



UCL INSTITUTE OF CHILD HEALTH

Great Ormond Street
Hospital for Children



NHS Trust

Research
Review 2009
The child first and always



Contents

Overview

- 05 Director's report
- 09 Chief Executive's report
- 11 Research and Development report

Research

- 14 Gaining insights from the complexity of immunity
Professor Tessa Crompton
- 17 Combating cancer with catechins
Dr Arturo Sala
- 18 How rare disorders can unlock the pathway to precise diagnoses
Dr Detlef Böckenhauer
- 21 Tracing the living pathways of the brain
Dr Chris Clark
- 23 Finding the triggers to mend a broken heart
Professor Paul Riley
- 24 Linking up the metabolic chain
Professor Simon Heales
- 27 Long-term health through life-long research
Professor Carol Dezateux CBE
- 29 Winding back the biological clock on damaged muscles
Dr Jennifer Morgan
- 31 Malfunctioning stem cells seeding cancer and epilepsy
Dr Thomas Jacques
- 33 Dietary supplements to prevent neural tube defects
Dr Nick Greene

People

- 36 Awards, honours and prizes 2009
- 40 Grants and donations 2009
- 44 Senior academic staff 2009
- 51 Administration 2009

Cover: Five-year-old Katie and Snowball, her teddy, are staying on Parrot Ward for a few days while doctors check if Katie has a blocked stent. They have been keeping busy by making plaster-cast animals, which are now dry enough for them to paint.

Left: Rosie, who is nearly three, is just finishing her strawberry jelly before going to her outpatient appointment on Safari Ward.



Careful, methodical and ethically-conducted clinical research asks “what can we do to help?” – the foundation for global improvements in medicine.

Using laser light to create visual slices through living tissue, our confocal microscope facility allows researchers to examine biological samples at the limits of optical resolution, revealing how nature works at the smallest possible scale.



Director's report
During 2009, the UCL Institute of Child Health (ICH) increasingly looked outwards, developing new relationships and working to increase its contribution to paediatric research throughout the UK and beyond.

In 2009, we were delighted to become part of UCL Partners, a new Academic Health Sciences Centre, in which UCL is joined by its four closest, research-active NHS trusts: Great Ormond Street Hospital for Children NHS Trust (GOSH), University College London Hospitals, Moorfields Eye Hospital NHS Trust and the Royal Free Hampstead NHS Trust. Child Health was one of the first areas of excellence to be adopted by UCL Partners. Led from the Institute by Professor David Goldblatt, the theme will have an early focus on obesity and diabetes, with intensive work planned in order to reach out more effectively into the community to improve the health of all children.

Several projects of national importance received significant grant awards. The Baby Biobank was launched in March, with funding from Wellbeing of Women (£1.6 million). Professor Gudrun Moore and Professor Lesley Regan (Imperial College London) lead the Baby Biobank, which will collect and provide to the scientific community, samples from pregnancies to advance understanding of disorders such as fetal growth restriction. The Institute hosts the Human Developmental Biology Resource (HDBR), which received five-year renewal funding (£2.1 million) from the Medical Research Council and Wellcome Trust. The HDBR, led by Professor Susan Lindsay (Newcastle University) and I, is the UK's only fetal tissue bank, enabling studies of gene expression in relation to genetic disease and birth defects. The UK Collaborative Study of Newborn Screening for Medium Chain Acyl Dehydrogenase Deficiency (MCADD) was launched during the year, led by Professor Carol Dezateux and funded by the Department of Health and National Screening Committee. Every newborn baby in England will now be offered a screening test for MCADD, a serious inborn error of metabolism.

We welcomed Terence Stephenson as Nuffield Professor of Paediatrics in succession to Professor Sir Al Aynsley-Green. Terence was formerly Dean of the Medical School at the University of Nottingham and is President of the Royal College of Paediatrics and Child Health. He takes on the task of developing general and adolescent paediatrics as an academic strength of the Institute, encompassing community-based and secondary-level paediatrics. Dr Simon Heales also joined the Institute as our first Professor of Clinical Chemistry, the result of a joint initiative with GOSH. The aim is to provide academic leadership and innovative research in clinical biochemistry, a vital area underpinning much of GOSH's work in identifying and treating inborn errors of metabolism and related childhood disease.

Institute staff who received honours and awards included Professor Carol Dezateux, who became a CBE for services to science. Carol's research is concerned with early life influences on child health, and the effectiveness of screening and other clinical and public health strategies to improve the health of children. Professor Mehul Dattani became Chair of the British Society for Paediatric Endocrinology and Diabetes, and Dr Richard Chin, one of ICH's academic clinical lecturers, was awarded the SPARKS (Sport Aiding Medical Research for Kids) prize 2009 for research into population aspects of childhood epilepsy. SPARKS also honoured Dr Maria Bitner-Glindzicz and Dr Shamima Rahman for excellence in medical research in relation to their work on preventable deafness, and Professor Janet Stocks and Dr Rachael Gregson for their studies of chest physiotherapy in ventilated children.

Marilena is a long way from home. She and her family are here from Cyprus and are staying on Butterfly Ward.



Sophie loves the playroom on Lion Ward and today she is making a card for her mum. She is being treated for a type of cancer called neuroblastoma. She has been in and out of hospital for a long while, but is full of beans and can often be found on one of her “little walks” around the ward.

Director’s report continued

A number of Institute members gained academic promotion at UCL. Dr Lyn Chitty became Professor of Genetics and Fetal Medicine for research into fetal ultrasound and the diagnosis of birth defects. Lyn recently obtained one of the first programme grants from the National Institute for Health Research (NIHR) to develop non-invasive prenatal diagnosis.

Therese Hesketh became Professor of Global Health for research into health and population issues in China. Therese recently gained £3.3 million from the Department for International Development to improve access to care in rural and urban China.

Paul Riley became Professor of Molecular Cardiology for ground-breaking advances in identifying a new source of stem cells in the heart, and pin-pointing the molecules that activate these stem cells. His research offers hope of improved therapies for disease in children’s and adult hearts.

Neil Sebire became Professor of Paediatric and Developmental Pathology for research into the pathology of the placenta, and a recent influential paper in *The Lancet* demonstrating a role for bacterial infection in unexplained infant deaths.

Andrew Taylor became Professor of Cardiovascular Imaging for research into examination of children’s hearts using magnetic resonance imaging (MRI). Andrew was recently made Senior Research Fellow of the NIHR in recognition of his achievements.

Staff promoted to Reader were Dr Nick Greene for research into the development and prevention of spina bifida, Dr Juan Pedro Martinez-Barbera for developmental biology studies of the forebrain and pituitary gland, and Dr Paul Winyard for research into disorders of the kidney, as well as leadership of educational programmes at the ICH.

Andrew Copp
Director
UCL Institute of Child Health



Chief Executive's report
At Great Ormond Street Hospital for Children NHS Trust (GOSH), our role is to provide the very best care and treatment for our patients and to give them the best possible chance of living long and healthy lives. To achieve this, we have always been and will always continue to be dedicated to finding better ways to treat, and hopefully cure, childhood illnesses.

Some of the very best research work is carried out in collaboration with other partners, nationally and internationally. In a world first in children, British and Italian doctors recently worked together to transplant a new trachea into a child, and used the child's own stem cells to rebuild it. The application of this technology should greatly reduce the risk of rejection of the new trachea, as the child's stem cells will not generate any immune response. This technique is also considerably simpler and potentially applicable to many centres around the world, including those in poorer countries.

This revolutionary procedure involved a Europe-wide team of laboratory scientists and hospital-based clinicians working together to deliver this extraordinary treatment. At GOSH, the surgery was led by Professor Martin Elliott, who developed Europe's first specialist tracheal surgery service for children.

Over the past 10 years, the Bone Marrow Transplant Unit at the hospital, led by Dr Paul Veys, has pioneered the use of gentler chemotherapy drugs to enable donor bone marrow grafts in children with primary immunodeficiencies, a condition which prevents them from fighting infections. This approach has significantly improved the outcome for such patients, and has now become standard care in many European and US centres. However, certain subgroups of patients, such as babies under one year old, still have a high risk of mortality from transplant, even with gentler chemotherapy.

This year, GOSH and the UCL Institute of Child Health developed bone marrow transplants that use virtually no chemotherapy. For the first time, researchers used antibodies that recognise the bone marrow to prevent rejection and create space for the donor stem cells. This antibody treatment avoids some of the most devastating short and long-term side effects caused by standard chemotherapy, such as hair loss, sickness, organ damage and infertility. The research was led by Dr Persis Amrolia, a consultant in bone marrow transplants at the hospital.

These are just two examples of what can be achieved by dedicated and motivated academics and clinicians working together to find better ways to help children with life-threatening and life-limiting illnesses. There are currently more than 700 research projects ongoing across the joint organisation – and many more examples of the pioneering work our researchers are carrying out are shown here.

Research and development will continue to be central to what we do, and with Child Health as one of the initial themes of the UCL Partners Academic Health Science Partnership, we look forward to collaborating even more widely in our work and finding even more ways to help the children who need us.

Jane Collins

Jane Collins
Chief Executive
Great Ormond Street Hospital
for Children NHS Trust

Philippos loves being the centre of attention on Elephant Ward, where he is being treated for leukaemia. He always has a big grin on his face, especially at mealtimes.



Research and Development report

Consolidating our programme of research for patient benefit.

During 2009, one important area of activity for research at Great Ormond Street Hospital for Children NHS Trust (GOSH) and the UCL Institute of Child Health (ICH) was the consolidation of research for patient benefit. The referral pattern of patients to GOSH results in relatively large numbers of complex and rare disorders gathering under the care of a hospital team. Such experience with rare conditions not only provides the opportunity to optimise care, but also enables pioneering research to be undertaken. Increasingly, research that will lead to patient benefit has been highlighted as a critical focus for the research carried out in the NHS, and GOSH and the ICH have responded.

The purpose-built Somers Clinical Research Facility (CRF), designed to provide both bespoke accommodation for ambulatory patients participating in research and a focus for research expertise for the joint organisation, celebrated its first birthday in December 2009. The facility has seen increasing numbers of patients pass through its doors – CRF staff have helped to establish 40 clinical studies, and more than 90 patients visit the facility each month for research studies. A wide range of studies is being undertaken, ranging from first-in-man new treatments for children with life-threatening neuromuscular and metabolic diseases, early and late phase studies in rheumatological and renal disorders, through to cohort and long-term observational studies in meningitis, autism and dermatology. This illustrates the breadth and range of the type of research performed in the environment of a tertiary care hospital with patient benefit as its focus.

GOSH and the ICH host the only National Institute for Health Research Specialist Biomedical Research Centre focused solely on children. We are very aware of our responsibility to contribute to the training of the next generation of

child health-focused scientists and clinical academics, and we therefore decided to award, in open competition, several training fellowships to support scientists at the pre- and post-doctoral phases of their career, as well as clinician scientists. One of the prerequisites for obtaining these awards has been the focus of the projects on linking laboratory-based scientists with those in the clinic. UCL has a superb scientific environment and it is the responsibility of closely-linked NHS and university units to translate some of that science into patient benefit.

GOSH and the ICH have enthusiastically embraced the first year of the UCL Academic Health Science Partnership, UCL Partners (UCLP). Child Health was adopted as one of the initial themes of the partnership and I was appointed as its lead. In September 2009, we held the inaugural symposium of the UCLP Child Health theme. This showcased some of the fine research being pursued across the wider north London child health community. Presentations focused on eye disease, obesity, epilepsy, stroke and service improvements, with a focus on respiratory disease, and several of these are being taken forward as UCLP initiatives. We hope, through these workstreams, to encourage research at the population level, ultimately bringing health gains and benefits to a much wider population than that represented on the wards in our hospital.

Professor David Goldblatt
Director of Clinical Research and Development

Ms Jo Southern
Head of Research and Development Office

It's pretty hard to take a photograph of 22-month-old Mohammed, as he is the fastest patient on Butterfly Ward. He spends most of his time zooming around, pushing his police-car walker in front of him.



Patients' needs lie at the heart of everything we do. Through a commitment to research, we strive to treat the untreatable.

A few days ago, Jessica had a brain tumour removed – not that you would know it from the way she plays dominoes. It's a fairly long walk for her to the playroom a few doors along the ward, but the journey back was a lot easier – a piggyback from dad.



Professor Tessa Crompton

“Twenty years ago, I chose to investigate T-cell development because I thought it would be straightforward: thymocytes were easy to separate and characterise, and I naively believed that the experiments would be quick to do. I was wrong. My long-term research goal – to work out how environmental cues from the thymus stroma regulate T-cell development – has proved elusive, our understanding of how cells mature seems superficial, and experiments are difficult to do. This unpredictability, and the complexity of biological systems, is why I love research.

“In 2007, my lab moved to the UCL Institute of Child Health, where our research has already benefited from support from the Immunobiology Unit and stimulus from new collaborators. We now look forward to working with Mike Hubank to investigate genetic changes in thymocytes as they mature, and with Graham Davies to refine thymus transplantation.

“Our research attempts to address basic scientific questions, but I hope that knowledge of the molecular regulation of T-cell development will improve treatments for many childhood diseases.”

Gaining insights from the complexity of immunity

Professor Tessa Crompton has dedicated her research to arguably one of the human body’s most complex biological processes. Her ambition is to understand the way in which we produce the millions of subtly and uniquely different immune cells that recognise and protect us from disease.

T-cells are the body’s primary defence against infection. Their correct growth and development is crucial to forming the full complement of circulating white blood cells that recognise and destroys disease-causing pathogens. Conversely, incorrect T-cell development leaves the body unprotected against infection, and can lead to malignant diseases, such as leukaemia, and a variety of autoimmune disorders.

Professor Crompton’s research has focused on the thymus, the organ responsible for producing and fine-tuning T-cells prior to their release around the body. Her group aims to understand how environmental cues from the thymus regulate the growth of different kinds of T-cells, and how developing T-cells called thymocytes interpret and integrate such signals. “Understanding the molecular regulation of T-cell development is important if we are to understand how the body fights disease,” Professor Crompton says. “We were the first team to show that a biological signalling molecule called Hedgehog had a role in T-cell growth and development in the thymus, a result which no one expected and which has kept us hard at work ever since.”

Hedgehog signalling would appear to act on T-cell development in the thymus in a somewhat counterintuitive way, stimulating T-cell growth at some stages and inhibiting it at others. Professor Crompton is keen to find the biological mechanisms that enable the same signal to act in opposite ways within the same organ.

“The way the thymus controls T-cell development is by selectively weeding out those developing T-cells which would attack the body’s own cells,” she says. “An interesting line of our work has shown that, as these developing T-cells migrate through different regions of the thymus, they receive different doses of Hedgehog signal. The strength of Hedgehog signal they receive

seems to be one factor that determines the fate of the developing cell, telling it to continue to mature and divide, or to die. So the structure of the thymus itself, and the position of the developing cell within that structure, would appear to be crucial to the signalling processes which fine-tune T-cell function.”

Professor Crompton is collaborating with Dr Graham Davies to see if their knowledge of the thymus can translate into improving the outcomes of patients with DiGeorge syndrome. This genetic condition affects one in 4,000 newborns. Symptoms vary between individuals, but can, in severe cases, include an underdeveloped or absent thymus, which means that children are unable to produce the T-cells needed to fight disease.

Last year, thanks to research funded by Great Ormond Street Hospital Children’s Charity, Dr Davies carried out the UK’s first thymus transplants in children with DiGeorge syndrome, as a way of replacing their missing T-cells. Such transplants have been a relative success, despite there being no opportunity to tissue-match the thymus transplant.

“These transplants are saving patients’ lives, even though they are not matched to tissue type,” Professor Crompton adds. “We now need to try to improve recovery of the immune system in these patients, so that they are in a better position to make the full repertoire of T-cells to fight off infectious disease. I think that, as we learn more about T-cell development and the structure of the thymus, we will be able to improve the technology for thymus transplantation, and we’ll see better outcomes in patients like those in need of a transplant with DiGeorge syndrome. To improve treatment for patients, we must gain a deeper understanding of the biological processes that control growth and development.”



James’ story

by his parents, Mandy and Tim

“The day after James was born, our lives were shattered when we were told he had a loud heart murmur. Taking him home from hospital felt like he was on loan: we knew we would be back – not the feeling we had expected. He was diagnosed a week later with the heart defect Tetralogy of Fallot, and we were told he would need surgery in his first year.

“Things went from scary to completely surreal a few weeks later when we discovered that he had DiGeorge syndrome and a zero T-cell count.

“Children with DiGeorge syndrome can have many problems, including cardiac defects, an inability to metabolise calcium, immunodeficiency, feeding problems, poor muscle tone, developmental delay and poor hearing. When James was a tiny baby, we were so scared for his future. We just wanted his life to be worth living.

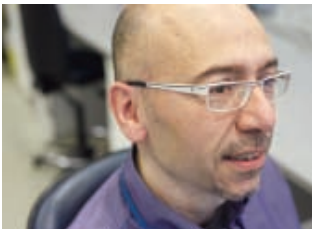
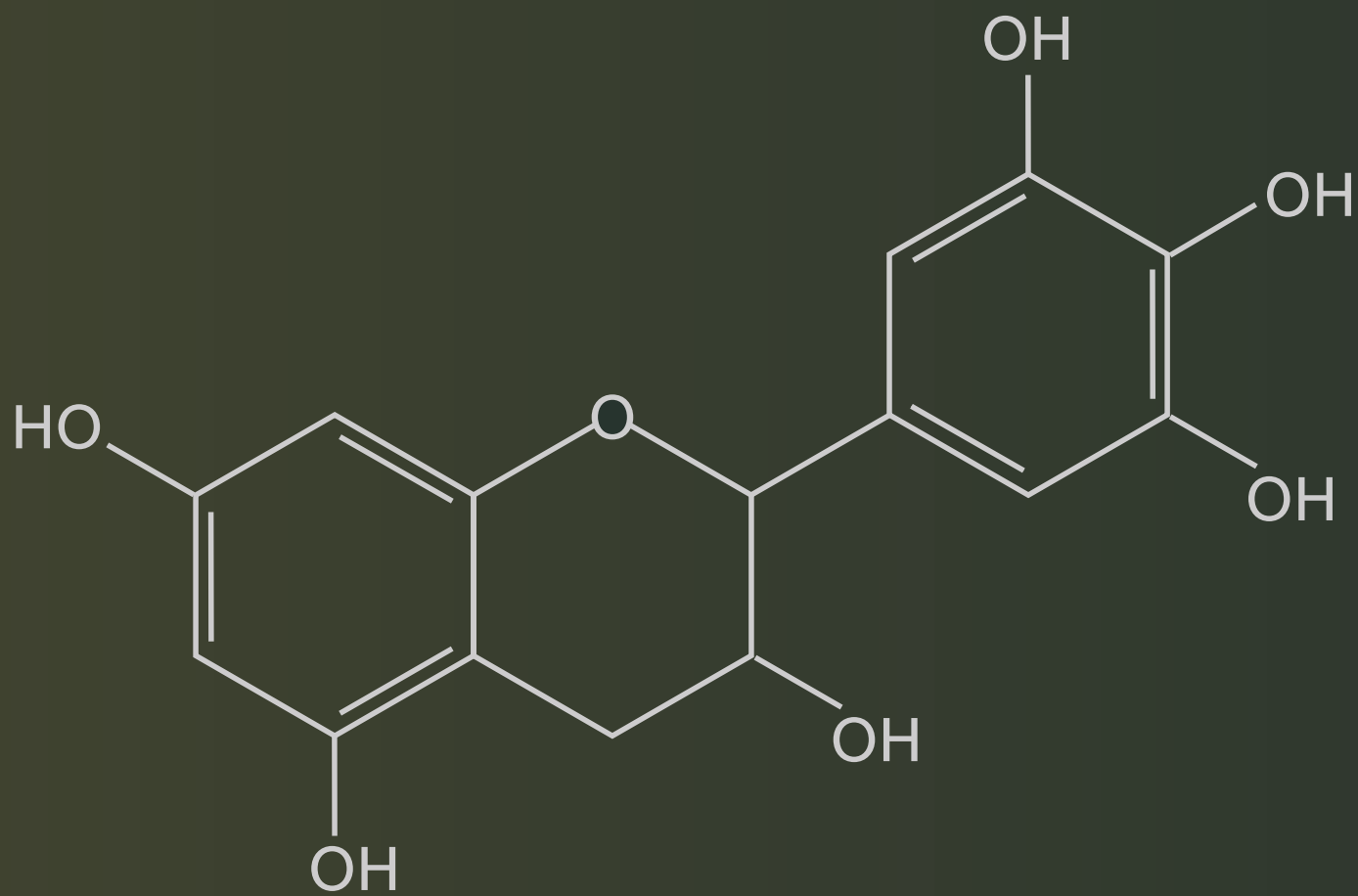
“Our local hospital immediately put us in touch with Dr Graham Davies, and we met with him at Great Ormond Street Hospital for Children NHS Trust [GOSH] and learnt about his groundbreaking thymus transplant research. It was daunting to think that this was so new and had never been performed in Europe before, but also amazing to think that if it worked, James would develop a normal immune system.

“From that point on, we have lived in a bubble. No one can hold James other than us and his grandparents, he has never played with other children, and we stay away from public places.

“In March 2009, at six months old, James had his heart surgery, and we saw his lips and toes turn from blue to pink! He then had his thymus transplant in May. Luckily, we had been able to keep him infection-free up to that point. It was an agonising wait for a suitable thymus, and for it to be cultured successfully. It has taken enormous patience and positive thinking while we have waited for the first T-cells to appear, with complications along the way. But, finally, it feels like our little boy has been born, as it looks as if the transplant is working and James’ T-cell numbers are increasing week by week.

“Hopefully it won’t be long before life can return to normal. We look forward to trips to the zoo, swimming lessons, holidays, toddler clubs and playtime with cousins, none of which would ever have been possible for James if it wasn’t for the remarkable research work that GOSH carries out.

“James is a bright, strong and affectionate little boy, full of fun and loved by many.”



Dr Arturo Sala
“My fascination with science started early, and as a kid I was intrigued by the natural world and the oceans. So, when it was time to go to university, I chose biological sciences with the intention of becoming a marine biologist. However, in the first year I changed my mind and became more interested in how cells become transformed, leading to cancer. These were very exciting times: the first ‘oncogenes’, or genes causing cancer, were being discovered, paving the way for the new emerging field of molecular oncology. Since then, I have worked in oncology and have focused my studies on neuroblastoma, an enigmatic form of childhood cancer.

“There is still no definite cure for metastatic neuroblastoma, and at the UCL Institute of Child Health, we are well placed in the race to find a treatment for this devastating tumour. In the past few years, we have been able to elucidate some important molecular pathways deregulated in neuroblastoma, and we hope to see a day in which no more children will die of this disease.”

Green tea contains molecules known as polyphenols, which are characterised by their many phenol rings. One member of this molecular family, catechins, forms the basis of Dr Sala’s research into new treatments for neuroblastoma.

Combating cancer with catechins
Dr Arturo Sala is making use of a group of compounds called catechins, commonly found in green tea, to treat one of the most deadly forms of childhood cancer. The application is new, but the use of green tea as a remedy is thousands of years old.

Despite having been known to medicine for more than 100 years, neuroblastoma – a type of cancer affecting nerve cells – remains lethal in a majority of cases. In its severest and, tragically, most common form, even intensive treatment is successful less than 50 per cent of the time. Due to the aggressive nature of the chemotherapy required, surviving children may face problems with cognition and impaired development, and, therefore, are at risk of social disadvantage as they grow into adulthood.

“Clearly we are missing something,” says Dr Sala, whose work at the UCL Institute of Child Health focuses on investigating new therapies for neuroblastoma. “We need to understand the molecular basis of how this cancer is so resistant to existing treatments, as well as ways to combat it without recourse to toxic chemotherapy drugs that carry such devastating side effects.”

As part of a number of ongoing studies to find medicines that re-activate the body’s natural immune defences against cancer, Dr Sala has begun to investigate the effectiveness of catechins. These are part of a family of chemicals called polyphenols and are derived from an extract of green tea.

“There are any number of stories of green tea being good for you,” Dr Sala says. “But we have observed a significant reduction in tumour growth in mice with neuroblastoma when we provided them with drinking water containing catechins. In addition, we are gathering evidence to demonstrate how this common extract of green tea could have such a remarkable effect.”

Early indications are that catechins might act to stop the neuroblastoma producing a type of cell, known as myeloid derived suppressor cells, which prevents the immune system from attacking tumours. Should initial experiments prove successful, Dr Sala hopes his efforts will have unlocked new non-toxic methods to boost the body’s innate defences against neuroblastoma.

By partnering with clinicians at the hospital, Dr Sala aims to apply any new treatments initially to children who relapse. There is an urgent need to find strategies to help prevent neuroblastoma recurring fatally in children who have already undergone an initial round of intensive chemotherapy, often several years earlier. Novel uses of ancient therapies such as green tea could provide new hope in the treatment of a disease, which has arguably evaded modern medicine for far too long.



Dr Detlef Böckenhauer

“As a clinician-scientist, I am in the privileged position of being able to combine a challenging and rewarding clinical practice with my research interest of understanding the basis of my patients’ diseases.

“I became interested in paediatric nephrology early on in medical school, as I was fascinated by the fact that simple biochemical analysis of blood and urine can enable a very detailed understanding of the exact nature of a patient’s problem. Yet kidney disease at its worst is kidney failure, which has a devastating impact on quality of life, not only for the patient but for the entire family. Unlike in adults, where it is typically caused by a multitude of factors, the majority of children with kidney failure were born with it, suggesting a genetic basis.

“The decoding of the human genome and advances in the technologies of DNA analysis have dramatically accelerated the discovery of the genetic basis of disease. This is what our group specialises in, as we hope that a better understanding of genetics will enable us to develop novel treatments. I have this dream of a brave new medicine, where we will be able to rapidly pinpoint the precise molecular problem in individual patients, and on this basis, provide personalised and specific counselling and treatment.”

How rare disorders can unlock the pathway to precise diagnoses

Finding new cures for disease is not the only significant step in helping children at Great Ormond Street Hospital for Children NHS Trust (GOSH). Dr Detlef Böckenhauer’s work in the Renal Unit demonstrates that it is often crucial for us to understand disease on a molecular level if we are to diagnose and treat patients according to their unique medical needs.

Dr Böckenhauer is clear on his mission as a researcher. “My ambition is to find and define the precise molecular pathways which are broken in children with kidney disease,” he says. “So often we try to alleviate the symptoms of a disease without really understanding its cause. In too many cases, the treatment we offer can have wildly different effects, despite children presenting with similar symptoms.”

Where the root cause of kidney disease is unknown, exposure to these treatments is not a trivial matter. Knowing whether or not a certain medical approach will work from the outset can spare children from having to undergo biopsies, or prevent them from having to take immunosuppressant drugs that compromise their ability to fight disease. More importantly, however, finding the biological cause of a disease can be a major step in obtaining information to guide better treatments, as well as giving profound insights into other, seemingly unrelated disorders.

Dr Böckenhauer’s most recent success has been identifying the genetic basis of a rare form of kidney disease that prevents children from retaining vital mineral salts.

“We’re all ultimately descended from sea-dwelling fish,” Dr Böckenhauer explains. “We can think of the kidneys as having evolved to keep some of that sea inside us, in the form of dissolved salts such as sodium and potassium. Without the kidneys maintaining this stable internal mixture of salts, our brain, nerves and muscles could not function: our hearts would not beat, our minds could not think. We have several unique specialist renal clinics here at GOSH, one of them with Dr William van’t Hoff, dedicated particularly to disorders of salt handling. This allowed us to identify a group of children who couldn’t retain

specific salts, and in addition were suffering from epilepsy, ataxia [an inability to co-ordinate muscle movements] and deafness.”

Working with Professor Robert Kleta at UCL and colleagues in Leeds and Germany, Dr Böckenhauer used linkage analysis, in which the DNA of affected family members is compared with that of healthy individuals, to identify the inherited genetic fault. If successful, this approach can pinpoint the region of DNA that harbours the faulty gene. They found a candidate gene active in kidney, brain and inner ear cells which, when it was mutated in animal models, mimicked the symptoms of the children with salt-handling deficiencies seen in the clinic. The team named the resultant condition EAST syndrome (epilepsy, ataxia, sensorineural deafness and tubulopathy).

“Thanks to the combined research of a number of experts and clinical specialists, we could link all of the symptoms of EAST syndrome to a single genetic defect,” Dr Böckenhauer says. “By doing so, we can now finally provide patients like Ben, and their families, with a diagnosis and explanation, which is a huge relief for them as they have often worried for many years about the nature of the disorder and what the future may hold. We can also collect clinical information, which suggests that EAST syndrome is not progressive: a huge reassurance to the families.

“Further to this, we also have a viable animal model which we can use to screen potential therapies, and which gives us an insight into new routes to treat each of the component symptoms of the disease. Sharing our knowledge means we can really make progress in treating the actual disorders, as opposed to just battling to lessen the symptoms.”



Ben’s story

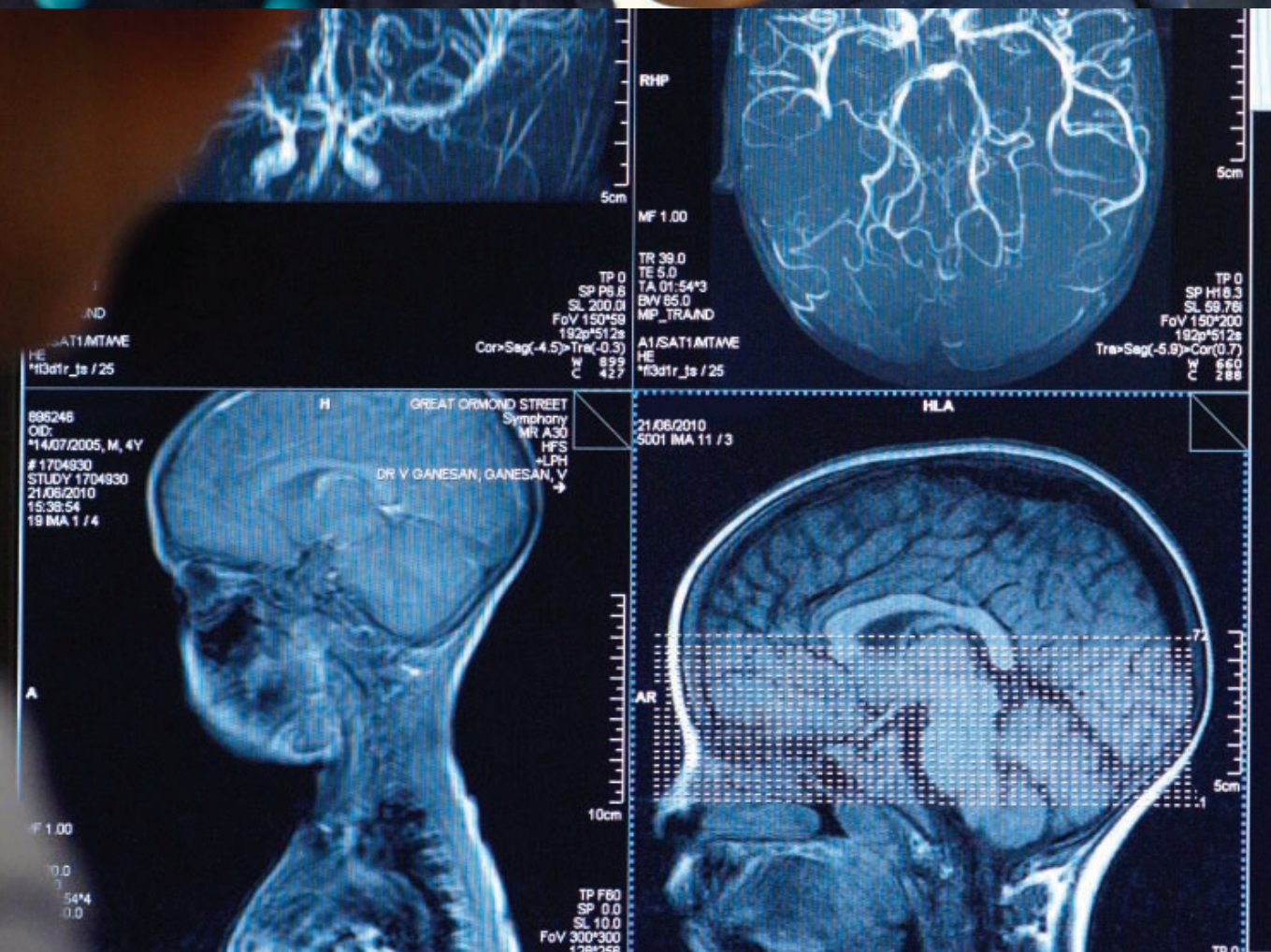
by his parents, Pat and Tony

“We adopted our son Ben when he was 15 months old, knowing that he was having nocturnal seizures, and he was diagnosed with epilepsy at 18 months old. Ben went on to fail some of his milestones, and at two years of age, was diagnosed as having severe hearing loss which was delaying his speech, and severe ataxia which was delaying his gross motor functions.

“Ben was later found to have a renal tubular defect, after he became ill with vomiting and dehydration every six weeks or so. He underwent many tests at our local hospital and other specialist hospitals, but a diagnosis could not be established, although the specialist agreed that all of Ben’s symptoms were connected. Ben was eventually referred to Great Ormond Street Hospital for Children NHS Trust [GOSH], but they could not give us a diagnosis either.

“In 2009, when Ben was 20 years old and we had given up hope of a diagnosis, GOSH contacted us again. They had discovered a new syndrome and wanted to test Ben’s DNA to see if he had the defective gene. We went to GOSH again, and Ben had lots of tests and was finally diagnosed with EAST syndrome. There is no cure yet, but with continuing research, better treatment may be found to cut down on all the medication Ben has to take.

“We are very grateful to the doctors at GOSH for not giving up on Ben. It amazed us that after so many years they remembered he had the symptoms of this rare syndrome.”



Dr Chris Clark

“I’ve been working in the field of neuroimaging for about 15 years, and had the good fortune to work on a technique called diffusion MRI [magnetic resonance imaging], which has since blossomed into one of the most important methods for studying the structure and organisation of the living human brain. I originally trained in physics, and my research group at the UCL Institute of Child Health [ICH] now consists of a range of specialists, including computer scientists, statisticians, neurologists and neurosurgeons.

“I had previously worked in research groups looking at adults with multiple sclerosis, epilepsy and vascular dementia. I came to ICH four years ago to undertake neuroimaging research in children. Because the brain is rapidly changing during normal development, this research presents a number of challenges and difficulties. I have always placed the translation of new techniques into the clinical arena very highly. I really enjoy working with a wide range of colleagues at Great Ormond Street Hospital for Children NHS Trust, the scope of whose work is unparalleled in the UK. I hope that the work we have done on mapping the pathways of the brain for neurosurgical planning will become a routine clinical service and improve outcomes for children following surgical procedures.”

Jahnai is having a head MRI scan today. He is a big Spider-Man fan and has chosen to watch the film during his hour-long scan.

Tracing the living pathways of the brain

Dr Chris Clark is applying the latest techniques in magnetic resonance imaging (MRI) to document the natural history of the brain in ways as yet unexplored in a paediatric setting. Building up a picture based on the living connections of nerves could transform surgical planning and reveal new insights into the biology of the body’s least understood organ.

MRI can reveal the internal structure of the human body on an unprecedented scale. When applied to brain imaging, it provides detailed information on the shape of the various regions of tissue, and allows doctors to see how blood flows to different areas as well as helping them to locate and identify tumours and other structural abnormalities.

A somewhat less well known application of MRI is tractography. This makes use of the directional properties of the complex network of nerves running throughout the brain to reveal structures previously only visible by dissecting brain tissue itself. Dr Clark is carrying out research to explore its value when compared to conventional MRI.

“What’s exciting is that tractography allows us to make a living map based on the intricate directional structure of the nerves and other tissues criss-crossing the brain,” he says. “It also gives us a way to quantify the unique structure of each region of tissue – a kind of navigational signature – which is immensely useful when comparing different regions. Previously, the only means we had to see many of these structures in detail was by examining sections of brain tissue taken from post-mortems.”

Applications of tractography are already showing their value in clinical settings. One strength of the technique lies in its ability to visualise regions of brain tissue which are physically connected and therefore functionally linked. Making a map of these connections of nerve tissue – the ‘tracts’ that give the technique its name – provides a unique, patient-specific insight into the structure of the brain, arming surgeons with invaluable information.

“Taking cancer as an example, tractography enables us to see if a tumour is either growing within or disrupting the path of a major tract of nerves running through the brain,” Dr Clark says. “This information just hasn’t been available before. It means surgeons can plan their operations much more precisely, as they’re in a far better position to determine whether surgically removing a child’s tumour will damage key nerve pathways. Getting this right is critical – it can mean the difference between successful surgery and life-long paralysis.”

Dr Clark and his colleagues have also begun expanding this work to classify different tumour types according to their unique signature. They compared information from scans of tumours in the cerebellum – a region of the lower brain which accounts for over half of all childhood brain tumours – with tissue collected during surgery. Their findings suggested that this imaging could give accurate diagnostic information on the type of tumour in more than 80 per cent of cases. It will also reliably identify some tumours prior to surgery, which would otherwise be impossible to distinguish using alternative imaging methods.

Dr Clark hopes to build on this work to form the basis of a clinical tractography service. “The unique information we’re gathering means we can start looking at how tissues in the brain respond to treatments in a much more sensitive manner, and begin to build up a far more detailed picture of how the brain develops in healthy children,” he says. “There’s potential for us to improve treatments for children with conditions from epilepsy to cancer, and inform surgery on a number of levels – we’re starting down an entirely new way of looking at the brain.”



Professor Paul Riley

“I fell rather serendipitously into cardiovascular sciences while I was a post-doctoral fellow in Canada 12 years ago. I was inspired by the likes of Eric Olson, a US heavyweight within the field, to pursue research into how the heart is put together during development, as a means to assess what goes wrong in human congenital heart disease.

“A significant focus, here at the UCL Institute of Child Health, has been on a downstream molecule, thymosin $\beta 4$, which both underpins the formation of blood vessels in the embryonic heart and stimulates new vessel growth in adult hearts after an ‘attack’. This work was ultimately rewarded with an Outstanding Achievement Award from the European Society of Cardiology, something for which I’m indebted to my laboratory team.

“Now I’m hoping to expand into biological-based ‘drug discovery’ and develop ways to stimulate resident heart stem cells, both in children with premature heart failure and in adults following injury: an exciting foray into translational medicine.”

Close partnerships between our researchers and clinicians allow advances in the laboratory to be applied to innovative medical areas. Professor Riley is working closely with our heart transplant team to see if his discoveries can inform future treatments for heart failure.

Finding the triggers to mend a broken heart

Having received widespread acclaim for his work in finding a protein that triggers blood vessel development in the heart, Professor Paul Riley is expanding his efforts to discover new ways of combating heart disease. His hope is to pave the way for therapies that avoid the need for organ donation.

Science rarely, if ever, progresses in terms of so-called ‘paradigm shifts’, where a single discovery affects a field of research so drastically that all prior research must be re-evaluated in light of the new findings. That said, researchers’ careers can certainly be transformed as a result of a single publication – a fact well known to Professor Riley and his team, who hit the headlines recently with their study into the mechanisms of heart development.

“Our paper, published in *Nature*, described the function of a protein called thymosin $\beta 4$, which we demonstrated could stimulate the growth of new blood vessels by activating stem cells in the epicardium [the outer layer] of the developing and adult heart,” Professor Riley says. “The paper completely changed my life – suddenly I was being asked to present at conferences all over the world and carry out reviews for top-tier journals. I’m very grateful for the efforts of my own team of researchers and the help of other leading research groups at the UCL Institute of Child Health, whose input has been crucial to the success of this work.”

Professor Riley’s challenge now is to determine exactly how thymosin $\beta 4$ acts on epicardial stem cells, as well as elucidate what these cells are capable of in terms of their potential for use in new treatments for heart disease. Following the blood vessel study, Professor Riley is working on methods to determine whether thymosin $\beta 4$ can repair damaged tissue in cases where heart muscle has been killed off as a result of a heart attack.

He is also looking to further expand the number of routes available for new treatment options by discovering additional triggers that can stimulate heart tissue repair. An exciting approach lies in collaborative work with UCL Chemistry to screen hundreds of small drug molecules to find those with a potential clinical application. Professor Riley’s team has found one drug in particular which, remarkably, triggers the heart stem cells to grow into a type of cell called a myofibroblast, usually only seen in scar tissue.

“We’d never expect to see stem cells grow into this kind of cell, which is responsible for scarring and fibrosis, during development, as the embryonic tissue they are derived from simply does not scar,” Professor Riley says. “This suggests we’re targeting a pathway actively suppressed in the developing heart. If we can either modify our small molecule or find a further drug that blocks this suppressive effect, then we may establish yet another route into promoting the heart to heal itself. It’s a fantastic field to work in, as every new step we take brings us a little bit closer to finding an alternative to invasive surgery and the need for heart transplantation.”



Professor Simon Heales

“Literally thousands of chemical reactions are occurring in each of the cells in our bodies. These metabolic pathways are, for most of us, very finely tuned and enable us to function in a normal way. Unfortunately, approximately one in 500 children is born with an inherited metabolic disease. This means that such children have an enzyme deficiency that leads to a failure of critical chemical reactions.

“During my PhD, I worked on one metabolic pathway affecting the brain’s ability to produce chemicals essential for neurological development. As I worked on the project, I became increasingly aware that the better we understand these complex biochemical pathways, the greater chance we have of developing new diagnostic methods and successful treatments.

“I completed my PhD in 1988 and was totally hooked on the area of inherited metabolic disease. For the past 22 years, I have been fortunate enough to be part of a team that has discovered previously unknown inherited disorders and developed new treatments for children suffering from them. For a scientist, to see some of your biochemical knowledge translate into patient care is an amazing experience – one that many do not get. However, the strong clinical-scientific environment created here makes this a regular experience.”

Linking up the metabolic chain

As Professor of Clinical Chemistry, Simon Heales is responsible for advancing research to help children born with a broken link in the chain of metabolic reactions that sustain human life. As there are thousands of such reactions where a life-limiting error can occur, keeping an open mind is a minimum requirement when studying a field known to deliver unexpected results.

Professor Heales originally started his career as a biochemist at the UCL Institute of Child Health (ICH) in 1988. “Coming back to Great Ormond Street Hospital for Children NHS Trust [GOSH] was like coming home,” he says. “What’s really driven my work is the potential to interact with so many other specialists. I’m always surprised by whose insight will provide a clue to a new line of research.”

Specialist research is certainly critical to progress in metabolic medicine – a field covering any disease affecting one of the several thousand biochemical reactions occurring within the body. Finding the broken link in what is a vast interwoven metabolic chain can be a considerable task for clinicians when children present with a metabolic disease. Sadly, due to a relative lack of expertise, many families are referred to multiple hospitals around the UK without receiving any kind of formal diagnosis.

Professor Heales is set to build on a history of successes to improve the treatment of metabolic disease, by drawing on close links between clinicians, scientists and diagnostic laboratories at GOSH, the National Hospital for Neurology and Neurosurgery, and the ICH – now united under the banner of UCL Partners. One such success lies in his long-standing work to diagnose and treat a series of disorders that affect children’s metabolism of neurotransmitters – chemical messengers in the brain – and vitamin B6.

“Some of these children present with movement disorders and seizures, due to their illness preventing them from producing neurotransmitters such as dopamine,” Professor Heales says. “Some of them had family members

who died from these disorders, and until our efforts, there was neither a reliable diagnosis nor a cure. By screening samples of patients’ cerebrospinal fluid, we found the exact molecular pathways that weren’t functioning – the missing links in their metabolism. As a result, we have so far been able to define four entirely new metabolic disorders.”

A precise diagnosis not only reassures patients and families, but with input from other specialist units, it also paves the way for effective treatments. In the case of one of the disorders, called TH (tyrosine hydroxylase deficiency), a drug already licensed for Parkinson’s disease was able to restore dopamine production and lead to a dramatic improvement in children’s controlled movements. With UCL Partners increasingly bringing together experts with complementary skill sets, Professor Heales is understandably buoyant as he looks to the future.

“Taking the neurotransmitter dopamine as an example, a recent link has connected what was once thought to be a totally unrelated metabolic disorder affecting the lysosome [a component of the body’s cells which recycles spent proteins] and Parkinson’s disease. It now appears that patients who carry a copy of a mutation responsible for a common lysosomal disorder called Gaucher’s disease are five times more likely to develop Parkinson’s disease.

“People often wonder why we persist in trying to understand what many consider to be rare disorders. Exploring the metabolic link between Parkinson’s and Gaucher’s disease is just one of a number of unexpected applications of the research we do, in addition to developing targeted treatments that clearly benefit children with metabolic disease. You just never know what the broader benefits of your work will be in the long run.”

Jake’s story

by Shoba Vazirani and Lisa Flint

“When Jake was born in 1998, I sensed something wasn’t right. Over the next few months, he developed feeding problems and wasn’t able to put on weight as quickly as other babies. Our GP referred us to a specialist who said Jake had quadriplegic cerebral palsy – he’d never walk or talk. It was a massive blow which left us devastated.

“I set about learning everything I could about cerebral palsy and quickly became convinced that my son didn’t have it – his symptoms just didn’t fit.

“Over the next two and a half years, I tried many treatments to help Jake walk. But, by sheer chance, he was finally – and correctly – diagnosed. He got a diarrhoea bug, was rushed to hospital and took five weeks to recover. He was put through a battery of tests again, but this time the neurologist said he’d do one extra – taking a sample of Jake’s cerebral spinal fluid.

“Just a week later, the neurologist called saying the sample was positive. Jake had Aromatic L-Amino Acid Decarboxylase Deficiency [AADC], a rare genetic illness affecting the brain’s ability to produce neurotransmitters essential for everyday living.

“It was at this time we met Professor Simon Heales, the person who had uncovered the astonishing diagnosis. We learnt that only around 17 cases of AADC had been diagnosed worldwide, and that Jake was the only British child with the condition. Despite this, it was honestly like winning the lottery, because we finally knew that Jake didn’t have cerebral palsy and that he might be able to receive the proper treatment.

“Jake was three and a half then. With medication developed to treat Parkinson’s disease in adults, by the time he was five, he was walking. He’s now 12 and takes between six and nine different drugs several times each day to preserve his mobility and health. Sadly, as well as AADC, he also has autism, a heart condition, asthma and severe hay fever. But compared to where he was, it’s a small price to pay. He’s able to live as normal a life as possible for someone with such a rare disease, and every day we have with him is truly a blessing.”





Professor Carol Dezateux CBE
“My passion for paediatrics was fired as a medical student. An interest in research came later, inspired by my experiences as a junior doctor working during a winter epidemic of bronchiolitis in Hackney. But the possibility of research training only materialised once I started working at Great Ormond Street Hospital for Children NHS Trust.

“I began as a doctoral research fellow in Janet Stocks’ laboratory, measuring airway function in young babies recovering from bronchiolitis, but knew that I would need formal training in epidemiology to progress my research ideas. Catherine Peckham, newly appointed as Professor of Paediatric Epidemiology at the UCL Institute of Child Health, encouraged me to obtain a Wellcome Trust training fellowship in epidemiology.

“This broad training has helped me to develop my research at the interface of clinical, laboratory and epidemiological sciences. One of my goals as Head Director of the Medical Research Council (MRC) Centre of Epidemiology for Child Health and Chair of the MRC Training and Careers Group is to encourage young clinicians into research, especially in epidemiology.”

Delivering health benefits to children worldwide requires a broad understanding of what influences children’s health – politics as much as science. A broad reading list keeps Professor Dezateux abreast of topical issues relevant to her work.

Long-term health through life-long research
Putting advanced medical developments into practice in a way that benefits entire populations requires a particular type of translational research, often overlooked in the race to find new cures. Professor Carol Dezateux’s work aims to bridge this so-called ‘second translational gap’ with studies which aim to understand the factors underpinning a healthy life.

An early influence on Professor Dezateux’s career came from her experiences as a young paediatrician looking after babies with acute bronchiolitis – an inflammation of the airways of the lungs. Although the specific virus which most commonly causes bronchiolitis affects almost all children within their first two years of life, doctors didn’t understand why some babies with this infection developed wheezing and became severely ill, while others experienced only minor symptoms. Professor Dezateux soon realised that investigating children at the point at which they were admitted to hospital could not answer questions about the environmental and social factors contributing to their illness. She therefore embarked on an ambitious study to measure healthy babies’ airways in the first weeks of life.

“As a result of mothers being willing to place their trust in our research, we carried out a series of tests on healthy babies,” Professor Dezateux says. “We found that mothers smoking during pregnancy and early infant exposure to tobacco smoke affected airway size and growth in very early life, and increased the risk of babies developing wheezing illnesses.”

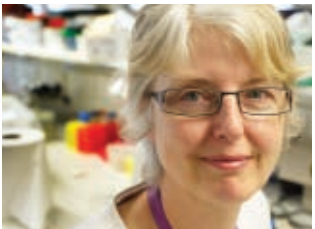
Professor Dezateux has since directed her research to investigating the early origins of disease. “My interests have always been in using biological measures obtained in early life to help understand how the environment in its broadest sense affects a child’s health and development,” she says. “The challenge is how you introduce these measures into large-scale surveys which follow healthy children from birth – so-called birth cohort studies – so that you can begin to determine the often subtle factors contributing to disease, before children become ill.”

The most recent of these UK-wide birth cohorts – the Millennium Cohort Study¹ – has enabled Professor Dezateux and colleagues at the Medical Research Council Centre of Epidemiology for Child Health to identify factors underlying the significant levels of obesity among pre-school children, from a range of ethnic backgrounds. Her latest research aims to establish the extent to which reduced physical activity contributes to obesity in young children, the incidence of which has nearly doubled over the past 25 years.

“Children are getting more overweight, at an earlier age; if we’re to help reverse the trend, we need to find out precisely why this is. Our research team has used accelerometers [devices which record the duration and intensity of motion] to measure activity in more than 7,000 children participating in the Millennium Cohort Study. This will add to and enhance information we’re capturing on children’s growth and body fat, nutrition and psychological development, to form a more complete picture of the experiences of children from diverse social, ethnic and family backgrounds.

“Extending this kind of research to the wider population is critical if we’re to make a lasting impact on issues of major public health importance for children,” Professor Dezateux stresses. “Cohort studies have the potential to address key questions about the interplay between biology and the environment during early life. They enable us to understand what influences the health and well-being of children and the adults they will eventually become. I’m confident that our work will lay a strong and unique foundation for future research and improvements in child health, which will last far longer than my lifetime.”

¹ The Millennium Cohort Study is one of three internationally renowned cohort studies managed by the Centre for Longitudinal Studies, based at the Institute of Education, University of London.



Dr Jennifer Morgan

“My interest in skeletal muscle regeneration started many years ago, when I tested the idea of using muscle precursor cells called satellite cells to repair and regenerate skeletal muscle in a model of Duchenne muscular dystrophy. I joined Professor Francesco Muntoni’s group with a Medical Research Council [MRC] joint collaborative career development award in stem cell research in 2005, and moved to the UCL Institute of Child Health when the Dubowitz Neuromuscular Centre relocated here in 2008.

“Obtaining a Wellcome Trust university award has enabled me to pursue my studies on the contribution of satellite cells to skeletal muscle regeneration, and we are also investigating the possibility of using other types of stem cell to treat muscular dystrophies. I am a co-investigator of the MRC Centre for Neuromuscular Diseases, which will help us to translate our basic science research into treatments for neuromuscular diseases.”

Winding back the biological clock on damaged muscles

By developing methods to deliver advanced cell therapy to regions of damaged muscle tissue, Dr Jennifer Morgan hopes to help children born with Duchenne muscular dystrophy. Her latest work points to a treatment that may restore healthy muscle in young and old patients alike.

Duchenne muscular dystrophy is a severe form of degenerative muscle-wasting disease, affecting one in 3,500 boys. The disorder arises owing to an inherited fault in children’s ability to produce the protein dystrophin, a crucial structural component of muscle tissue. This leads to a progressive loss of muscle fibres as children grow, causing them to become steadily weaker until voluntary muscle movement is practically impossible.

Research into treating Duchenne muscular dystrophy relies on finding ways to re-introduce functional dystrophin protein into muscles, enabling them to regenerate and preventing subsequent muscle fibre loss. Working in the Dubowitz Neuromuscular Centre, Dr Jennifer Morgan’s research focuses on understanding how to repair damaged muscle tissue via innovative cell therapies.

“Healthy muscles are in a permanent state of equilibrium, with damaged muscle fibres being repaired or replaced by muscle stem cells,” says Dr Morgan. “The problem in children with Duchenne muscular dystrophy is that their disease unbalances this equilibrium. As the progressive effects of a lack of dystrophin take hold, the process of breaking down fibres outpaces any new growth, so healthy muscle ends up being replaced by non-functioning fibrous and fatty tissue.”

Dr Morgan’s work looks at the therapeutic potential of satellite cells, a population of muscle stem cells that ordinarily lies dormant within muscle tissue. She and her team found that when they injected normal cells into models of damaged muscle tissue similar to that found in patients with Duchenne, these satellite cells repaired and replaced damaged muscle fibres and restored production of dystrophin.

“Not only did we see immature muscle tissue repaired,” adds Dr Morgan, “we also found that satellite cells were just as efficient at restoring functional muscle in old tissue. This means that any eventual therapy based on satellite cells may well be of benefit to both young children with Duchenne and older people affected by more severe loss of muscle function.”

The challenge now is to find a stem cell that clinicians can deliver around the whole body via a single injection into the bloodstream, as opposed to multiple injections directly into the muscle tissue. “Working with translational researchers across the Medical Research Council Centre for Neuromuscular Disease means that we can draw on facilities such as their rare-disease samples biobank,” says Dr Morgan. “This will be crucial as we test ways to deliver stem cells into muscle via the blood. Combined with ongoing clinical trials to restore dystrophin function in Duchenne patients via novel gene-based therapies, it means we’re really beginning to build critical mass in this area.”



Researchers such as Debbie Briggs (above), who is working with Dr Morgan, benefit from the Somers Clinical Research Facility (below), where clinical trials of new therapies can be safely tested in an environment designed to meet patients’ needs.



Dr Thomas Jacques

“There are two very exciting challenges in pathology that attracted me as a junior doctor. The first is to diagnose children with diseases of the nervous system, particularly those with brain tumours. Based almost entirely on this diagnosis, children with brain tumours can be committed to often lengthy courses of treatments, frequently with toxic drugs. My holy grail would be to be able to say precisely which children will respond to a particular treatment and which will not: a fundamental but difficult question. This is a very exciting time, as we are harnessing new molecular technologies to refine our diagnoses and get closer to the goal of directing treatments to specific patients.

“The second opportunity that pathology offered me was to understand diseases of the brain by studying tissue from patients. This is leading us to very exciting insights into the mechanisms of epilepsy in childhood. These studies will help us to understand how the diseases arise and, ultimately, to improve treatment.”

An archive of tissue samples enables our researchers to make connections between the unique disease profiles of each patient, forming a platform for future discoveries, such as Dr Jacques’ work linking cancer and epilepsy.

Malfunctioning stem cells seeding cancer and epilepsy

Since their discovery, stem cells have received a lot of attention as potential therapeutic agents to cure disease. Dr Thomas Jacques’ research investigates how their growth, when misdirected, can instead be the cause of disease – an area he hopes will provide therapeutic answers to a number of urgent clinical questions.

Brain tumours are the second most common form of cancer in childhood. Yet there remain few answers to the fundamental questions of why different brain tumours arise and what biological mechanisms trigger their growth.

Histopathology – testing and observing samples of tissue under a microscope for markers of disease – plays a key role in defining how severe a child’s tumour is, and therefore, how much aggressive chemotherapy is needed to fight it. Dr Jacques is keen to apply more advanced histopathological methods to diagnose tumours more precisely according to their specific disease profile, and tailor treatments accordingly.

“Children with brain tumours present with little in the way of medical history to give an indication of what has caused the cancer,” Dr Jacques says. “Which means it has been very hard for us to try to work backwards to determine why these tumours occur. My research focuses on the very early stages of tumour growth – an area where we’re now starting to gain some fascinating insights.”

Historically, one of the major problems faced by researchers studying tumours has been to determine if any cell can become cancerous, or whether there is a population of specific precursor cells where the wrong genetic trigger induces uncontrolled malignant growth. To help resolve this issue, Dr Jacques’ team and colleagues at the Institute of Neurology investigated an area of the brain containing populations of stem cells. They found that a discrete series of genetic mutations in a specific type of stem cell triggered clearly defined types of tumour. When they induced the same genetic mutation in more mature brain cells, there was no tumour growth.

The results suggest that there are a limited number of stem cells able to cause cancer in the mature brain. Capitalising on this, with support from Great Ormond Street Hospital Children’s Charity, Dr Jacques is undertaking further research to find biological markers which will pinpoint different subtypes of such tumour-causing stem cells. And he’s hopeful that results will stimulate better clinical treatments. “The project we’re now working on aims to accurately detect the presence of these tumour stem cells, and find the link between the type of cell and the eventual severity of the child’s cancer. If successful, it means we’ll be a step closer to offering children with brain tumours a personalised treatment based on a detailed biological understanding of their disease.”

These errant cells also seem to play a part in other, non-malignant diseases. Dr Jacques and his team have preliminary evidence to indicate that a different series of abnormal stem cells might play a role in forming brain defects known to cause a highly debilitating type of childhood epilepsy – focal cortical dysplasia. “Given the lack of reliable disease models for studying such severe forms of epilepsy, our work could really help to drive advances in this field,” he adds.



Dr Nick Greene
“My research has focused on the early development of the central nervous system, beginning in 1993 when I came to the UCL Institute of Child Health to carry out PhD studies with Professor Andrew Copp. With the exception of a period of post-doctoral training elsewhere, we have worked together ever since, and it has been a hugely enjoyable and productive collaboration.

“The challenge of my work is to understand what might seem quite a simple process: how does a sheet of cells turn into a tube which will then become the brain and spinal cord? In fact, this is a remarkably complex, though elegant process, the failure of which leads to serious birth defects.

“It’s tremendously exciting that we have now reached a point where we can apply the findings of our laboratory research to possible clinical uses. Speaking to patients taking part in our clinical trial is a great motivation to find out how and why these disorders happen.”

Research can provide robust evidence for innovative medical uses of even common substances. Regulatory frameworks must recognise the potential of expert studies to inform both healthcare and consumer choice, and must facilitate those, such as the inositol trial, which seek to address urgent clinical issues.

Dietary supplements to prevent neural tube defects
Inositol, a common vitamin related to glucose, could be key to reducing the incidence of babies born with neural tube defects such as spina bifida. Following four years of painstaking preparatory work, Dr Nick Greene and colleagues have launched the UK’s first clinical trial to test its potential use.

Neural tube defects constitute the second most common birth defect after congenital heart disease, affecting one in 1,000 newborns. Such defects arise when the neural tube – the developing structure which will become the brain and spinal cord – is not completely formed in the early embryo. Dr Greene has spent his research career looking for the biological causes of these defects, and may be close to a landmark discovery to help further reduce their occurrence.

The risk of neural tube defects is reduced by up to two-thirds if women take folic acid supplements during pregnancy. “However, we’re still a long way off knowing why such defects still occur,” Dr Greene says. “The problem has been that we understood neither the genetics behind the disease, nor how to prevent neural tube defects in those cases that don’t respond to folic acid. Our goal is to use laboratory models to build up a picture of the genes and proteins that are needed to build the neural tube, and to understand how they act together to produce the mechanical force needed for its closure.”

These laboratory models also provide a means to test possible preventive methods, and in one such model, Dr Greene and his team discovered that a naturally-occurring carbohydrate called inositol could prevent disease, even in the absence of dietary folic acid.

“Having shown that inositol was a candidate for preventing neural tube defects, in 2005 we received a grant from the Medical Research Council to begin clinical trials in mothers at risk of having a baby with the disease,” Dr Greene says. “It then took us four years to get to the point where we could start recruiting patients to take part in the trial. The reason is that, although inositol is available as a health food supplement, we’ve had to ensure that the product we give to parents meets strict medical manufacturing requirements.”

Dr Greene and his team have now gained the necessary regulatory approvals. Despite the delay, they are now in a far better position to roll out the Prevention of Neural Tube Defects with Inositol (PONTI) trial to other centres – initially in the UK and then, ultimately, internationally. Should it prove successful, the PONTI trial will be the first of its kind since the early 1990s to demonstrate the effectiveness of a nutritional supplement as a means to prevent birth defects.

While the trial is ongoing, Dr Greene is continuing work to unravel the genetic and cellular causes of neural tube defects. “We’re confident that we’re making progress in finding the precise genetic pathways that malfunction in neural tube defects and how these relate to folic acid metabolism,” he says. “If we can pinpoint the genes that increase a child’s risk of neural tube defects at the same time as the PONTI trial, it would give us a new route into preventing the disease. Then we’re in a real position to make a difference.”

Our unique breadth of
specialist staff nurture
new ideas, applying them
to urgent medical problems,
to transform children's lives.

Biomedical Scientist Oliver
Campos carries out research on
the array of biological tissues in
Dr Thomas Jacques' laboratory at
the UCL Institute of Child Health.

Awards, honours and prizes 2009

Staf from the UCL Institute of Child Health and Great Ormond Street Hospital for Children NHS Trust received national and international recognition for their research achievements during 2009.

Dr Anil Abeywickreme was awarded a PhD for his thesis, *The effect of BMP4 on Haematopoietic Stem Cells*.

Dr Melanie Adams was awarded a PhD for her thesis, *The management of feeding difficulties in children with cerebral palsy in Bangladesh*.

Dr Topun Austin was awarded a PhD for his thesis, *Assessment of Cerebral Oxygenation in The Newborn Infant Using Optical Techniques*.

Dr Sean Barry was awarded a PhD for his thesis, *Investigating the role of JAK/STAT and MAPK Pathways in Ischaemia/Reperfusion Injury and Inflammation*.

Dr Suzanne Bartington was awarded a PhD for her thesis, *An epidemiological Assessment of Viral Infections in Pre-school Aged Children Using Biomarkers in Oral Fluid*.

Dr Kate Bennett was awarded a PhD for her thesis, *The use of proteomics and mass spectrometry to investigate the interactions between proteases and protease inhibitors in the skin barrier*.

Dr Richard Chin was chosen as the winner of the Young Investigator of the Year Medal for 2009. The award is generously funded by SPARKS (Sport Aiding Medical Research for Kids) and is given annually for excellence in research to an outstanding medically-qualified research worker in British paediatrics.

Dr Louise Elizabeth Coats was awarded a PhD for her thesis, *Physiological adaptation to acute relief of adverse right ventricular loading conditions*.

Professor Anthony Costello was made a Fellow of the Academy of Medical Sciences.

Professor Helen Cross was appointed as clinical adviser to the update of the National Institute of Clinical Excellence guidelines for the diagnosis and management of the epilepsies in primary and secondary care. Professor Cross was also appointed Chair of the Task Force for the International League Against Epilepsy (ILAE)/International Bureau for Epilepsy and the World Health Organization Global Campaign against Epilepsy; Chair of the Task Force of ILAE for Paediatric Epilepsy Survey (Commission for Paediatrics), and Chair of the Education Sub-Committee of the ILAE Commission of European Affairs.

Dr Gloria Dimco was awarded a PhD for her thesis, *A novel role for STAT1 in Cell Cycle regulation*.

Dr Robert Dinwiddie received an award for life-time achievements from the British Paediatric Respiratory Society.

Dr Agnes Dzwonek was awarded an MPhil for her thesis, *The role of Mannose-binding Lectin (MBL) in paediatric infection*.

Miss Lindsey Edwards was awarded a PhD for her thesis, *Crohn's Disease-in vitro and ex vivo innate epithelial responses to bacterial stimulation*.

Dr Christina Georgoula was awarded a PhD for her thesis, *Molecular genetic analysis of infantile hypertrophic pyloric stenosis*.

Professor Christopher Gillberg was awarded the King's Medal (Sweden) for distinguished service to child psychiatry and the award of the most distinguished scholar in the University of Gothenburg.

Professor David Goldblatt, Director of Clinical Research and Development for the joint Institution and Director of its affiliated National Institute for Health Research Specialist Biomedical Research Centre, was appointed as the programme manager for Child Health.

Dr Rachael Gregson received the SPARKS Excellence in Medical Research award for quantifying physiotherapy techniques in ventilated children.

Dr Mike Grocott was awarded an MD for his thesis, *Measuring morbidity following major surgery*.

Dr Esther Hamblion was awarded a PhD for her thesis, *The Epidemiology of Hereditary Retinal Disorders in the United Kingdom*.

Professor David Hatch received an Honorary Fellowship of the Royal College of Paediatrics and Child Health.

Mrs Summer Hawkins was awarded a PhD for her thesis, *Obesity in pre-school children: an analysis of policy-relevant risk factors*.

Dr Daljit Hothi was awarded an MD (Res) for his thesis, *An investigation into the mechanisms, consequences and moderators of intradialytic hypotension in paediatric haemodialysis*.

Dr Rosalie Hughes was awarded a PhD for her thesis, *An investigation into the mechanisms by which bim gene expression is regulated in sympathetic neurons*.

Mr Ioannis Kokkinopoulos was awarded a PhD for his thesis, *Characterisation of neural Progenitors from the Adult Retina and Ciliary Epithelium*.

Dr Albert Kwok was awarded a PhD for his thesis, *A Comparative study of cationic formulations for the delivery of siRNA and DNA*.

Professor David Latchman was appointed by the Mayor of London as a member of the Promote London Council. He was also appointed a member of the London Steering Committee of the European Regional Development Fund and the European Social Fund.

Dr Joy Lawn was awarded a PhD for her thesis, *4 Million Neonatal Deaths: An analysis of available cause of death data and systematic country estimates with a focus on birth asphyxia*.

Dr Kingyin Lee was awarded a PhD for his thesis, *FGF and TGFbeta signalling in craniosynostotic osteoblasts*.

Dr Charles Li was awarded a PhD for his thesis, *The role of MHC Class I in Inflammatory Muscle Diseases*.

Dr Halima Moncrieffe won a prize at the Young Investigators Meeting of the 2009 Paediatric Rheumatology European Society meeting for her oral talk, *Generation of novel pharmacogenomic candidates in the response to MTX in juvenile idiopathic arthritis (JIA): correlation between gene expression and genotype*.

Dr Joanna Morrison was awarded a PhD for her thesis, *Understanding the effect of a participatory intervention with women's groups to improve maternal and neonatal health in rural Nepal*.

Awards, honours and prizes 2009 continued

Miss Marta Munoz-Alegre was awarded an MPhil for her thesis, *Development of a novel system to induce double-strand breaks in mammalian cells*.

Professor Brian Neville was awarded an Excellence in Epilepsy award by the UK Chapter of the International League Against Epilepsy.

Dr Shao J Ong was awarded a PhD for his thesis, *Epithelial innate defence to Neisseria meningitidis*.

Dr Soo Park was awarded a PhD for his thesis, *An investigation of genetic risk factors in primary open-angle glaucoma*.

Dr Mark Peters was awarded a Higher Education Funding Council for England Senior Lecturership.

Miss Shawnelle Ponde was awarded a PhD for her thesis, *Programming and the Genetic Variability of Body Composition: A Twin Study*.

Ms Ammani Prasad received a Fellowship of the Chartered Society of Physiotherapy.

Dr Joel Rae was awarded a PhD for his thesis, *Analysis of the requirement for the Chx10 homeobox gene and FGF signalling for normal retinal development*.

Dr Ramya Ramanujachar was awarded a PhD for her thesis, *The Molecular Basis of E2A-HLF Induced Pre-B Acute Lymphoblastic Leukaemia in Childhood*.

Miss Victoria Randall was awarded a PhD for her thesis, *Investigation into the phenotypic and gentic overlap between CHARGE and DiGeorge syndromes during development of the pharyngeal apparatus*.

Dr Clare Rees was awarded a MD for her thesis, *Surgical Management and Epidemiology of Necrotizing Enterocolitis*.

Miss Kate Sadler was awarded a PhD for her thesis, *Community-based therapeutic care: treating severe acute malnutrition in Sub-Saharan Africa*.

Mr Hemant Sahni was awarded UCL's John and Elizabeth Sherris Best Graduate Student Award for 2008/09.

Dr Emma Sala Soriano was awarded a PhD for her thesis, *Interactions Between Human Dendritic Cells and T Cells Following Adenoviral Infection in Health and Autoimmune Disease*.

Dr Samira Salek-Ardakani was awarded a PhD for her thesis, *Investigating the molecular basis of AMKL and MDS*.

Dr Sujith Samarasinghe was awarded a PhD for his thesis, *Selective allodepletion to improve anti-viral and anti-leukaemic responses after haploidentical transplantation*.

Professor Neil Sebire, with co-author Professor Harold Fox (University of Manchester), won two awards for the textbook *Pathology of the Placenta*, which was commended at the British Medical Association Medical Book Awards 2008 and highly commended at the Royal Society of Medicine/Society of Authors Awards. Professor Sebire was also awarded the prestigious Walter GJ Putschar Lectureship at the Massachusetts General Hospital and Harvard Medical School. Professor Sebire gave his first lecture on *Gestational trophoblastic disease: diagnostic difficulties and current issues*, and the other on *Controversies and developments in perinatal and placental pathology; old problems, new approaches*.

Dr Shahla Shah was awarded an MD(Res) for her thesis, *The epidemiology of anophthalmos, microphthalmos and coloboma in the United Kingdom*.

Dr Rukshana Shroff was awarded a PhD for her thesis, *Uraemic vascular damage and calcification in children with chronic kidney disease*.

Professor David Skuse was invited to the Gordon Research Conference on Genes and Behaviour in Ventura, California. Professor Skuse gave a talk on *Genetic influences on development and functioning of neural systems associated with social cognition in humans*.

Dr Samatha Sonnappa was awarded a PhD for her thesis, *Ventilation Inhomogeneity as an Indicator of Airways Disease in Preschool Children with Wheeze*.

Dr Helen Spencer was awarded an MD for her thesis, *Assessment and feasibility of haematopoietic stem cells to differentiate in to lung epithelial cells in the adult and growing airway*.

Dr Sanja Stanojevic was awarded a PhD for her thesis, *Development and use of international reference centiles for lung function in early childhood*. Dr Stanojevic also received a special 2009 Klosterfrau award for her research contributions to childhood asthma.

Professor Janet Stocks received the SPARKS (Sport Aiding Medical Research for Kids) Excellence in Medical Research award for quantifying physiotherapy techniques in ventilated children.

Dr Maria Theodorou was awarded a PhD for her thesis, *Nystagmus and visual acuity*.

Dr Claire Townsend was awarded a PhD for her thesis, *Antiretroviral therapy and pregnancy outcome in HIV-infected women in the United Kingdom*.

Dr Don Urquhart was awarded an MD for his thesis, *Exploration of the relationship of hypoxia and measures of clinical status and inflammation in children with Cystic Fibrosis*.

Dr Ajana Vaidya was awarded a PhD for her thesis, *Size at birth: an examination of meaning and usefulness. A prospective study of a cohort of infants born in Nepal*.

Miss Nisha Vastani was awarded a PhD for her thesis, *Mechanisms of thermal sensitivity in rodent primary afferent neurons innervating the skin*.

Dr Conrad Vink was awarded a PhD for his thesis, *A Lentivirus-Transposon vector for Safer Gene Therapy*.

Dr Vanessa Walf-Vorderwuelbecke was awarded a PhD for her thesis, *A model to investigate the oncogenic activity of MLL-fusions in Acute Myeloid Leukaemia*.

Dr Suellen Walker received a Special Interest Group in Paediatric Pain Young Investigator award from the International Association for the Study of Pain.

Dr Gillian Watterson was awarded an MD for her thesis, *The Mechanism and Efficacy of Peripheral Opioids in Paediatric Inflammatory Pain*.

Dr Yin Wu was awarded a PhD for his thesis, *A study of non-classical immune intereactions with influenza A virus*.

Miss Yan Yang was awarded a PhD for her thesis, *Characterisation of Parkinson's Disease-Associated Genes and their Regulation*.

Grants and donations 2009

The UCL Institute of Child Health continues to receive grants from the following organisations:

A

Abbott Laboratories
Abbott Nutrition
Academy of Medical Sciences
Actelion Pharmaceuticals
Action Duchenne
Action Medical Research
Age UK
Anatomical Society of Great Britain and Ireland
Annabel McEnery Childrens’ Cancer Fund
Antisoma Research Limited
Arthritis Research Campaign
Arthritis Research UK
Association Française contre les Myopathies (AFM)
Association for International Cancer Research
Association Monégasque Contre les Myopathies
Asthma UK
AstraZeneca Pharmaceuticals
Autism Speaks
AVI BioPharma

B

Baily Thomas Charitable Fund
Bart’s Charitable Trust
Baxter Healthcare Corporation
Bayer Schering Pharma AG
Beaufour Ipsen Pharma
BIAL
Big Lottery Fund
Bill & Melinda Gates Foundation
BioMarin Pharmaceuticals
Biotechnology and Biological Sciences Research Council
Bliss
Bone Cancer Research Trust
Bone Marrow Research Trust
British Academy of Childhood Disability
British Council
British Educational Communications and Technology Agency
British Eye Research Foundation
British Heart Foundation

British Journal of Anaesthesia

British Lung Foundation
British Skin Foundation
BUPA Foundation

C

Cambridge University Hospitals NHS Foundation Trust
Cancer Research UK
Cardiothoracic Unit, Great Ormond Street Hospital for Children NHS Trust
Centocor
Child Growth Foundation
Child Health Research Appeal Trust
CHILDREN with LEUKAEMIA
Children’s Brain Diseases Foundation (USA)
Children’s Cancer and Leukaemia Group
Children’s Hyperinsulinism Fund
Children’s Liver Disease Foundation
Children’s Research Fund
Children’s Trust
Chronic Granulomatous Disorder Research Trust
CLEFT
CLIC Sargent
Colgate-Palmolive (UK) Limited
Coronary Artery Disease Research Association (CORDA)
CP Charitable Trust
CREA
Cyberonics Europe
Cystic Fibrosis Trust
Cystinosis Foundation
Cystinosis Research Network

D

Department for Children, Schools and Families
Department for Innovation, Universities and Skills
Department for International Development
Department of Health
Diabetes UK
Dimbleby Cancer Care Research Fund
Duchenne Parent Project
Dystrophic Epidermolysis Bullosa Research Association (DebRA)

E

EC Marie Curie
Economic and Social Research Council
Edward Jenner Institute
EISAI Ltd
Eli Lilly and Company Limited
Elimination of Leukaemia Fund
Emergency Nutrition Network (ENN)
Engineering and Physical Sciences Research Council
Epilepsy Research Foundation
Epilepsy Research UK
European Commission
European Respiratory Society
European Society for Immunodeficiencies
European Union

F

Fight for Sight
Mr and Mrs Fitzpatrick
Food and Agriculture Organization of the United Nations
Fondation Leducq
Fondation Genevoise de Bienfaisance
Valeria Rossi di Montelera

G

Gavriel Meir Trust
Généthon
Genex Biosystems
Genzyme Corporation
Genzyme Europe
Gilead Sciences
GlaxoSmithKline Biologicals
Great Ormond Street Hospital
Children’s Charity
Guide Dogs for the Blind Association

H

Hammersmith Hospital NHS Trust
Health Foundation
Health Protection Agency
Health Technology Assessment
Heart Research
HJ Heinz Company Limited
Hestia Foundation
Higher Education Funding Council for England
Histiocytosis Research Trust
Hospital for Sick Children
HSA Charitable Trust
Human Early Learning Partnership

I

Ichthyosis Support Group
Institute of Education
International Association for the Study of Pain
International Centre for Child Studies
Ipsen Ltd
Isis Pharmaceuticals

J

Janssen-Cilag Ltd
Jeans for Genes
Jennifer Trust for Spinal Muscular Atrophy
Johns Hopkins University, USA
Joint Information Services Committee

K

Kay Kendall Leukaemia Fund
Kidney Research Aid Foundation
Kidney Research UK
Kids Company
Kids Kidney Research

Grants and donations 2009
continued

L

Leukaemia and Lymphoma Research

M

Macula Vision Research Foundation
Macular Disease Society
Mary Kitzinger Trust
Mason Medical Research Foundation
Medical Research Council
Medical Research Council of Canada
Medtronic
MEND Central Ltd
Meningitis Research Foundation
Meningitis Trust
Merck & Co Inc
Merck Sharp Dohme
Moulton Charitable Trust
MSS Research Foundation
Mundipharma Research Limited
Muscular Dystrophy Association
Muscular Dystrophy Campaign
Myositis Support Group

N

National Alliance for Autism Research
National Institute for Health
Research (NIHR)
National Institute of Mental Health (USA)
National Institutes of Health
National Kidney Research Fund
National Patient Safety Agency
National Screening Committee
Neuroblastoma Society
Newlife Foundation for Disabled Children
NHS Blood and Transplant
NIHR Biomedical Research Centre
North Bristol NHS Trust
North Thames Cleft Regional Service
Novartis
Novartis Pharmaceuticals
Novo Nordisk
Novo Nordisk Pharmaceuticals
Nutricia Ltd

O

Octapharma AG
Olivia Hodson Cancer Fund
Organon Laboratories Ltd
Orphan Europe (UK) Ltd

P

Paediatric Rheumatology
Discretionary Fund
Philips Avent
Philips Electronics (UK) plc
Physiotherapy Research Foundation
Primary Immunodeficiency Association
PTC Therapeutics
Public Health Laboratory Service
Communicable Disease
Surveillance Centre

Q

Quintiles (UK) Ltd

R

Rank Bequest
Research Autism
Research into Ageing (formerly British
Foundation for Age Research)
Research into Childhood Cancer
Rho Incorporation
Roche Products
Ronald McDonald House Charities
Royal Academy of Engineering
Royal College of Paediatrics and
Child Health
Royal College of Surgeons of England
Royal National Institute for Deaf People

S

Samantha Dickson Brian Tumour Trust
Sanofi-aventis
Sanofi Pasteur
Santhera Pharmaceuticals
Save the Children UK
Saving Newborn Lives (Save the
Children USA)
Scope International AG
SENSE
Sheffield University
Shire Human Genetic Therapies
UK Limited

SHS International
Siemens
Siemens Medical Solutions
Sir Jules Thorn Charitable Trust
Skeletal Dyplasia Group for Teaching
and Research
SMA Europe
Society for Pediatric Radiology
SPARKS (Sport Aiding Medical Research
for Kids)
Stanford University, California, USA
Stanley Thomas Johnson Foundation

T

Takeda Europe Research and
Development Centre Ltd
Tavistock and Portman NHS
Foundation Trust
Teenage Cancer Trust
The Royal Society

U

UBS Optimus Foundation
University College London
Hospitals Comprehensive Biomedical
Research Centre
UK Newborn Screening Programme
Ulverscroft Foundation
UNICEF
United Nations High Commissioner
for Refugees
UCL
University College London Hospitals
Foundation NHS Trust
University of Hamburg, Germany
University of Iowa, USA
University of London Central Research Fund
University of Manchester, UK
University of Oxford, UK
University of Sheffield, UK

V

Vitaflo Limited
Vitol Charity Fund

W

Walter Swindon Charitable Trust
Warburg Settlement
Wellbeing
Wellbeing of Women
WellChild
Wellcome Trust
Welton Foundation
World Health Organization
Wyeth Laboratories

Senior academic staff 2009

Biochemical and nutritional sciences theme

Theme Leader
Professor Alan Lucas

Nutrition Unit
Medical Research Council Professor of Paediatric Nutrition and Head of Unit
Professor Alan Lucas MA MB BChir FRCP MD FRCPCH FmedSci
Professor of Biochemistry
Professor David Muller BSc PhD
Professor in Childhood Nutrition
Professor Atul Singhal MB BS DCH MRCP MD
Emeritus Professor
Professor Brian Wharton MD MBA DSc FRCP(L)(E)(G) FRCPCH DCH
Reader in Paediatric Nutrition
Dr Jonathan Wells MA MPhil PhD
Reader in Childhood Nutrition
Dr Mary Fewtrell MD BMBCh FRCPCH MRCP DCH MA
Senior Lecturer
Dr Margaret Lawson MSc PhD SRD

Surgery Unit
Nuffield Professor of Paediatric Surgery and Head of Unit
Professor Agostino Pierro MD FRCS (Eng) FRCS (Ed)
Reader in Paediatric Gastroenterology
Dr Keith Lindley BSc PhD MRCP(UK) MRCPCH
Senior Lecturer
Dr Simon Eaton BSc PhD
Honorary Senior Lecturers
Mr David Albert FRCS
Mr Peter Ayliffe FRCS (Eng) FRCS (Maxfac) FDS RCS (Eng)
Mr Martin Bailey BSc FRCS
Mrs Mary Calvert BDS FDSRCS (Ed) MOrth MSc
Mr Joe Curry MBBS FRCS (Eng) FRCS (Paed Surg)
Mr David Drake MB BChir FRCS FRCPCH

Mr Robert Evans BSc BDS MScD FDSRCS (Eng) DOrth MOrth RCS (Ed)
Mr Ben Hartley BSc MB BS FRCS (ORL-HNS)
Mr Robert Hill RCS
Dr Susan Hill BM MRCP DCH
Mr Barry Jones MS FRCS
Mr David Jones FRCS FRCS Ed (Orth)
Mr Loshan Kangesu BSc MBBS FRCS MS FRCS (Plast)
Mr Edward Kiely FRCSI FRCS
Dr Michael Mars PhD BDS FDS DOrth
Mr Fergal Monsell MSc FRCS FRCS (Orth)
Mr Hilali Noorden MA FRCS (Eng) FRCS (Orth)
Dr Neil Shah MB BS PhD
Mr Paul Smith FRCS
Dr Virpi Smith FIBMS PhD
Mr Brian Sommerlad FRCS
Mr Stuart Tucker FRCS

Cancer theme

Molecular Haematology and Cancer Biology Unit
Reader in Molecular Neurobiology and Acting Head of Unit
Dr Jonathan Ham BSc PhD
Emeritus Professor of Haematology and Oncology
Professor Judith Chessells MD FRCP FRCPath
Visiting Professor of Molecular Haematology
Professor Paul Brickell BA MA PhD
Visiting Professor of Haematology and Oncology
Professor Ian Hann MD FRCP FRCPath
Reader in Paediatric and Developmental Pathology
Professor Neil Sebire BSc MB BS DM FRCPath
Senior Lecturers
Dr John Anderson BA MB BS MRCP PhD
Dr Michael Hubank BA PhD
Dr Arturo Sala PhD
Honorary Senior Lecturers
Dr Peppy Brock MD PhD

Dr Julia Chisholm MRCPCH MA PhD
Dr Ann Goldman MB BCh FRCP
Dr Gill Levitt BSc MRCP DCH
Dr Raina Liesner BA MB BChir MRCP
Dr Antony Michalski MB ChB MRCP
Dr David Webb MD FRCP
FRCPath MRCPCH
Lecturer
Dr Owen Williams BSc PhD
Honorary Lecturer
Dr Alison Leiper MB BS MRCP

Cardiorespiratory sciences theme

Theme Leader
Professor John Deanfield

Cardiac Unit
The British Heart Foundation Vandervell Professor of Congenital Heart Disease and Head of Unit
Professor John Deanfield MD BChir FRCP
Professor of Cardiothoracic Surgery
Professor Marc de Leval MD FRCS (retired in 2006, but still holds honorary contract)
The British Heart Foundation Joseph Levy Professor of Paediatric Cardiac Morphology
Professor Robert Anderson BSc MD FRCPath (retired)
Professor of Cardiology
Professor Philipp Bonhoeffer MD FSCAI
Professor of Cardiothoracic Surgery
Professor Martin Elliott MD FRCS
Professor of Cardiology
Professor William McKenna BA MD DSc FRCP FESC FACC
Professor of Cardiovascular Imaging
Professor Andrew Taylor BA (Hons) MD MRCP(UK) FRCR
Senior Lecturers
Dr Andrew Cook PhD (British Heart Foundation Lecturer)
Dr Perry Elliott MBBS MD MRCP
Ms Catharina van Doorn MD FRCS (C/Th)

Honorary Senior Lecturers
Dr Kate Brown BChir MRCP (joint with Portex)
Dr Michael Burch MB ChB MD FRCP FRCPCH
Dr Allan Goldman MB BChB MRCP MSc
Dr Nick Piggott MB BS MRCPI MRCPCH
Dr Philip Rees MB BChir DRCOG FRCP
Dr Margrid Schindler MD MB BS FFICI-ANZCA (joint with Portex)
Dr Ian Sullivan MB BChir FRACP
Mr Victor Tsang MB BS MS MSc FRCS FRCS (Ed)
Dr Robert Yates BSc(Med) MB Bch

Portex Anaesthesia, Intensive Therapy and Respiratory Medicine Unit
Professor of Respiratory Physiology and Head of Unit
Professor Janet Stocks PhD
Smiths Medical Professor of Anaesthesia and Critical Care
Professor Michael (Monty) Mythen FRCA
Emeritus Professor of Paediatric Anaesthesia
Professor David Hatch
Honorary Professor in Cardiovascular Genetics
Professor Hugh Montgomery BSc MB BS MRCP MD
Honorary Reader in Paediatric Intensive Care
Dr Quen Mok MB BS MRCP MRCPI DCH
Honorary Reader in Respiratory Paediatrics
Dr Colin Wallis MB ChB FCP (Paed) MD DCH FRCP
Senior Lecturers
Dr Eleanor Main BA PhD
Dr Mark Peters MB BCh MRCP
Dr Suellen Walker MB BS MM(PM) MSc FANZA FFPMANZCA

Honorary Senior Lecturers
Dr Paul Aurora BSc MB BS MRCP MSc
Dr David de Beer BSc MB ChB DCH FRCA
Dr Robert Bingham MB BS FRCA
Dr Ann Black MB BS DRCOG FRCA
Dr Philip Cunnington MB BS DA FRCA
Dr Robert Dinwiddie MB ChB FRCP FRCPCH DCH
Dr Hilary Glaisyer MB BS MRCP FRCA
Dr Andreas Goebel MD PhD FRCA
Dr Louise Harding MB BS FRCS
Dr Jane Herod BSc MB BS FRCA
Dr Richard Howard BSc MB ChB FRCA
Dr Elizabeth Jackson BSc MB BS MRCP FRCA
Dr Ian James MB ChB FRCA
Dr David Kilner PhD
Dr Adrian Lloyd-Thomas MB BS FRCA
Dr Richard Martin MBBS FRCA DCHyp FRSM MSBST
Dr Angus McEwan MB ChB FRCA
Dr Reema Nandi MB BS FRCA, MD
Dr Kar-Binh Ong BA MB BS FRCA
Dr Andy Petros MB BS MSc FRCP FRCPCH
Dr Christine Pierce MD BSc BBS MRCP
Dr Helen Spencer
Dr Ranjan Suri MBChB MRCPCH MD
Dr Michael Sury MB BS DA FRCA
Dr Mark Thomas BSc MBBChir FRCA
Dr David Walker BM (Hons) MRCP FRCA
Dr Isabeau Walker BSc MBBChir FRCA
Dr Glyn Williams MBBS FRCA MD

Patient Care Research and Innovation Centre
Chair of Children’s Nursing Research and Head of Unit
Professor Linda Franck PhD RN RGN RSCN FRCPCH FAAN
Senior Lecturer
Dr Faith Gibson MSc (Cancer Nursing) RSCN RGN CertEd RNT PhD
Honorary Senior Lecturer
Dr Debbie Sell SRSLT FRCSLT PhD

Senior academic staff 2009
continued

General and adolescent
paediatrics theme

General and Adolescent Paediatrics Unit
Nuffield Professor of Child Health and Head of Unit (from October 2009)
Professor Terence Stephenson DM FRCP FRCPCH
Professor of Paediatric Gastroenterology
Professor Alan Phillips PhD, FRCPCH
Professor of Paediatrics
Professor R Mark Gardiner MBBCh MD FRCPCH FMedSci
Honorary Professor of Paediatric Medicines Research
Professor Ian Wong BSc MSc PhD MRPharmS ILTM (HE)
Emeritus Professor of Child Health
Professor Brent Taylor PhD MB ChB FRCP FRACP
Reader in Molecular Cell Biology
Dr Sara Mole PhD
Reader in Adolescent Health and Acting Head of Unit (until October 2009)
Dr Russell Viner FRCP FRCPCH FRACP PhD MBBS
Senior Lecturers
Dr Eddie Chung MBChB MRCP
Dr Hannah Mitchison PhD
Dr Alastair Sutcliffe MD MRCP MRCPC
Honorary Senior Lecturers
Dr Deborah Christie DClinPsych
Dr Haitham Elbashir MBBS FRCPCH DCH MD (Lond)
Lecturers
Dr Indrani Banerjee PhD
Dr Jill Ellis PhD
Dr Kate Everett PhD
Dr Christina Georgoula MRCPC
Dr Camilla Salvestrini MD

Genes, development and disease theme

Theme Leader
Professor Peter Scambler

Clinical and Molecular Genetics Unit
Professor of Clinical and Molecular Genetics and Head of Unit
Professor Gudrun Moore BA PhD
Professor of Paediatric Metabolic Disease and Hepatology
Professor Peter Clayton MD FRCP FRCPCH
Professor of Genetics and Fetal Medicine
Professor Lyn Chitty BSc PhD MB BS MRCOG
Professor of Paediatric Endocrinology
Professor Mehul Dattani MD FRCP
Professor of Clinical Chemistry (from April 2009)
Professor Simon Heales BSc PhD CSci FRCPath
Professor of Clinical Genetics and Dysmorphology (until December 2009)
Professor Raoul Hennekam MD PhD
Professor of Paediatric Endocrinology
Professor Peter Hindmarsh BSc MB MD BS FRCP (joint with UCL Medicine)
Emeritus Professor of Paediatric Genetics
Professor Marcus Pembrey BSc MB BS MD FRCP FRCPCH FMedSci
Emeritus Professor of Child Health and Growth
Professor Michael Preece MD MSc FRCP FRCPCH
Emeritus Professor of Molecular Genetics
Professor Susan Malcolm PhD FRCPath
Emeritus Professor of Molecular Embryology
Professor Marilyn Monk
Emeritus Professor of Biochemistry
Professor Bryan Winchester MA PhD

Honorary Professor of Neonatal Paediatrics
Professor John Wyatt BSc MB BS DCH FRCP (joint with UCL Paediatrics and Child Health)
Reader and Wellcome Trust Senior Fellow in Clinical Science
Dr John Achermann MA MD MRCP MRCPC (joint with UCL Medicine)
Reader and Honorary Consultant in Clinical Genetics
Dr Maria Bitner-Glindzicz BSc MB BS PhD FRCP
Senior Lecturers
Dr Elizabeth Carrey PhD
Dr Khalid Hussain MB ChB MSc MRCP MRCPC
Dr Shamima Rahman MA MRCP MRCPC PhD
Honorary Senior Lecturers
Dr Angela Barnicoat BSc MD DRCOG FRCP
Dr Caroline Brain MB MD FRCP FRCPCH
Dr Maureen Cleary MD MRCP MB ChB
Dr Ying Foo PhD
Dr Stephanie Grunewald MD
Dr Richard Jones BSc MBChB DPhil MRCPath
Dr Melissa Lees MRCP MSc MD FRACP
Dr Alison Male BSc MBBS MRCP
Dr Elisabeth Rosser FRCP BSc MB BS
Dr Ashok Vellodi FRCP FRCPCH
Dr Louise Wilson BSc MB ChB FRCP
Lecturers
Dr Kevin Mills PhD
Dr Owen Williams PhD (joint with Molecular Haematology and Cancer Biology Unit)

Molecular Medicine Unit
Professor of Molecular Medicine and Head of Unit
Professor Peter Scambler BSc MB ChB FRCPath FMedSci
Professor in Molecular Cardiology
Professor Paul Riley BSc PhD

Professor of Computational Biology
Professor Peter Hammond BA PhD
Professor of Medical and Molecular Genetics, Wellcome Trust Senior Research Fellow and Honorary Consultant in Clinical Genetics
Professor Philip Beales BSc MD MRCP
Senior Lecturer
Dr Hannah Mitchison BSc PhD
Lecturer
Dr Kate Everett BSc MPhil PhD PgDip

Medical Molecular Biology Unit
Professor of Human Genetics and Head of Unit
Professor David Latchman MA PhD DSc FRCPath FRSA
Reader in Molecular and Cellular Biology
Dr Anastasis Stephanou BSc PhD
Honorary Senior Lecturer
Dr Richard Knight MD PhD
Lecturer
Dr Vishwanie Budhram-Mahadeo BSc PhD

Nephro-Urology Unit
Reader in Nephrology and Head of Unit (from January 2010)
Dr Paul Winyard BM BCh MA PhD FRCPCH
Honorary Professor of Nephrology and Head of Unit (until December 2009)
Professor Adrian Woolf MA MD FRCPCH
Honorary Professor of Nephrology
Professor Robert Kleta MD PhD
Emeritus Professors of Paediatric Nephrology
Professor Martin Barratt FRCP CBE
Professor Michael Dillon FRCP FRCPCH
Reader in Paediatric Nephrology
Dr Lesley Rees MD FRCP FRCPCH

Honorary Readers in Paediatric Nephrology
Dr Richard Trompeter FRCP FRCPCH
Dr William van't Hoff BSc MD FRCP FRCPCH
Honorary Senior Lecturers
Dr Detlef Böckenhauer MD PhD
Mr Francis Calder MB FRCS (joint with Guy's and St Thomas' NHS Foundation Trust)
Mr Abraham Cherian MBBS MS DNB FRCS FRCS (Paed Surg)
Mr Peter Cuckow FRCS (joint with North Middlesex University Hospital)
Mr Patrick Duffy MB FRCS
Mr Vass Hadjianastassiou DM(Oxon) FEBVS(Vasc Surg) FRCS (Gen Surg) BSc (joint with Guy's and St Thomas' NHS Foundation Trust)
Dr Daljit Hothi MBBS MRCPC MD
Mr Geoff Koffman MBChB FRCS (joint with Guy's and St Thomas' NHS Foundation Trust)
Mr Nizam Mamode BSc MBChB MD FRCS(Gen) (joint with Guy's and St Thomas' NHS Foundation Trust)
Dr Stephen Marks MBChB MSc MRCP(UK) DCH FRCPCH
Mr Imran Mushtaq MD FRCS
Dr Rukshana Shroff MD MRCPC PhD
Mr John Taylor MD FRCS (joint with Guy's and St Thomas' NHS Foundation Trust)
Dr Kjell Tullus MD PhD FRCPCH
Honorary Lecturers
Ms Eileen Brennan RGN RSCN ENB 147 DMS MSc
Mr Divyesh Desai MB MChir
Dr Sarah Ledermann MRCP

Infection and immunity theme

Theme Leader
Professor Christine Kinnon BSc PhD

Immunobiology Unit
Professor of Vaccinology and Immunology, Director of Clinical Research and Development, and Head of Unit
Professor David Goldblatt MB ChB PhD FRCP FRCPCH
Professor of Immunology
Professor Robin Callard BSc MSc PhD DipMath BA (Maths) DSc
Professor of Experimental Immunology
Professor Tessa Crompton PhD
Professor of Paediatric Dermatology
Professor John Harper MD FRCP FRCPCH
Emeritus Professor of Molecular Immunology
Professor Malcolm Turner DSc (Med) PhD FRSC FRCPath
Lecturer
Dr Wei-Li Di MB BS PhD

Infectious Diseases and Microbiology Unit
Professor of Infectious Disease and Immunology and Head of Unit
Professor Nigel Klein BSc MB BS MRCP PhD FRCPCH
Honorary Professors
Professor Diana Gibb MBChB (Hons) MRCP MD MSc Dip Obs FRCPCH
Professor Alan Phillips PhD FRCPCH
Senior Lecturer
Dr Mona Bajaj-Elliott BSc PhD
Dr Paul Brogan BSc (Hons) MBChB (Hons) MRCPC MSc PhD (joint with Rheumatology Unit)

Senior academic staff 2009
continued

Honorary Senior Lecturers

Dr Susan Hall BSc PhD
Dr John Hartley BSc MB BS MSc DTM&H
MRCP FRCPATH
Dr Marian Malone MB BCh BAO FRCPATH
Dr Karyn Moshal MBChB MRCP
MRCPCH DTM&H
Dr Vas Novelli FRACP FRCP FRCPCH
Dr Delane Shingadia MBBS MPh
MRCP FRCPCH
Dr James Soothill MD MB BS FRCPATH
Clinician Scientist (until October 2009)
Dr Helen Baxendale BSc MB BS PhD
MRCP MRCPCH
Honorary Clinical Senior Lecturer
Dr Garth Dixon BSc MB ChB PhD
MRCP FRCPATH

Molecular Immunology Unit

Professor of Molecular Immunology and Head of Unit
Professor Christine Kinnon BSC PhD
Professor of Paediatrics and Immunology
Professor H Bobby Gaspar BSc MB BS MRCP
Professor of Paediatric Immunology and Wellcome Trust Senior Fellow
Professor Adrian Thrasher MB BS PhD
FRCP FRCPATH FMedSci
Professor of Human Molecular Genetics
Professor Robin Ali BSc PhD (joint with Institute of Ophthalmology)
Reader in Molecular Biology
Dr Kenth Gustafsson PhD
Reader in Transplantation Immunology
Dr Persis Amrolia BSC MBBS MRCP
MRCPATH PhD
Reader in Molecular Genetics
Dr Stephen Hart BSc MSc PhD
Honorary Reader in Paediatric Immunology
Dr E Graham Davies MA FRCP FRCPCH
Honorary Reader in Stem Cell Transplantation
Dr Paul Veys MBBS FRCP
FRCPATH FRCPCH

Senior Lecturer

Dr Waseem Qasim BMedSci MBBS
MRCP MRCPCH PhD
Honorary Senior Lecturers
Dr Cathy Cale BSc MB ChB PhD MRCP
MRCPCH MRCPATH
Dr Alison Jones MRCP PhD
Dr Penny Titman PhD
Lecturers/Clinician Scientists
Dr Siobhan Burns MB BCh MRCPI PhD
Dr Austen Worth PhD

Rheumatology Unit

Reader in Paediatric Rheumatology and Head of Unit
Dr Lucy Wedderburn BA PhD MB BS
FRCP MRCPCH
Professor of Paediatric Rheumatology and Director of the Centre of Paediatric and Adolescent Rheumatology
Professor Patricia Woo CBE MB BS BSc
PhD FRCP FRCPCH FMedSci
Senior Lecturer
Dr Paul Brogan BSc (Hons) MBChB (Hons)
MRCPCH MSc PhD (joint with Infectious Diseases and Microbiology Unit)
Honorary Senior Lecturer
Dr Clarissa Pilkington BSc MBBS MRCPCH
Lecturers
Dr Bin Gao MMed PhD

Neurosciences and mental health theme

Theme Leader

Professor Francesco Muntoni

Behavioural and Brain Sciences Unit

Professor of Behavioural and Brain Sciences and Head of Unit
Professor David Skuse MD FRCP
FRCPsych FRCPCH
Professor of Developmental Psychopathology
Professor Peter Hobson MB BChir PhD
CPsychol FRCPsych

Honorary Senior Lecturers

Rachel Bryant-Waugh MSc DPhil
Margaret DeJong MDCM, FRCPsych(Can)
FRCPsych(UK)
Dr Jon Goldin MB BS
Paramala Santosh MRCPsych MD
DipNB(Psych)

Developmental Biology Unit

Reader in Developmental Biology and Head of Unit

Dr Patrizia Ferretti PhD

Reader in Developmental Biology

Dr Jane Sowden BA PhD

Reader in Craniofacial Developmental Biology and Genetics

Dr Phil Stanier PhD (joint with Neural Development Unit)

Honorary Senior Lecturer

Dr Agn  s Bloch-Zupan BChD MBiolMedSc
Specialist Certificate PhD

Developmental Cognitive

Neuroscience Unit

Professor of Developmental Cognitive Neuroscience and Head of Unit

Professor Faraneh Vargha-Khadem
MA PhD

Visiting Professor

Professor Mortimer Mishkin MA PhD

Reader in Developmental Cognitive Neuroscience

Dr Torsten Baldeweg MD

Reader in Developmental Cognitive Neuroscience

Dr Michelle de Haan PhD

Honorary Senior Lecturers

Dr Luc Berthouze BSc Msc PhD

Dr Margaret Mayston BSc Msc PhD

Dr Peter Rankin BSc Msc DClinPsy

Lecturers

Dr Frederique Liegeois BSc MSc PhD

Honorary Lecturer

Dr Alexander Hogan PhD

Dubowitz Neuromuscular Centre

Professor of Paediatric Neurology and Head of Unit

Professor Francesco Muntoni MD FMedSci

Honorary Professor

Professor Caroline Sewry PhD FRCPATH

Reader in Cell Biology and Wellcome

Trust University Award holder

Dr Jennifer Morgan PhD

Neural Development Unit

GlaxoWellcome Professor of Developmental Neurobiology,

Head of Unit and Director

Professor Andrew Copp MBBS

DPhil FRCPATH FMedSci

Reader in Craniofacial Developmental Biology and Genetics

Dr Phil Stanier PhD (joint with Developmental Biology Unit)

Reader in Developmental Neurobiology

Dr Nick Greene BA PhD

Reader in Developmental Neurobiology

Dr Andrew Stoker PhD

Reader in Developmental Neurobiology and Wellcome Trust University

Award holder

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Senior Lecturer

Dr Alan Burns BSc PhD

Lecturer

Dr Erwin Pauws BSc PhD

Clinician Scientists

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Dr Nikhil Thapar BSc BM MRCP(UK)

MRCPCH(UK) PhD

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Lecturer

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FRCPsych (Hons) FRCPCH

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Professor Tony Moore FRCOphth

Honorary Reader

Miss Isabelle Russell-Eggitt MA

FRCS FRCOphth

Senior Lecturer

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Principal Research Fellow

Dr Richard Clement PhD BSc

Honorary Senior Lecturers

Mr Kanwal Nischal FRCOphth

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Honorary Lecturer

Dr Alki Liasis PhD CPSM

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Dr Christopher Clark PhD
Rank Professor of Biophysics and Head of Unit (until October 2009)
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Honorary Professor of Paediatric Imaging
Professor Isky Gordon FRCR FRCP FRCPCH
Honorary Professor of Medical Physics
Professor Andrew Todd-Pokropek PhD (joint with Department of Medical Physics and Bioengineering, UCL)
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Dr Lorenzo Biassoni MD FEBNM
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Dr Melanie Hiorns MB BS MRCP FRCR
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Dr Amaka Offiah MRCP FRCR PhD
Dr Oystein Ølsen cand.med (Norway) PhD
Dr Catherine Owens MRCP FRCR
Dr Derek Roebuck BMedSc FRCR FRANZCR FHKAM (Radiology)
Dr Karen Rosendahl MD PhD
Dr Dawn Saunders MB MD FRCR
Senior Lecturers
Dr Mark Lythgoe PhD
Lecturers
Dr Jonathan Clayden MSc PhD
Dr Martin King PhD

Population health sciences theme

Theme Leader
Professor Carol Dezateux CBE

Centre for International Health and Development
Professor of International Child Health and Head of Unit
Professor Anthony Costello MA MB BChir FRCP FRCPCH
Professor of Global Health
Professor Therese Hesketh MFPHM MRCPCH PhD MPH DTM&H DCH
Professor of International Child Health
Professor Andrew Tomkins MB BS FRCP FRCPCH FFPHM FMedSci
Professor of Disability Studies
Professor Sheila Wirz MEdFCST PhD
Emeritus Professor of Child Health and Nutrition
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Reader in Global Health
Dr Sarah Hawkes MB BS PhD
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Dr Felicity Savage MS BM BCh FRCP
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Paediatric Epidemiology and Biostatistics Unit
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Dr Claire Thorne BA MSc PhD

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Professor Christine Kinnon BSc PhD
Theme Leader, Neurosciences and Mental Health Theme
Professor Francesco Muntoni FMedSci
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Professor Carol Dezateux CBE MD MSc FRCP FRCPCH FFPHM FMedSci
Chief Executive, Great Ormond Street Hospital for Children NHS Trust
Dr Jane Collins MSc MD FRCP FRCPCH
Medical Director, Great Ormond Street Hospital for Children NHS Trust
Mr Rob Evans BSc BDS MScD FRSFRCS
Institute Manager
Ms Justine Abbott BA (Hons) MBA MSc
Head of Research and Development Office
Ms Jo Southern BSc MSc

Administration 2009

continued

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(until January 2010)

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Mr Andrew Ross MA MBA

Thomas is three years old and is being treated for a rare skin condition. He is known as King Thomas on Penguin Ward because he holds court whenever he is there. His favourite games include hiding all of the nurses' chairs in the playroom and playing hide and seek with his doctors.



**UCL Institute
of Child Health**
30 Guilford Street
London WC1N 1EH
020 7242 9789

**Great Ormond Street
Hospital for Children
NHS Trust**
Great Ormond Street
London WC1N 3JH
020 7405 9200
www.gosh.nhs.uk

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research-ich/research-and-development](http://www.ucl.ac.uk/ich/research-ich/research-and-development)
or www.gosh.nhs.uk for an online version
of this review.



Bengali

অনুবোধ করলে নিম্নলিখিত ঠিকানায় থেকে এই লেখার
অনুবাদ, বড় অক্ষর, ব্রেল বা অডিও বিবরণ পাওয়া
যাবে।

English

Translations, large print, Braille or audio
versions of this report are available upon
request from the address below.

French

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l'adresse ci-dessus. Des versions en gros
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Polish

Tłumaczenia są do uzyskania na
żądanie pod podanym powyżej adresem.
Dokumenty w formie dużym drukiem,
brajlem lub audio są także do uzyskania
na żądanie.

Punjabi

ਇਸ ਰਿਪੋਰਟ ਦੇ ਤਰਜਮੇ, ਅਤੇ ਇਹ ਰਿਪੋਰਟ ਵੱਡੇ ਅੱਖਰਾਂ
ਜਾਂ ਬ੍ਰੇਲ ਵਿਚ, ਜਾਂ ਸੁਣਨ ਵਾਲੇ ਰੂਪ ਵਿਚ ਹੇਠ ਲਿਖੇ ਪਤੇ ਤੋਂ
ਮੰਗ ਕੇ ਲਏ ਜਾ ਸਕਦੇ ਹਨ।

Somali

Turjubaan ayaa cinwaanka kor ku qoran
laga heli karaa markii la soo codsado.
Daabacad far waa-wayn, farta indhoolaha
Braille ama hab la dhegaysto ayaa xittaa
la heli karaa markii la soo codsado.

Tamil

**பெரிய அச்சில், இந்த
அறிக்கையின்
மொழிபெயர்ப்புகள், பெரிய
அல்லது ஒலி பதிப்புகள்
விண்ணப்பித்தால் கீழ்க்கண்ட
விலாசத்தில் கிடைக்கும்**

Turkish

Talep edilirse yukarıdaki adresten
çevirileri tedarik edilebilir. Talep edilirse,
iri harflerle, Braille (görme engelliler için)
veya sesli şekilde de tedarik edilebilir.

Urdu

گزارش کرنے پر یہ رپورٹ ترجمے، بڑے حروف
کی چھپائی، بریل یا آڈیو ریکارڈنگ ذیل پتے سے
حاصل کی جا سکتی ہے۔

Design Manager
Great Ormond Street Hospital
Fourth floor
40 Bernard Street
London WC1N 1LE
E.design.work@gosh.org