewis – Registered Nurse Research Assistant
Annie Mills – Senior Research Officer

OBESITY RESEARCH GROUP
Janice O’Connor – Senior Research Assistant
Kristy McGregor – Research Assistant
Anthea Lee – Research Assistant
Jessica Finlay – Research Assistant
Dr Shirley Alexander – Academic Fellow
Sandra Harris – Administrative Officer
Vanessa Shrewsbury – Secretary

Students
Vanessa Shrewsbury – PhD student
Genevieve Dwyer – PhD student
Charmaine Tam – PhD student

ONCOLOGY RESEARCH UNIT
Prof Peter Gunning – Unit Head
A/Prof Jennifer Byrne – Group Leader
Dr Daniel Catchpoole – Group Leader
Dr Geraldine O’Neill – Group Leader
Janett Clarkson – Research & Development Manager
Cuc Bach – Research Assistant
Tina Borovina – Animal Technician
Lisa Corcoran – Research Assistant
Dr Susan Fanayan – Research Officer
Dr Thomas Fath – Research Officer
Dachuan Guo – Senior Research Assistant
Jayne Hardy – Research Assistant
Dr Doramys Hernandez – Postdoctoral Fellow (Swiss Fellowship)
Jeff Hook – Senior Research Assistant
Jessica Hyman – Research Assistant
Helen Jankowski – Research Assistant
Kerrie Jones – Tumor Bank Database Administrator
Belinda Kramer – Senior Hospital Scientist
Dr Antonio Lee – Research Officer
Frances Lemiert – Research Assistant
Dr Kimberley Lilischkis – Project Officer
Christine Lucas – Research Officer
Nicole Mackie – Technical Officer
Maha Mahmassani – Research Assistant
Skye McIver – Research Assistant
Megan Nicholson – Electron Microscopist
Dr Galina Schevzov – Research Scientist
Andrew Scott – Research Officer
Radhika Singh – Quality Officer
Dr Justine Stehn – Research Officer
Moira Tampoe-Mahendran – Administrative Officer
Dr Bernadette Vrhovski – Research officer
Julie Ward – Research Support Officer
Dr Judith Weidenhofer – Research Officer
Mark Wheeler – Tumor Bank Co-ordinator
Adrienne Williams – Research Assistant
Jessie Zhong – Research Assistant

Students
Ahmed Al Oqaily – PhD student
Jason Coombes – PhD student
Lauren Cowell – PhD student
Sarah Creed – PhD student
Nikki Curthoys – PhD student
Jeff Hook – PhD student
Prathibha Kahatapitya – PhD student
Belinda Kramer – PhD
Loretta Lau – PhD student
Claire Martin – PhD student
Mona Shehata – PhD student
Keerthi Thamotharampillai – PhD student
Franco Ubaudi – PhD student
Steven Wolf – PhD student
Malek Zhilif – PhD student
Agnes Chan – Masters student
Hamish Clarke – Honours student
Jessica Smith – Honours student
Tom Denee – Dutch Pharmacy student
Nina Heytveldt – Dutch Pharmacy student
Shiwei Ng – Dutch Pharmacy student
Lucy Soema – Dutch Pharmacy student
Alexander Fong – GMP Honours student
Adam Nelson – GMP Honours student
Julian Ng – GMP Honours student

ORTHOPAEDIC RESEARCH AND BIOTECHNOLOGY UNIT
A/Prof David Little – Unit Head
Dr Aaron Schindeler – Senior Research Officer
Dr Michelle McDonald – Research Officer
Dr Negin Amanat – Research Officer
Dr Craig Godfrey – Research Officer
Alyson Morse – Research Assistant
Hoai-Lan Mai – Technical Assistant
Kathy Mikulec – Technical Assistant
Lauren Stanmore – Technical Assistant
Dr Mark Latimer – Research Fellow
Dr Piers Mitchell – Research Fellow
Dr Tim O’Mara – Research Fellow
Dr Michalis Zenios – Research Fellow
Dr Manoj Ramachandran – Research Fellow
Dr Magnus Tägil – Research Fellow
Dr Lorrainne Harry – Research Fellow

Students
Renjing Liu – PhD student
Nicole Yu – PhD student
Deshan Goonetilleke – Engineering placement
Sandy Kijumnuayporn – Summer scholar
Joseph Choi – Visiting student

RESPIRATORY MEDICINE RESEARCH
Prof Peter van Asperen – Head of Department and MacIntosh Professor of Respiratory Medicine
Dr Peter Cooper – Staff Specialist
A/Prof Dominic Fitzgerald – Staff Specialist
Samantha Forbes – Research Nurse
Meredith Larkin – Research Nurse
Merilyn McArthur – Research Nurse  
Dr Karen McKay – Research Fellow and Co-ordinator  
Tracey Marshall – CNC Asthma Education 
Anna Middleton – Research Physiotherapist 
Margherita Pitman – Research Support Worker  
Dr Paul Robinson – Respiratory Fellow and PhD student  
Dr Hiran Selvadurai – Staff Specialist  
Brad Martin – CF Fellow  
Kim Gillett – Departmental Administrator  
Debra Roberts-Jones – Secretary 
Jane Gauci – Asthma Educator  
Brendan Kennedy – Manager, Respiratory Function Unit 
Ilaria Verrocchio – Scientific Officer 
Beth Weldon – Scientific Officer 
Edith Weatherall – Administrative Officer 
Mary Corbett – CF Co-ordinator  
Sharon Hunt – CNC, CF Unit 
Sharon Simonds – CNC, CF Unit  
Brooke Harris – CNC, CF Unit  

Students  
Kirstie Moore – Masters student  
Lucia Smith – PhD student  
Phillipa Yabsley – Honours student

SUDDEN INFANT DEATH SYNDROME AND SLEEP APNOEA RESEARCH  
A/Prof Karen Waters – Department Head 
Dr Rita Machaalani – Postdoctoral Fellow  
Dr Bronwyn Relf – Postdoctoral fellow  
Chenda Kol – Research Assistant 
Elissa Rao – Research Assistant  
Rashmi Nair – Research Assistant  
Kate Blakeney – Administration Assistant  

Students  
Samantha Tang – PhD student  
Joanna MacLean – PhD student  
Carla Evans – PhD student  
Rebecca Hensley – PhD student  
Sara Tan – Honours student  
Warde Elias – Honours student  
Leshni Pillay – Honours student  
Michelle Corke – Summer scholar  
Rashmi Nair – Summer scholar

THE GRACE CENTRE FOR NEWBORN CARE RESEARCH UNIT  
A/Prof Nadia Badawi – Co-Head Grace Centre, Neonatologist  
A/Prof Kaye Spence – Clinical Nurse Consultant  
Dr Peter Barr – Neonatologist  
Dr Nadia Badawi – Neonatologist  
Dr Kathryn Carmo – Neonatologist  
Dr Robert Halliday – Neonatologist  
Sharon Laing – Research Officer 
Dr Alison Loughran-Fowlds – Neonatologist 
Kaye Spence – Clinical Nurse Consultant Neonatology 
Karen Walker – Research Officer

VIROLOGY  
A/Prof Alison Kesson – Department Head  
Hanady Elbab – Research Assistant

WOUND HEALING LABORATORY/CHILDREN’S HOSPITAL BURNS RESEARCH INSTITUTE  
Dr John Harvey – Department Head, Burns Unit 
A/Prof Andrew Holland – Director, Burns Research Institute 
Dr Rachael Z Murray – Department Head, Wound Healing Laboratory 
Dr Vasant Rajan – Burns Fellow  
Dr Hugh Martin – VMO  
Dr Erik La Hei – Surgeon  
Dr John Pitkin – Surgeon  
Dr Peter Hayward – Surgeon 
Dr John Vandervord – Plastic & Reconstructive Surgeon  
Dr Robert Gates – Plastic Surgeon 
Dr Jenny Yuan – Burns Fellow  
Ya Wang – Research Assistant  
Bremilla Putnathanan – Research Assistant
We are constantly amazed by the passion and commitment of our donors and supporters in the community. Your interest in our work and unwavering support of our aspirations is one of our greatest strengths.

Our donors recognise that by supporting research, they are helping to create better futures for children and families, not just here in Sydney but across the state, our nation and indeed internationally. Our research into the causes, cures and treatments of childhood illnesses knows no boundaries and, with your support, the possibilities are endless.

We thank all our supporters for their invaluable contribution and we look forward to a long partnership in improving child health. The hope and comfort that your support brings to sick children and their families is truly priceless.
How you can help

Help us imagine how things could be...

At first glance, so many of the children at The Children’s Hospital at Westmead appear to be healthy, happy kids without a worry in the world… but each one of them has a story to tell. And while some of them are now able to look forward to the future with hope, for the others, every day is a challenge.

Many questions about child health remain unanswered and new challenges are posed every day.

Without sufficient funding, research into life-saving medical solutions would not be possible and cures to life-threatening illnesses will never be found.

All children, no matter who they are or where they come from, deserve the chance to live life to their full potential. Medical research is about understanding and eliminating disease to enable children to grow up healthy and improve their lives.

As one of Australia’s leading paediatric hospitals, The Children’s Hospital at Westmead has a long and distinguished history of world-class medical research.

Starting with 40 scientists, research at the Hospital has grown to more than 250 staff working across 30 research groups and is expected to grow by another 120 staff over the next five to seven years, including 60 staff in clinical trials research.

Remarkable progress has been made in medical research over the past 50 years. This century, medical breakthroughs promise an era of unprecedented advancements in research.

The Children’s Hospital at Westmead is respected worldwide and has gathered together extremely skilled, internationally recognised researchers to solve medical problems, ultimately allowing Australian children and children all over the world the opportunity to reach their full potential.

However, new medical challenges continue to arise. Now, more than ever, your help is needed for our commitment to research to be maintained. Your support may be measured in financial terms, but the impact it will make on the lives of all children is truly immeasurable.

To capitalise on the important advances to date, The Children’s Hospital at Westmead must meet its substantial commitment to fund vital research projects in the years 2008–2020 and beyond.

We have some of the most talented and dedicated medical research staff in the world here at the Hospital. But what makes that critical difference is making sure they have access to the most up-to-date tools and resources they need to do their jobs and deliver the best possible outcomes.

The continued generosity and support of our donors makes this possible. We need your help to ensure we have enough recurring funds this year and in years to come to provide vital equipment and support our research programs, clinical trials and researchers, as and when needed.

The advances in treatment and techniques which are improving survival and recovery so dramatically would not be possible without the extraordinarily generous support shown to us by our donors and supporters.

Our funding priorities are to develop and sustain self sufficiency for our research facilities, infrastructure and activity including:

- building our clinical trials capacity
- maintaining and managing our equipment, capital works and assets
- continuing and growing our research programs
- providing internal grants to our research units and renowned researchers
- implementing new initiatives

We want to be able to continue our current research projects and undertake more potentially life-saving research.
How you can help

When one of our generous supporters was asked, “Why do you give?” the answer was “because it affirms my right to imagine how things could be.”

Imagine...

Your gift today will play a vital role in enabling us to understand, treat and cure paediatric diseases, as well as improve the quality of life for children suffering from serious illnesses for which there is no cure.

Research has revealed the causes of many diseases. Yet, aggressive persistence is required for more cures and treatments to be developed. Those children who have managed to win their battle with life-threatening illnesses would not be here today without the critical breakthroughs that medical research has achieved.

But clinical trials, tests and research cost a staggering amount of money. Without sufficient funding, the cure to such puzzling diseases will never be found. That’s why your compassionate support is so important to ensure that our work continues – so that our children have the best possible hope of a future.

Every donation makes a difference – and with your support, we can make a special difference to the lives of countless sick children and their families. We ask you today to please make a gift to help us continue our life-changing research that helps provide better care and medical services to sick children and their families.

Our researchers see first-hand the impact that our work has had on many children and their families. It provides some certainty, comfort and most of all, hope for a healthier and brighter future for children with serious illnesses. Your generosity plays a vital role in this and all of our achievements and is a constant motivation for our entire research team at The Children’s Hospital at Westmead.

Affirm your right to imagine how things could be...

...today’s research is tomorrow’s medicine

To send a donation via cheque or mail, please direct your donation to:
The Fundraising Department, ATTN: Research Office
The Children’s Hospital at Westmead
Locked Bag 4001
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