UCL INSTITUTE OF CHILD HEALTH

Great Ormond Street Hospital for Children
NHS Foundation Trust

Research Review 2011/12

The child first and always

Sean is 10 years old and has a condition called nephrotic syndrome, which causes his kidneys to fail. Earlier this year, Sean had both kidneys removed and was donated a kidney by his dad.

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Cover: Jessica, age 12, is a regular visitor on Island Day Unit. She has a port wine stain and is currently having laser treatment to reduce its intensity.

Jack, 13, has been visiting Great Ormond Street Hospital fairly frequently since February, due to problems with his airway and lungs. He is very impressed with the new ENRC Cardiac Day Care Unit on Walrus Ward, especially because of all the new TVs which he can watch sports on. He loves football and is a huge Manchester United fan!

Director's report

The UCL Institute of Child Health (ICH) is Europe's largest academic centre for research and education in children's health and disease. As part of University College London's (UCL) Faculty of Population Health Services, our research stands to improve the lives of children and families across the UK and around the world.

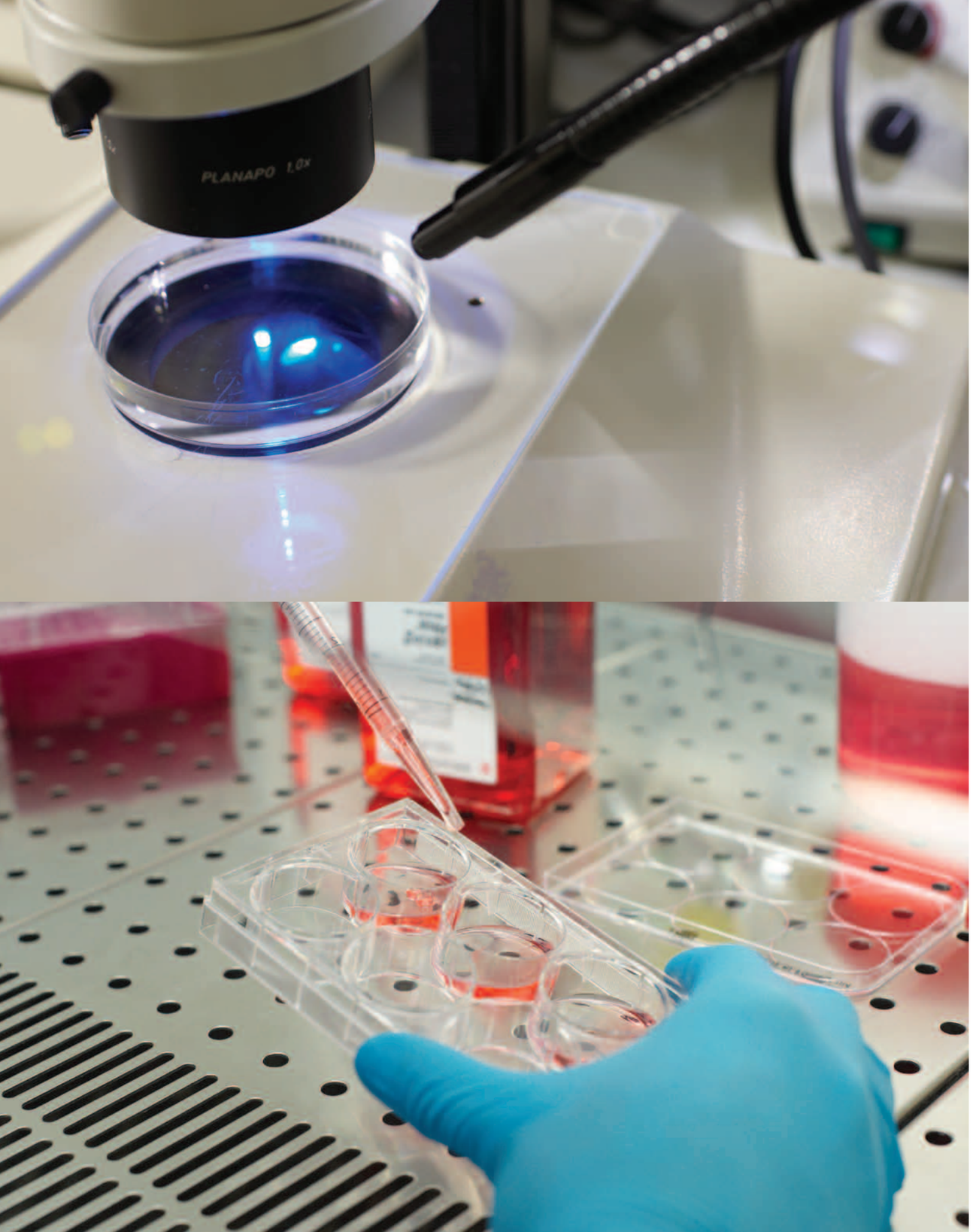
The ICH became a founding member of the Faculty of Population Health Sciences in 2011, created as part of a restructuring of the UCL School of Life and Medical Sciences. This emphasises the Institute's strength in epidemiology through its Medical Research Council (MRC) Centre of Epidemiology for Child Health (Head: Professor Carol Dezateux), and the UCL Institute for Global Health (Head: Professor Anthony Costello). Excitingly, the faculty brings together the entire life course of human health, from conception to birth (the Institute for Women's Health), from newborn to adolescence (the ICH), and from young adulthood to old age (the Institute of Epidemiology and Health Care). Our basic science and clinical research, which continues to be the ICH's largest area of concentration, is now focused ever more on translation into improvements in children's health, not only for individuals and families, but also at the population level.

The Institute welcomed new senior academics during the year. Dr Paul Gissen came from Birmingham University to develop a research programme in metabolic paediatrics, as a joint appointment with UCL's MRC Laboratory of Molecular and Cell Biology. Dr Manju Kurian also joined the ICH from Birmingham to develop her work on the genetic basis of children's movement disorders. Dr Nadia Micali arrived from the Institute of Psychiatry as holder of a prestigious National Institute of Health Research Clinician Scientist award, to develop her research programme into eating disorders in young people.

Education plays an ever more important role in the life of the ICH, as a key part of our mission to train the next generation of child health professionals. Professor Christine Kinnon was appointed Vice-Dean for Education within the Faculty of Population Health Sciences, and the Institute launched two new Masters level courses in cell and gene therapy and child and adolescent mental health. It was with great joy and anticipation that we opened a completely refurbished Wolfson Centre in Mecklenburgh Square. As our first ever dedicated teaching and conference room resource, the centre offers numerous meeting rooms with audio-visual facilities, a coffee bar, a student common room, and two computer cluster rooms for the taught course students. This superb new facility was made possible by funding from Great Ormond Street Hospital Children's Charity and the Child Health Research Appeal Trust.

In this time of financial stringency, it becomes ever more challenging to obtain significant research grants from the research councils and major research charities. Congratulations are due, therefore, to the many Institute staff who achieved significant awards during the year. These include Professor Pete Scambler (Molecular Medicine Unit), who was awarded £1.21 million by the British Heart Foundation for a programme grant entitled *Tbx1 and cardiovascular morphogenesis: genetic networks and tissue interactions;* Professor Faraneh Vargha-Khadem (Developmental Cognitive Neurosciences Unit) who obtained £1.43 million for an MRC programme grant *Investigation of hypoxia/ischaemia and patterns of neuropathology associated with memory impairment, from infancy through adolescence;* and Dr Paul Gissen (Clinical and Molecular Genetics Unit) who was awarded a Wellcome Trust Senior Clinical Fellowship worth £1.29 million for his project *The role of two key intracellular trafficking genes, VPS33B and VIPAR, in development and disease.*

Professor Phil Beales of the Molecular Medicine Unit was elected a Fellow of the Academy of Medical Sciences (FMedSci), in recognition of his contributions to human genetics and birth defects research.



State-of-the-art facilities and equipment are crucial to maintaining our status as one of the world's leading paediatric research institutions.

Director's report
continued

Professor Anthony Costello (UCL Institute for Global Health) was awarded the James Spence Medal, the highest honour of the Royal College of Paediatrics and Child Health. Among our staff who achieved senior promotion at UCL, Jane Sowden became Professor of Developmental Biology and Genetics for her research into the genetics and stem cell therapy of eye disorders, and Russell Viner became Professor of Adolescent Health for his work on diabetes, obesity, and risk-taking behaviours in young people. Promoted to Reader were Khalid Hussain, a paediatric endocrinologist investigating disorders of the pancreas; Shamima Rahman, a metabolic paediatrician specialising in mitochondrial disorders; and Alastair Sutcliffe, a paediatrician studying health outcomes of children conceived by assisted reproductive technologies.

I am pleased to report a continuing excellent performance by members of the Institute in publishing the outcomes of their research in the highest ranking journals. In 2011, I instigated *Paper of the Month* as part of my regular news bulletin for staff, and this has generated considerable competition among authors! To mention just a few of the landmark studies in 2011: new gene discoveries were reported by Phil Beales and colleagues for 3MC syndrome *(Nature Genetics),* and by Khalid Hussain's team for an inherited form of hypoglycaemia *(Science);* stem cells capable of repairing the heart after infarction were reported by Paul Riley and co-workers *(Nature),* while stem cells yielding pituitary tumours were identified by JP Martinez-Barbera's group *(Proceedings of the National Academy of Sciences);* a first step towards clinically applicable gene therapy for Duchenne muscular dystrophy was reported by Francesco Muntoni's team, while 50-year mortality trends in children and young people from low-, middle-, and high-income countries were published by Russell Viner and colleagues (both studies in the *Lancet*).

This will be my last report as Director of the ICH: I step down in September 2012, after almost 10 years in the post. It has been an enormous privilege to lead this outstanding Institute over a formative period in its existence. With the invaluable help of Great Ormond Street Hospital Children's Charity, we have been able to greatly improve the estate, converting the Institute's main building from only two floors of laboratories in 2003, to its current six-floor laboratory structure. We have extended the range of research and teaching to encompass general and adolescent paediatrics, alongside our rare diseases research.

At the same time, we have strengthened the Institute's close and productive relationship with Great Ormond Street Hospital. Perhaps most important of all, we have brought a new generation of young clinical and non-clinical investigators into team leader positions, ensuring that the highest quality of child health research will continue in future. The past 10 years have seen the full integration of the Institute within UCL, enabling the ICH to go forward as part of a world-class, multi-faculty university. I wish the next director every success and look forward to working, in my continuing role as Professor of Developmental Neurobiology, towards the very bright future that I am sure awaits the ICH.

Professor Andrew Copp
Director
UCL Institute of Child Health

Samaviya was diagnosed with cystinosis – a hereditary disease which also affects her aunts. She is one of the first patients to stay on Eagle Ward in the Morgan Stanley Clinical Building, and is very much enjoying having her mum stay by her bedside. The en suite is proving to be entertaining for Samaviya, as she loves playing with water!

Chief Executive's report

Great Ormond Street Hospital (GOSH) has recently become an NHS Foundation Trust. This is important because it will allow us to remain independent as an NHS hospital dedicated exclusively to the care of children. And it's the care of children that matters to all of us and our collective determination to find new and better ways to help them, through advancements in medical research.

Today, doctors all over the world are able to treat childhood conditions that were untreatable even 10 or 20 years ago. Doctors at GOSH and researchers at the UCL Institute of Child Health have been involved in many of these discoveries but we have much more to do.

For instance, there is now a very high survival rate for acute lymphoblastic leukaemia, the most common childhood cancer. Yet there are still some children who do not respond to the conventional treatment. In this report, you can read about a landmark trial led by Professor Persis Amrolia. He is using some of the latest research techniques and scientific thinking to try and find a solution for that minority of children with this type of leukaemia for whom the outcomes are still uncertain.

GOSH has a diverse and distinctive patient population. Many of the children who are referred here have rare or complex conditions. We probably see more of these children than any other hospital in the UK, and our partnership with the Institute offers us a unique opportunity to conduct research which will translate directly into patient benefit.

New scientific breakthroughs, particularly in genetics, cell therapies and regenerative medicine offer real hope for the future. Our clinicians and researchers across a wide range of specialties are all considering how these new techniques and discoveries could benefit our patients and sick children more widely. A few of these examples are contained in this report.

We are at an early stage of this work, particularly in the field of rare diseases, where so many remain undiagnosed. An important step forward is the National Institute for Health Research's renewal of our status as a Biomedical Research Centre. We are the only centre specialising in paediatrics and the five-year award gives us funding to help advance our research into these rare conditions affecting children.

Planning for our future research needs is vital and I am delighted that Great Ormond Street Hospital Children's Charity announced its plans to build a new Centre for Children's Rare Disease Research, which is planned to open in 2018. This new centre will complement the existing research activities which take place throughout the hospital and the Institute. Specifically, we hope it will provide platform technologies which might support the work of clinical and research teams across many of the hospital's 50 clinical specialties.

We have to be ready to take advantage of new knowledge as it becomes available. Only by planning for the future can we ensure that our work continues to help the children we see as quickly as possible.

It's been a great privilege to be Chief Executive of this great hospital and to have witnessed the extraordinary work carried out by scientists and doctors to help children. As I pass the baton on to the next Chief Executive, I know that they cannot fail to be impressed by the dedication and determination of the staff here and at the Institute to pursue medical research that will benefit children for generations to come.

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

Division of Research and Innovation report

The UCL Institute of Child Health (ICH) and Great Ormond Street Hospital's (GOSH) joint Division of Research and Innovation continues to support world-leading efforts in advancing paediatric medicine.

We were delighted that in August 2011, the National Institute for Health Research (NIHR), advised by an international panel of experts, confirmed a further five years of funding for the Biomedical Research Centre (BRC) based at GOSH and the ICH. The award is for a total of £36 million and supports the only such centre in the UK solely focused on paediatric experimental medicine.

Underpinning the strategy of the GOSH BRC is research to bring basic laboratory scientific advances into the clinical setting to maximise patient benefit. This programme of research includes accelerating the discovery of the molecular basis of childhood diseases, developing new diagnostics and imaging modalities, and evaluating novel treatments and gene, stem and cellular therapies. The main focus of our second BRC award will be on rare diseases, recognising the collective burden they represent and the way their study informs our understanding of generic and more common disease mechanisms.

Experimental medicine research at GOSH aims to deliver clinical improvements for the children in our care. This year's *Research Review* includes Professor Francesco Muntoni's work, which has shown for the first time that gene therapy can recover lost dystrophin expression in skeletal muscle of boys with Duchenne muscular dystrophy. The Somers Clinical Research Facility continues to play its crucial role in delivering trials of new therapies. Last year, a study of atypical haemolytic uraemic syndrome in kidney transplant patients, led by Dr Lesley Rees, showed treatment with eculizumab can restore normal kidney function and reduce the risk of graft rejection in kidney transplant recipients.

We also continue to advance the field of diagnostics as a means of identifying and preventing childhood illnesses. Work by Professor Lyn Chitty has helped in the development of a clinical service for non-invasive prenatal testing for foetal sex determination, and for Down's syndrome and genetic conditions such as cystic fibrosis and sickle call anaemia. This has the potential to reduce the number of invasive tests, which carry a one per cent risk of miscarriage, by up to 50 per cent.

Staffing support within the Division of Research and Innovation has continued to grow over the last year, with the development of a specialist team in research facilitation and the continued growth of our research governance, industrial collaboration, clinical trials, and costings and contracts teams. The NIHR has set a target for study set-up arrangements to be completed within 70 days which comes into effect in 2012, and preparations to meet this divisionally are well underway.

Our performance was also reviewed via a recent Medicines and Healthcare products Regulatory Agency routine inspection. As part of this, a number of GOSH-sponsored studies were selected for detailed routine analysis, along with examination of Research and Development procedures and governance arrangements. The division is delighted to report that there were no critical findings – a testament to the effort and dedication of all the staff whose work has contributed to supporting our research.

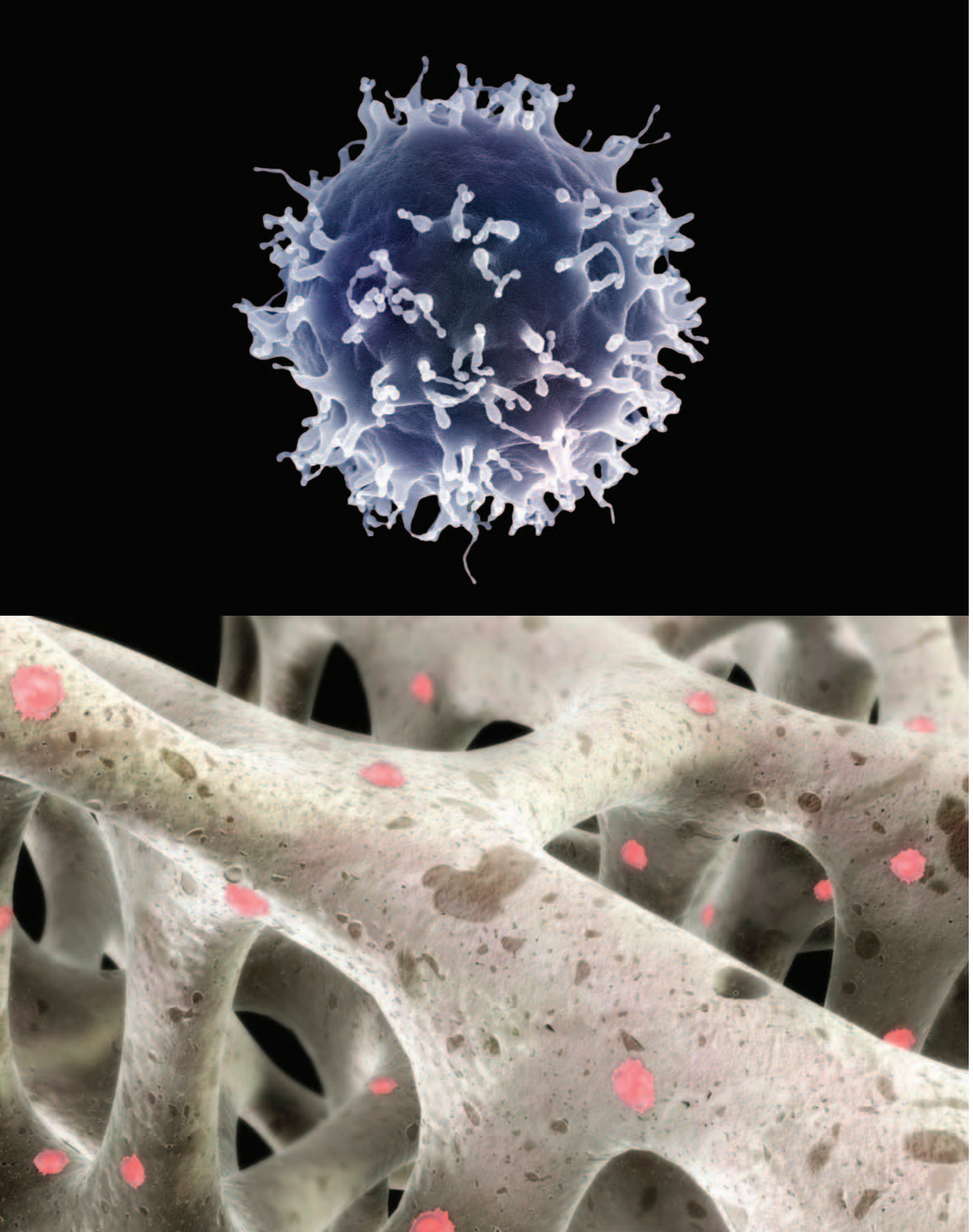
We hope that the examples in this year's review demonstrate just some of the breadth and scope of work currently underway to improve the lives of children at GOSH.

Professor David Goldblatt
Director of Clinical Research and Development

Kyomie is 12 years old and is staying on Sky Ward. She has cystic fibrosis and is having intravenous treatment every two months at Great Ormond Street Hospital. She is here today with her mum and baby sister.

Much of our research will revolutionise future medical practice. But it takes time to progress. We have to keep sight of how our daily work helps children get better.

Research into regenerative medicine is revealing how we can mimic nature's own healing pathways to grow healthy tissue. In this case, seeding stem cells onto a cell-free scaffold of the lungs as a first step towards a personalised transplant.



T-time for life-saving transplants

Since carrying out the UK's first successful bone marrow transplant in a child over 30 years ago, Great Ormond Street Hospital has led advances in blood and marrow stem cell transplantation. Dr Waseem Qasim is re-engineering disease-fighting T cells, to improve these treatments. His work could prevent life-threatening complications from viruses, and even pave the way for trials of a new therapy for HIV.

Dr Waseem Qasim
"Our immune system relies heavily on a group of white blood cells called T cells to orchestrate the body's defences against infection. I became interested in these cells at medical school as it was becoming apparent that they have remarkable memory properties important for vaccination, and are also the key cells involved in transplantation.

"During my paediatric training, I looked after children without functioning T cells, either because they had received chemotherapy, or they had been born with defective immune systems. There were also children born with HIV infection, a virus that directly attacks T cells.

"As we have learnt more about how such viruses infect cells, it has become possible to engineer T cells by using modified viruses. We can now introduce new genes into T cells to make them safer, redirect their specificity, or to alter their function and even protect them against HIV.

"As a clinician and a scientist, my job now is to help take the most promising of these emerging technologies into useful treatments, for blood and immune disorders, and other conditions where genetic correction may provide a cure."

T cells (above) are the body's principal disease-fighting agents. Our researchers are leading efforts to manipulate them to improve bone marrow transplants (below) and treat complex and incurable diseases.

The past two decades have seen remarkable improvements in the safety and clinical usefulness of blood and marrow stem cell transplants, which are now used to treat an ever-widening range of immune diseases, leukaemia, and other rare and inherited paediatric conditions affecting the skin, gut and blood.

Dr Waseem Qasim, a Consultant in Paediatric Immunology and Senior Lecturer in the UCL Institute of Child Health's Molecular Immunology Unit, explains what has been involved.

"Over the past 20 years, we've helped to turn blood and marrow stem cell transplants from a risky treatment of last hope, into curative therapies that in some cases have a success rate of over 90 per cent," he says. "Our challenge now is how to help children who don't have a well-matched donor like a brother or sister, and to improve the outlook for those who face life-threatening viral infections or side effects from existing treatments."

The key to this challenge lies in understanding and controlling T cells. These specialised blood cells provide life-long immunity against infection, co-ordinating the body's defences against viruses and fighting other disease-causing and foreign organisms. However, these very properties can lead to potentially life-threatening situations.

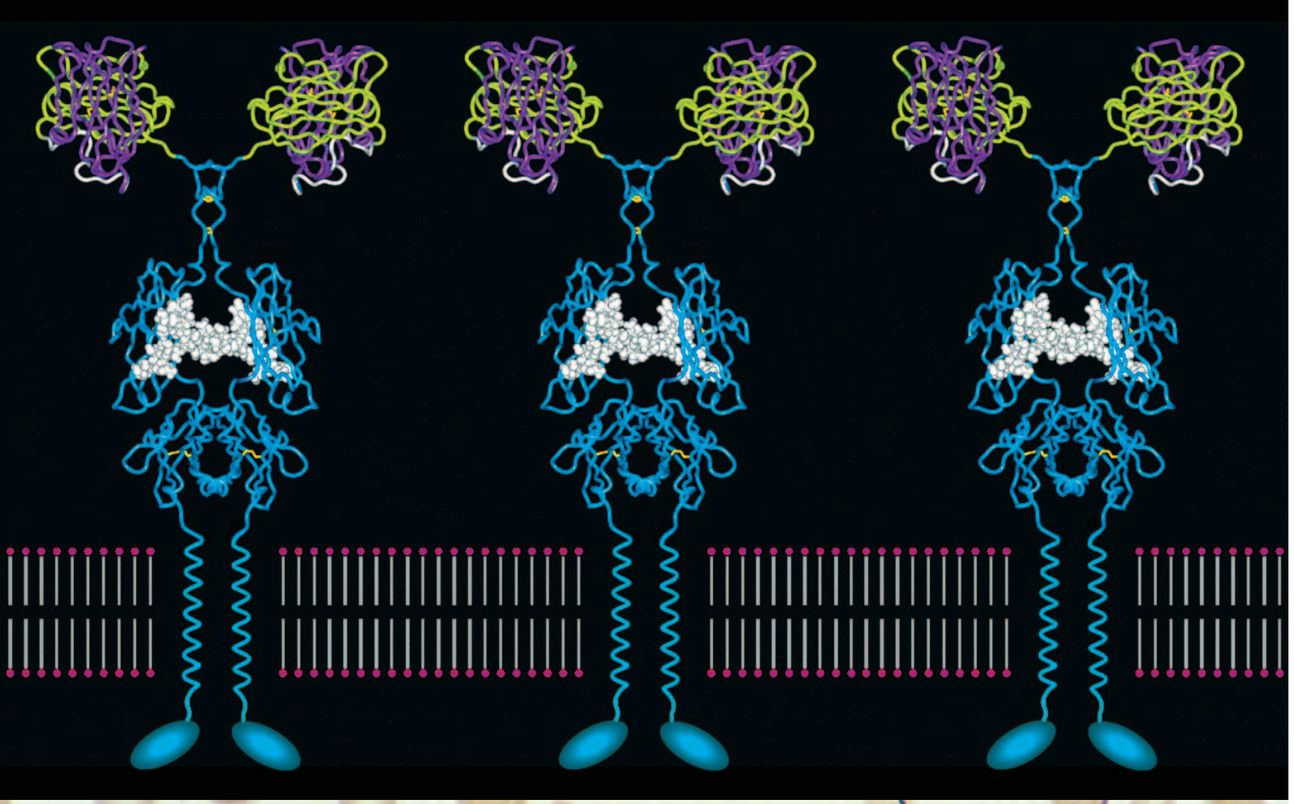
"Even with a well-matched transplant, T cells can be unpredictable," explains Dr Qasim. "If they mount an attack against healthy organs and tissues it can cause serious problems, requiring us to give drugs to suppress patients' immune systems for extended periods of time."

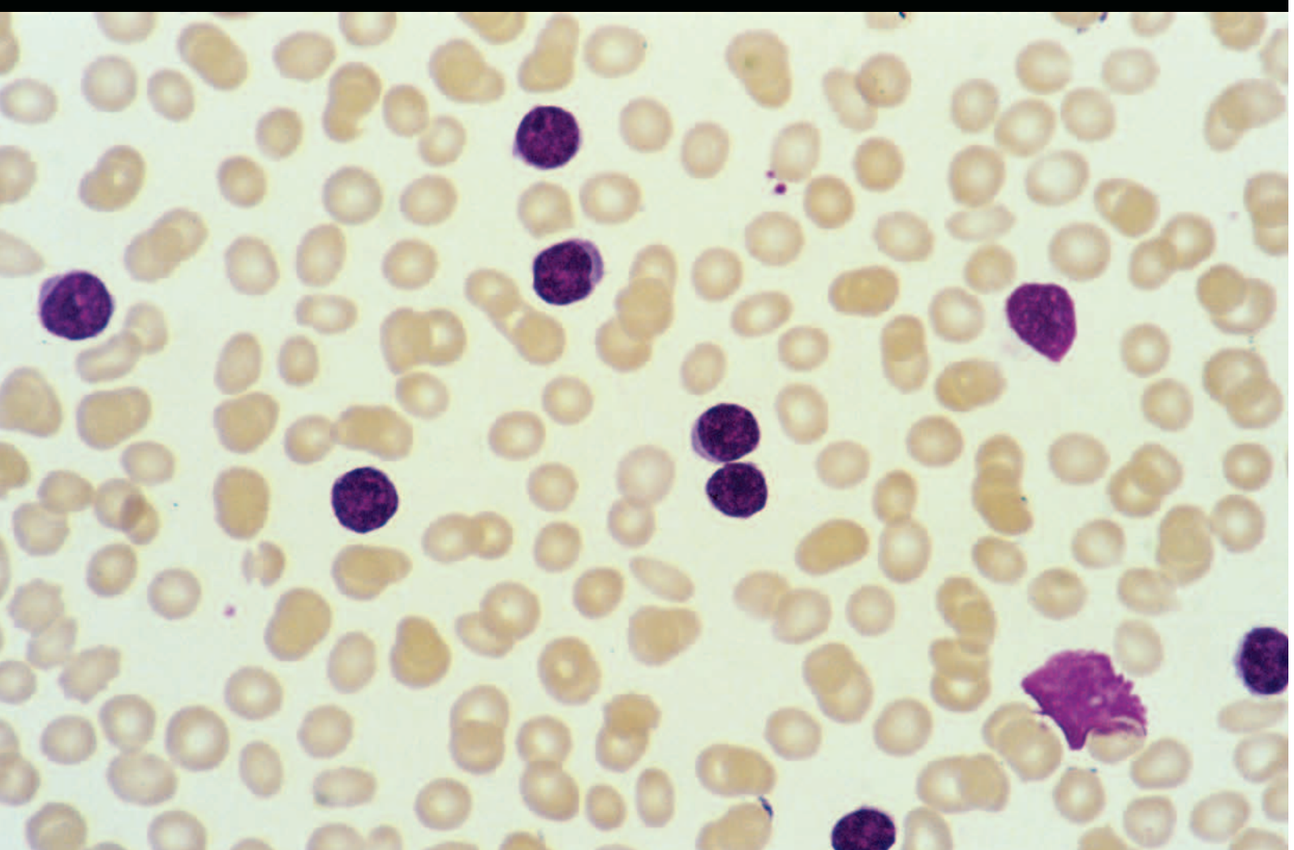
Dr Qasim notes that he has been fortunate to work in a setting where it is possible to translate scientific knowledge of how the immune system works into trials of new therapies. One approach draws on the same technology and expertise used in the hospital's world-leading trials of gene therapy. Here, modified viruses are being used to engineer T cells with controllable 'suicide' genes, so they can be safely eliminated if they begin to cause unwanted side effects after transplantation.

Another project currently underway is to reduce the risk of viral infections in children following a bone marrow transplant. It involves detecting and isolating T cells in the transplant which will react to viruses, but not attack healthy tissue. These cells can then be given to children if and when a viral infection occurs. In association with a London-based company, CellMedica, the UK Technology Strategy Board has recently agreed to fund a new clinical trial of this therapy for all children undergoing a high-risk transplant at the hospital.

With support from Great Ormond Street Hospital Children's Charity, the hospital's gene and cellular therapy programme is now being expanded in exciting new directions. In addition to trials of new cancer therapies, Dr Qasim and his colleagues plan to use similar gene therapy technology to treat children born with HIV.

"The loss of T cells in children with HIV mirrors the problems we see in children undergoing transplantation," explains Dr Qasim. "Remarkably, we've been able to use disabled versions of the HIV virus itself, to transfer genes coding for a protective protein into T cells, and prevent the 'wild type' virus from taking hold. This could offer the prospect of rebuilding the immune system of patients who can't tolerate antiretroviral drugs, which really would represent a landmark milestone in the treatment of this disease."





Beating leukaemia with the best of modern biomedicine

Fifty years ago, the nation's first children's leukaemia research unit was established at Great Ormond Street Hospital and the UCL Institute of Child Health (ICH), to tackle a disease thought by most to be incurable. Today, overall survival rates are upwards of 80 per cent. Professor Persis Amrolia is leading a landmark trial to help children at the highest risk of relapse, whose leukaemia remains unresponsive to existing therapies.

Professor Persis Amrolia

"Bone marrow transplantation, particularly for children, is an incredibly rewarding, intense and interesting field to work in: you look after critically-ill patients with a huge diversity of complications and the majority are cured and go back to a normal life. A key attraction for me is that this work really is at the cutting edge of science and medicine.

"My lab group focuses on two main areas. In the first, we're trying to reduce the toxicity of transplants by using gentler agents rather than intensive chemotherapy/radiotherapy. Our unit pioneered this approach in children with genetic diseases of the immune system and we are now trialling the use of stem cell-specific antibodies, ultimately aiming to perform transplants without any chemotherapy.

"We are also working on cellular therapy to improve children's immunity after a transplant. We have just started an exciting new trial of this approach, using genetically modified T cells to target relapsed leukaemia.

"I hope that the development of these sorts of targeted therapies will in the future make bone marrow transplants – currently still quite a blunt tool – both safer and more effective."

Re-engineering T cells in the laboratory to express a CD19 chimeric receptor (above) enables them to recognise and kill leukaemic cells (below) that persist after a bone marrow transplant, providing a new line of therapy for children with acute lymphoblastic leukaemia.

Research to deliver clinical advances in paediatric medicine faces numerous challenges. Children often have complex rare diseases and research trials must pool patients across numerous collaborating centres. The strict regulatory requirements for novel therapies impose an extremely high quality threshold to ensure patient safety, often difficult for academic institutions to attain. Notwithstanding these issues, Professor Persis Amrolia and colleagues have recently launched an EU-wide trial of a new therapy for leukaemia, at the very forefront of immunological, genetic, and cellular research.

"Acute lymphoblastic leukaemia (ALL) is the most common form of childhood cancer. Fortunately, the majority of children with this disease can now be cured by front-line chemotherapy," says Professor Amrolia, a Consultant in Bone Marrow Transplantation and Professor of Transplantation Immunology at the ICH. "For those whose leukaemia persists, many will need a bone marrow transplant. But if this also fails, we've traditionally had no further curative treatment to offer."

The limitations of existing treatments were twofold. Firstly, these patients have had the maximum amount of chemotherapy and radiotherapy it is possible to give – their bodies would not tolerate further exposure. Secondly, because the donated immune cells of a transplant do not recognise ALL, their immune systems could not mount an attack. Working with Dr Martin Pule, Senior Lecturer in Haematology at UCL, Professor Amrolia's team therefore sought to trial a completely new approach, combining the latest advances in genetic and cellular therapy.

"Cells with ALL express a protein called CD19 on their surface," explains Professor Amrolia. "We wanted to enable the body's disease-fighting immune cells – T cells – to recognise and kill ALL cells. So we combined a CD19-specific antibody with a T cell's 'kill' switch, to form a chimeric T cell receptor. Our laboratory tests showed T cells carrying this chimeric receptor could attack and prevent the spread of ALL. Also, as CD19 is not expressed on any cells outside of the bone marrow, these hybrid cells do not target other organs, reducing the risk of organ damage and potentially lethal graft-versus-host disease."

With a clever piece of scientific forethought, the team chose to genetically engineer T cells specific for Epstein-Barr virus (EBV), an infection that most people have been exposed to. This means that if the persistence and survival of the chimeric T cells in patients is poor, they will be able to boost the anti-leukaemic response, by vaccinating with EBV-containing cells. This could prove crucial to maintaining an effective level of leukaemia-fighting cells in patients' bloodstreams.

"This study will be the first clinical study of T cell gene therapy for paediatric ALL," notes Professor Amrolia. "To get this far has taken five years of painstaking scale-up work, involving colleagues across the hospital, UCL and its Cancer Trial Centre, and numerous collaborating centres across Europe. I'm grateful for funding from the EU FP6 framework, the Department of Health, Leukaemia & Lymphoma Research, CHILDREN with CANCER, Great Ormond Street Hospital Children's Charity, and the JP Moulton Charitable Foundation."

The team have now begun treating children at risk of relapse from 10 participating hospitals, across four countries. Professor Amrolia hopes that if this treatment can be shown to be safe and effective, it might cure children with otherwise untreatable leukaemia, and open up an entirely new approach to combating high-risk paediatric cancer.

Building a foundation for healthy lungs

In order to know when a child is sick, it is essential to have reliable indicators of what is normal when they are healthy. Dr Sooky Lum has the challenging task of designing studies that involve healthy children in research that underpins the vital work of the Portex Respiratory Unit at the UCL Institute of Child Health and Great Ormond Street Hospital.

Dr Sooky Lum

"Following an early career in midwifery, my first foray into respiratory physiological research was studying healthy infants soon after birth, to investigate how low birth weight influences subsequent lung function. The success of this study and follow-up assessments of these children through the preschool years means that we now have one of the largest pools of lung function data in healthy children from birth to school age. Without this information, the interpretation of lung function results from children with lung disease would be extremely difficult.

"I also played a major role in the initial organisation of the Asthma UK funded project which resulted in the first 'all-age' growth charts for spirometry for the white population. However, these charts are not appropriate for children of other ethnic origins due, at least in part, to differences in physique between ethnic groups. Increasing awareness of the ethnic variations that exist within some lung diseases emphasised the urgent need to develop growth charts for lung function that could be applied across all ethnic groups, leading to the current Size and Lung Function in Children (SLIC) study."

Impaired lung function is a marker of diseases ranging from the relatively rare but potentially extremely severe – cystic fibrosis, which affects around 9,000 people in the UK – to more common conditions like asthma, which affects several million.

Dr Sooky Lum has been involved in lung function research for the last 15 years. Her focus has been how best to capture information indicative of an underlying problem in a child's lungs, in particular during their early years of life.

"We know that we need to preserve early lung function as much as possible in children with lung disease, to delay any subsequent deterioration and avoid long-term damage," she observes. "In the last decade, we've made great progress. With sensitive equipment at our disposal, we can now obtain lung function results from birth, right through to adulthood. This includes assessing children during the challenging preschool years, at one time thought to be an impossible task due to the difficulty in measuring the lung function of this young age group." During this time, the team also recruited over three hundred healthy children to act as controls for their various research and clinical studies.

With funding from Asthma UK, the team developed a unique resource by collating their lung function data with that of international centres across the world. They produced a validated series of growth charts which modelled individuals' respiratory health, taking into account age and body size, from the ages of three to 80 years.

"Building rapport with these children and their families has been crucial," says Dr Lum. "We have children previously recruited to our research studies as controls, asking their parents: 'When can I go back to help the sick children in the hospital?' As they grow into their teenage years, we've continued involving these children in our current research. Their contribution has been invaluable."

Despite these advances, growth charts appropriate for groups other than white Europeans are lacking, especially for younger children. "As ethnic minorities make up 40 per cent of the London population, this has a direct impact on health provision," says Dr Lum. "Sickle cell disease, rather than cystic fibrosis, is now the most common inherited disease in London."

The team is now tackling this further challenge, aiming to develop improved growth charts that will take into account differences in body physique and be applied to all children, irrespective of ethnic background. With funding from the Wellcome Trust, they are assessing 1,600 primary school goers, in the Size and Lung Function in Children (SLIC) study. Dr Lum believes that if they can quantify and adjust for the unique body physique of each child when interpreting lung function, the need for ethnically-specific reference equations will no longer be necessary.

"The conditions we treat at the hospital affect children of all ethnicities," says Dr Lum. "With this research, we hope to improve our methods of interpreting a child's respiratory health, and better refine our treatments based on validated information which doesn't require us to categorise children by ethnicity. The people I've been fortunate enough to involve in this work really seem to understand what we're attempting to do – I hope this can continue and that we see real benefit from our work as a result."

Karen Giles
Head Teacher at Barham Primary School

"When we were approached by Dr Sooky Lum, I immediately recognised the relevance and importance of this research. Some of our pupils, staff and parents have asthma and other respiratory issues and I would like to think that they would be able to receive treatment specific to their unique characteristics.

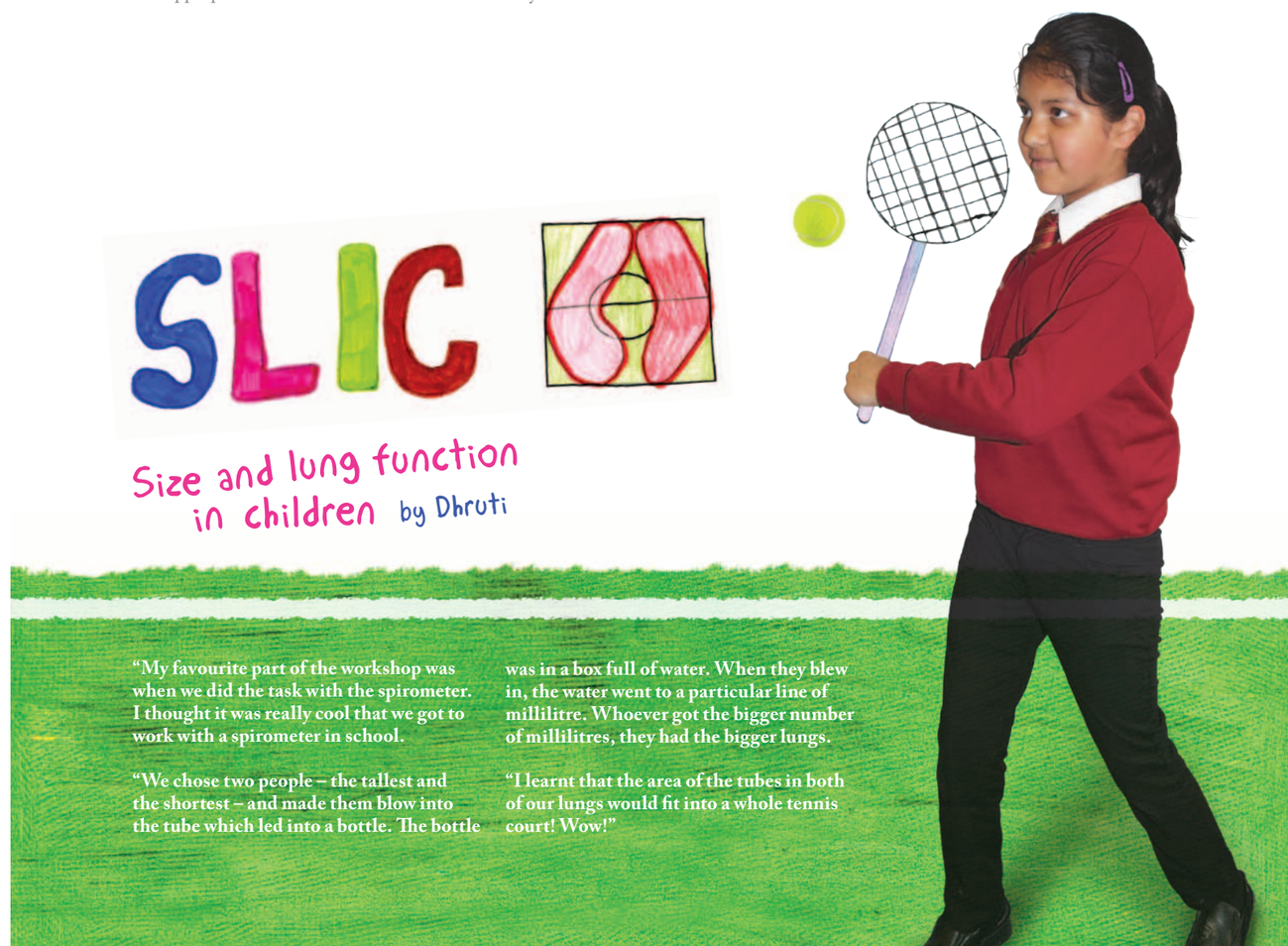
"I was impressed with how Dr Lum and her team sought to educate through the science workshops in exchange for our time and resources, as well as thinking through the provision of incentives and rewards to maximise participation. The science workshops were very well received, particularly as they were interactive and hands on. This style of learning promotes deeper and more profound learning.

"Communication to parents and our diverse local community via the local press, in both English and Gujarati, was comprehensive and appropriate.

"It is important for our pupils to get involved in a study for them, by them. One hundred per cent of our 720 pupils aged three to 11 years are from black and ethnic minority backgrounds, predominantly Indian, Sri Lankan, Pakistani, Somali, West African, African Caribbean, Eastern European and interesting mixes. We are the 'global majority' as the world comes to Barham.

"I want our children and wider community to support research, see research methods demystified, trust and therefore not be afraid of contributing. They are all potential scientists and will themselves be conducting various scales of research at university in the near future. Past and present pupils and children of staff members have been treated and cared for at Great Ormond Street Hospital, and this is our way of giving back. I like the idea that this is a longitudinal study and could also potentially pick up previously undiagnosed issues a child may have."

Involving healthy children like Dhruti in research helps to underpin important work to improve the lives of sick patients, as well as engage the next generation of young scientists.



Pioneering treatments that use the body's own ability to heal

One in 33 children in the UK is born with a birth defect, the primary cause of infant mortality in the Western world. If these diseases cause organ damage or failure, the global donor shortage makes relying on a transplant unrealistic. Research by Dr Paolo De Coppi is pioneering curative treatments made from a child's own living tissue.

Dr Paolo De Coppi

"I initially trained as a paediatric surgeon in Italy. My research interest arose from a frustration about not being able to do enough for children born with missing tissues or organs. We used transplantation as a bridge, or implanted prosthetics that mimicked tissues functionally, but could not grow with the child.

"I became interested in regenerative medicine – using cell therapy and tissue engineering to develop curative treatments – when I went to Boston to learn about the techniques they were developing. Today, I'm privileged to combine that with the work of leading researchers from across University College London, and the many paediatric specialties at Great Ormond Street Hospital.

"Children and newborns are an ideal population for regenerative medicine, as transplanting a relatively small amount of functional tissue could completely change their quality of life. I'm working to bring together clinical specialists with laboratory experts in stem cells and polymer science who can solve problems and speed up the process of developing new therapies.

"Ultimately, our aim is to generate transplantable tissues – trachea, oesophagus, diaphragm – for birth defects diagnosed prenatally. But we're working at the edge of clinical experimentation."

Our researchers are developing new ways of treating donated healthy tissue (above). Their aim is to remove donor cells, but leave in place the natural support structure for seeding a patient's own stem cells (bottom left vs. right).

One of the more serious and life-threatening birth defects treated at Great Ormond Street Hospital (GOSH) is tracheal stenosis – a potentially fatal narrowing of the windpipe which severely restricts airflow to the lungs. Compared to running a sprint while attempting to breathe through a thin straw, children require complex surgery to allow them to breathe normally. However, often there is simply not enough tissue present in infants' tiny windpipes for surgeons to reattach the ends after removing the critically narrow section. Artificial stents inserted to hold the windpipe open are prone to fail, with lethal consequences.

Dr Paolo De Coppi, Clinical Senior Lecturer at the UCL Institute of Child Health and Consultant Paediatric Surgeon at GOSH, continually sees the devastating effects of multiple reconstructive operations aimed at replacing a child's damaged organs, or following organ transplantation. Working as part of a multidisciplinary team, he is at the forefront of research into regenerative medicine.

This emerging field combines advances in gene therapy, biomaterials and nano-technology in an attempt to mimic the human body's complex growth and healing pathways. Dr De Coppi's ambitious vision is to build transplantable organs from a patient's own cells, avoiding the use of immunosuppressant drugs that are associated with a high risk of infection and tumour formation.

"Over the last decade, with Professors Martin Elliott and Martin Birchall, we began to experiment with transplanting donated tracheas," says Dr De Coppi. "Initially, to avoid rejection, we had to strip off the donor's cells, reducing the structure to just a supportive collagen scaffold. The therapy allowed us to establish Europe's only specialised airway clinic. Nevertheless, the technique only had a 65 per cent 12-year survival rate. We had to find a way of moving this on."

This they did when, in 2010, Ciaran Finn-Lynch became the first child in the world to undergo a pioneering new procedure. The team took stem cells from his bone marrow and incubated them with a de-cellularised windpipe along with growth signalling molecules. This engineered organ was then implanted successfully, with the stem cells forming an airway lining, and triggering growth of a functional blood supply to the trachea. Following a lengthy recovery, Ciaran has since returned home.

With the support of the Royal College of Surgeons, the Newlife Foundation, and Great Ormond Street Hospital Children's Charity, Dr De Coppi is expanding his work into exciting new areas, to apply advances in regenerative medicine to the clinical treatment of children with rare diseases such as heart defects, diaphragmatic hernia, and gut and neuromuscular diseases.

"Our research has found ways to isolate stem cells from a sample of amniotic fluid surrounding the growing infant in their mother's womb," he explains. "We've shown that these cells can be transformed into pluripotent cells, capable of growing into any tissue in the body. Our aim is to use these stem cells alongside the latest developments in nanotechnology, to design and grow personalised replacement tissues for children who urgently need surgery at the point they're born, or whose organs are failing."

Dr De Coppi is now working closely with colleagues across the UCL Centre for Stem Cells and Regenerative Medicine to deliver clinical trials which, if successful, could have far-reaching implications. "My hope is that we might build complex organs that not only function, but that can grow with children, eliminating any need for organ donors. We've got a patient population that really needs these alternatives urgently, if we're to offer them long-term quality of life."

From an unknown disorder to fundamental advances in science

Mitochondria are the tiny compartments present in most cells that generate the energy needed to sustain life. Mitochondrial diseases at their severest can be both untreatable and fatal. Following 20 years of determined work, Dr Shamima Rahman has led a sequence of discoveries, giving families hope and advancing our understanding of fundamental questions in human biology.

Dr Shamima Rahman

"My interest in mitochondrial disorders was kindled just over 20 years ago when I first joined the metabolic team at Great Ormond Street Hospital. The first genetic causes of mitochondrial disease had been discovered at the UCL Institute of Neurology just a couple of years before. It was immediately obvious to me that this was an extremely important subgroup of inborn errors of metabolism. The challenges, both then and now, are to provide accurate and prompt diagnoses, and to develop effective treatments.

"The last few years have witnessed great advances in genetic diagnosis for mitochondrial diseases, with the discovery of more than 100 disease genes, but it is likely that several hundred more genes will be linked to mitochondrial disease in the future. The funding we have received for exome sequence analysis will hopefully help to identify these genes, leading to improved genetic diagnosis for affected families.

"Our long-term goal is to translate this genetic knowledge into curative treatments for mitochondrial disease, something that several members of my team are currently working on."

When Dr Shamima Rahman first began researching mitochondrial diseases in 1991, little was known about their causes. She began by looking into one of the rarest and most severe types, Leigh syndrome. Usually affecting infants, it causes progressive and rapid brain and nerve damage.

"I started by devising a classification system for a complete set of 100 or more cases of Leigh syndrome recorded at the time," says Dr Rahman, Great Ormond Street Hospital Children's Charity Reader in Paediatric Metabolic Medicine at the UCL Institute of Child Health (ICH). "But the more I investigated, the more confused I became. The problem was the variety of different forms of the disease, even within this small number of patients.

"The situation families faced was pretty bleak," she says. "When I started working on these diseases, we could only make a genetic diagnosis in perhaps five per cent of cases. I could tell parents their child had a mitochondrial disease, but had no idea of whether their future siblings would inherit it, or what the root cause of the disease was. Finding the genetic origin of the condition could be a year away, 10 years away – or never. The families' willingness to help us progress with the research has been crucial."

This support, along with the increasing availability of gene-screening platform technologies, has allowed Dr Rahman to take huge strides in the intervening years. Her team has identified several disease genes, now known to be responsible for building some of the largest and most complex proteins in the human body, located within the mitochondrion's inner structure. Principally, they relate to a family of five enzymes, complexes I–V, which form a vital part of the mitochondrion's energy production cycle.

"I have a unique role in which I'm a paediatrician seeing patients, while leading a research group with access to specialists in exome sequencing, bioinformatics, and biochemistry. This could only be possible at the ICH and Great Ormond Street Hospital," she says. "Taking complex I mitochondrial diseases as an example, we've been able to identify the genetic basis of half of all the cases we see."

The impact of this work is profound. Parents can now receive counselling to inform them of the exact likelihood of passing any disease-causing gene to subsequent children. They can opt to have pre-implantation genetic diagnosis, should they wish to mitigate this risk. Furthermore, genetic information is revealing the precise workings of the mitochondrial complex enzymes: the key to addressing their failure in the disease process. In one case, patients can now be treated with vitamin B2 to reduce progression of their symptoms – something unthinkable 20 years earlier.

With support from Great Ormond Street Hospital Children's Charity, Dr Rahman is now embarking on further research to close the knowledge gap for the remaining forms of complex I mitochondrial diseases.

"Success in this research can be bittersweet," reflects Dr Rahman, "as the nature of these conditions often means there's still little we can do by way of treatment. But our work has begun to reveal how one of the most ubiquitous, yet remarkably poorly understood structures within our bodies, functions. The patients I see are helping us to fill in the bigger picture – I've every intention of finding new and better ways to treat their disease."

Callum's story

by his mum, Heather

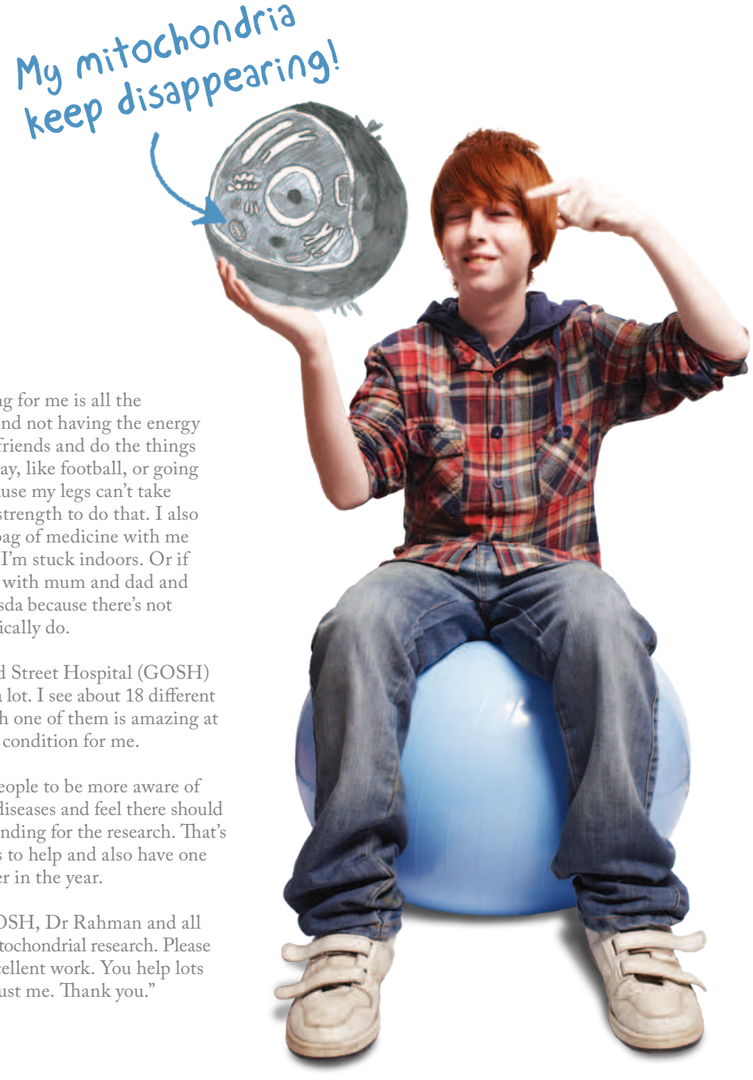
"Callum had always been ill since birth; in and out of hospital, with problems feeding, digesting, blackouts, and bad nasal bleeds. He was diagnosed with epilepsy aged seven. Then his weight kept going up and down along with his appetite.

"Later, we took Callum for an eye test, and were told he needed to see an ophthalmologist at our local hospital. He couldn't find any cause for his eye problems and referred us to Great Ormond Street Hospital (GOSH). After just our second appointment and a blood and urine test, Dr Rahman had diagnosed Callum with a mitochondrial DNA deletion with Kearns Sayre syndrome.

"This was a huge blow for us as parents and we were very unsure of how to deal with the diagnosis or what to expect. Since diagnosis, Callum has become less capable of doing day-to-day things for himself, and he and our family take each day as it comes.

"Callum has remained strong and hopeful that research may lead to some cure or help for all who have similar mitochondrial DNA deletion syndromes. With this in mind, he has managed to raise over £3,000 for mitochondrial research in the last three years, through fun days, raffles, and auctions.

"GOSH has been a godsend to us as without the specialists, we would never have known what Callum had. The whole team have been amazing and it is such a great hospital to be at for the care of your child."



Callum is a regular fundraiser in his home town of Reading and hopes to raise awareness of mitochondrial disorders and research to help other children with these rare diseases.

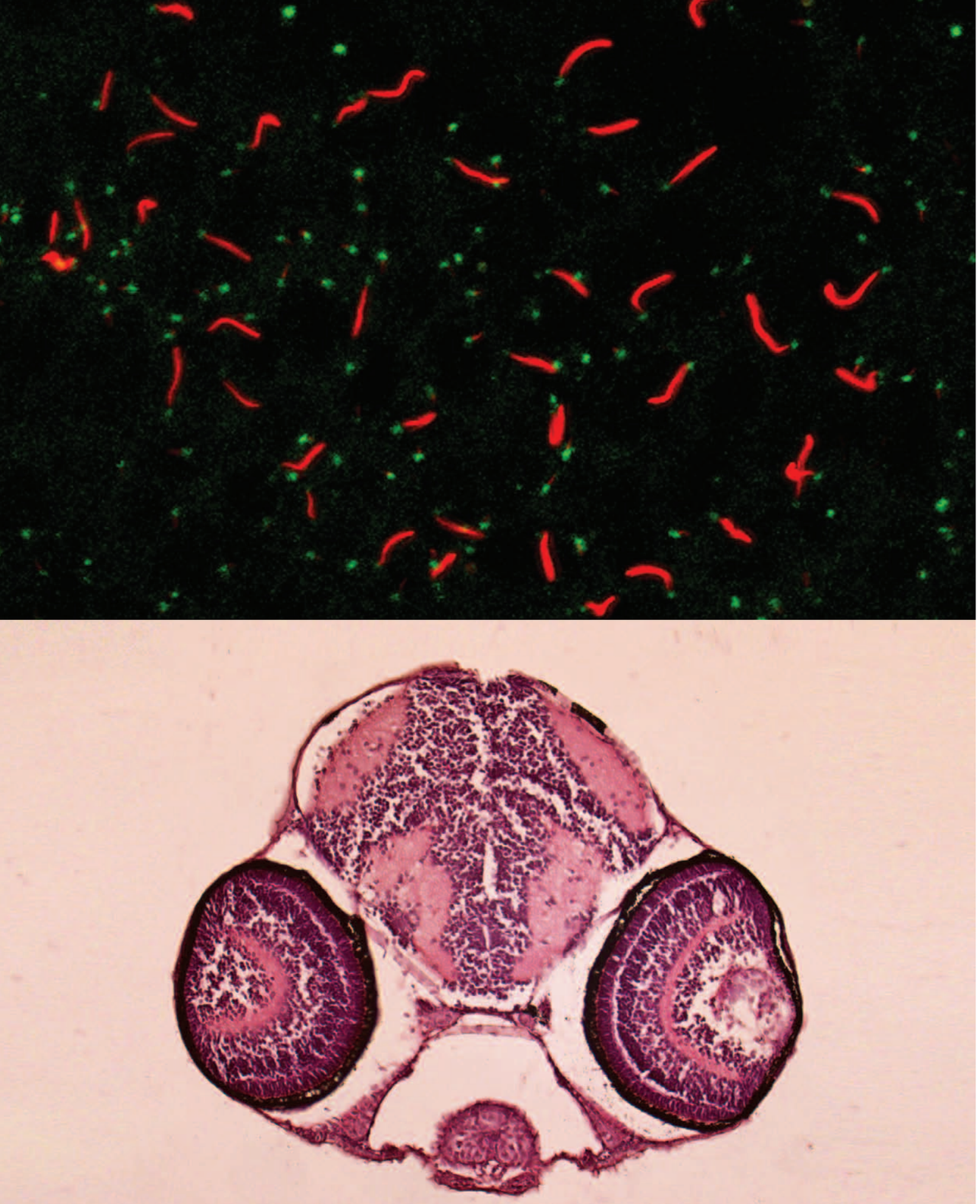
by Callum

"The worst thing for me is all the appointments and not having the energy to go out with friends and do the things they do every day, like football, or going into town, because my legs can't take the amount of strength to do that. I also have to take a bag of medicine with me everywhere, so I'm stuck indoors. Or if I do go out, it's with mum and dad and only as far as Asda because there's not a lot I can physically do.

"Great Ormond Street Hospital (GOSH) has helped me a lot. I see about 18 different doctors but each one of them is amazing at monitoring my condition for me.

"I would like people to be more aware of mitochondrial diseases and feel there should be a lot more funding for the research. That's why I do events to help and also have one planned for later in the year.

"Thank you GOSH, Dr Rahman and all who work in mitochondrial research. Please keep up the excellent work. You help lots of people, not just me. Thank you."



Finding genes, transforming therapies

There are at least 6,000 rare diseases, many of which remain a relative mystery to modern medicine. Taken together, these diseases affect one in 17 people in the UK. Using advanced DNA screening techniques to find faulty genes, researchers at Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (ICH) are translating these findings into new diagnostics and treatments.

Professor Phil Beales

"I can ascribe the current state of my research to two key episodes in my career. The first, serendipitous event occurred as a junior doctor in training when one evening I was called to accident and emergency to resuscitate a man in a diabetic coma who I came to realise had the rare condition, Bardet-Biedl syndrome (BBS). I was at that time developing my early research interests in the genetic causation of insulin resistance and diabetes. The study of this Mendelian disease (BBS), so I believed, presented an opportunistic and unique entry point into the study of more common afflictions.

"The second, more recent event came through my observation that several patients in the BBS register I compiled, had situs inversus (reversal of internal organ position), a developmental defect that can arise from abnormal cilia beating during early embryogenesis. This led to the recognition and discovery of cilia dysfunction underlying BBS and subsequently many other ciliopathies.

"Isaac Newton once said, 'If I have ever made any valuable discoveries, it has been owing more to patient attention than to any other talent'. This is as valid a statement today as it ever was."

From the workings of tiny organs like cilia (above), to model organisms like the zebrafish (below), GOSgene is revealing vital information to improve treatments for today's patients.

"The technology we now have at our disposal to look at a patient's entire DNA code simply didn't exist even three years ago," says Phil Beales, Wellcome Trust Senior Research Fellow and Professor of Medical and Molecular Genetics at the ICH.

"We can now obtain a readout of the DNA sequence of all of the protein-coding regions of a patient's genome for around £1,000 – but there's still a critical bottleneck in applying this information. Put simply, we do not know what most of these genes do to account for many of the diseases our patients have."

Professor Beales leads a unique initiative called GOSgene, established with support from the National Institute of Health Research Biomedical Research Centre at GOSH and the ICH. GOSgene aims to bring together clinicians and dedicated in-house research staff to acquire and share data on disease-causing genetic mutations, and rapidly apply this to design diagnostic tests and develop new therapies.

"We host more national specialised clinical services than any other hospital in the UK," says Professor Beales. "This means we see children with rare diseases in larger numbers and collaborate to share clinical knowledge. Combining clinical and genetic information means we can link up cases where a faulty gene leads to a defined syndrome and begin to understand the causes of disease, as well as its longer-term consequences for a child's health."

The GOSgene platform combines a number of high-throughput techniques to help clinicians determine which genetic mutations are most likely to give rise to disease. A second laboratory adopts a number of approaches to explore the function of proteins coded for by these genes. One method uses morpholino technology in zebrafish embryos – where a sequence of artificially generated DNA-like code is used to block the action of a gene.

The initiative has already generated landmark successes. In one example, researchers have identified the genetic cause of seven previously uncharacterised diseases related to the tiny 'hairs' found on the surface of the body's cells, called cilia. These so-called ciliopathies can give rise to severe birth defects of the eye, kidney, brain and bones.

To further translate this knowledge, Professor Beales' laboratory has developed a screening system in fish, to test an array of over 1,000 drugs, already approved and licenced for use in humans.

"By using existing drugs in new and sometimes better ways, we can offer a short-cut to the lengthy and expensive process of designing new drugs," comments Professor Beales. "Looking at the ciliopathies, we've already found several target drugs that we hope might reduce the kidney damage associated with ciliopathies."

Professor Beales' ultimate goal is a uniquely streamlined service where children with diseases of unknown cause have their genome sequenced and professionally analysed, with important and unique biological information fed back to guide treatment and counselling directly in the clinic.

"We need to make full use of the vital information we hold," says Professor Beales. "From our combined expertise and detailed patient records, to the often unique clinical symptoms our patients display. Through careful listening, observation and examination, we can learn much from our patients that together with their unique genetic information can be turned toward better clinical care. Such approaches form a crucial part of our position as a world-leading research centre."

Bringing neuromuscular therapies to market

Neuromuscular disorders cause the breakdown of muscle and nerve tissue. They can occur from birth or develop later in life, and be either stable or degenerative. Symptoms range from almost undetectable, to the progressive loss of muscle function, severe disability, paralysis and death. Professor Francesco Muntoni is pioneering new therapies to help children with these often devastating conditions.

Professor Francesco Muntoni

"My interest in children with neuromuscular disorders started when I was training in paediatric neurology and psychiatry in Italy. Little was known at the time. We did not even understand which gene caused conditions such as Duchenne muscular dystrophy.

"My move to London in the early 90s coincided with the first steps in understanding the molecular bases of these conditions. My combined clinical training, genetic laboratory and pathology expertise opened up a fascinating field of clinical research. I had a fair understanding of the disease process of individual conditions and used this knowledge to map specific milestones in the disease and improve anticipatory care.

"I have been involved in the discovery of more than 20 neuromuscular disease genes, several in my laboratories. In the last few years, I have concentrated on harnessing knowledge of basic disease processes to develop novel therapeutic interventions.

"The environment at Great Ormond Street Hospital, with its state-of-the-art Biomedical Research Centre and Somers Clinical Research Facility, makes it one of the most exciting places in the world to pursue these interests."

Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (ICH) form one of four nationally-commissioned centres for individuals affected by rare neuromuscular disorders, and this is the only centre dedicated to children with congenital muscular dystrophies and myopathies. Every year, GOSH treats more than 1,600 patients with these challenging muscle and nerve-wasting diseases.

"Just over five years ago, there were no new experimental therapies in development for muscular dystrophies," reflects Professor Muntoni, director of the GOSH/ICH Dubowitz Neuromuscular Centre. "Since then, we've had successes in identifying many more genes involved in neuromuscular diseases, what these genes do and how we can harness this knowledge to improve the lives of children."

Momentum in this area is certainly building. Professor Muntoni's team has identified over 20 disease-causing genes and pioneered clinical trials of new therapies – especially in Duchenne muscular dystrophy. These range from highly innovative first in man studies of new treatments, to examining how well a known treatment works in a new condition.

Last year, the team announced a breakthrough success in using antisense oligonucleotides – a genetic-based technology – to skip mis-spelled regions of diseased DNA and restore production of the vital missing protein, dystrophin, in the muscles of patients with one form of Duchenne muscular dystrophy.

"Our aim was to advance experimental therapy in Duchenne," says Professor Muntoni. "Via a consortium – MDEX – we found which antisense oligonucleotide molecules could achieve our aim and, with funding from the Department of Health and the Medical Research Council, performed a series of early clinical trials. However, there's only so far we can go with the initial work to develop these drugs. The next step has to be in the hands of the drug companies themselves, who need to fulfil the regulatory path to bring these therapies to wider clinical practice as medicinal products."

Professor Muntoni is still very much involved in this work, both in the design of future phase III trials to test whether these drugs can bring about improvements in children's symptoms, and also in developing entirely new antisense oligonucleotides. The immensely detailed clinical and academic knowledge of neuromuscular diseases he and his team have built up is being used in a variety of ways: from designing improved methods of assessing how well the drugs work, to determining which patients ought to be included in trials, and even whether there are improved ways of delivering the drugs to target children's muscles more effectively. And this for just one of a spectrum of muscular dystrophies, each urgently requiring new therapies.

Professor Muntoni remains cautiously confident that his work will bear fruit. "As a clinician, I've seen our knowledge applied to anticipate the problems these children face, and help them survive from childhood to adolescence, and on to become productive adults," he says.

"We're going in the right direction. If the next phase of our trials works, it would change the entire field of neuromuscular medicine. But it's a tall order. I'm privileged to work in an environment where we might be able to address some of the fundamental issues of these diseases – and to work with children and families to overcome the limitations of these conditions and improve therapies as best we can. I'd love to see a new drug developed from my research, out there in clinical practice and saving lives. In the meantime, it's incredibly rewarding just to be doing something useful."

Demi's story

by her mum, Tracy

"Demi was born six days overdue, a happy and healthy baby girl weighing seven pounds. We could not have been happier as a family. She crawled when she was six months old and walked when she was nine months old. Things began to change when Demi was 18 months old; she began to fall over frequently, she stumbled when she walked and bumped into door frames and walls. Since then, she has been diagnosed with sensory hearing loss and wears hearing aids in both ears. She is vision impaired and also lost the use of her upper limbs three years ago.

"She spent nine years undergoing tests at many different hospitals in Scotland and England. At the time we were referred to Great Ormond Street Hospital (GOSH) a few years ago, we were still no further forward in having a diagnosis for her condition.

"I was not expecting to have such a positive outcome. GOSH's doctors have been absolutely amazing in helping and supporting us. Demi is finally on medication which is proving to have a positive effect on her as we have seen some small improvements in such a short period of time.

"She relies heavily on us as her parents (and younger brother Reece) to do everything for her. But Demi takes it all in her stride and feels no self pity. Everybody who is lucky enough to meet her will know that she just accepts what is and adapts to everyday life in her own way. Demi is an amazing artist; she draws and plays on her Nintendo DS using her mouth, and has created some fantastic art work on this too.

"As a family, we will be eternally grateful to GOSH for everything they have done and continue to do for us and Demi."

Demi's condition remained a mystery until Professor Muntoni's team discovered a faulty gene affecting her neuromuscular system. She is now receiving targeted therapy to improve her symptoms, and enjoys painting and drawing with her mouth!

Giving communities a voice in international healthcare

In the developing world, the most common point at which a child dies is soon after birth. Dr David Osrin is combining community-based outreach with models of social research, to improve the lives of women and children in some of the world's most deprived rural and urban areas.

Dr David Osrin

"I always wanted to be involved in research in other countries, but research that was close to people. Illness, social aspects of disease, human health – these have always been key foci.

"When I first went to Nepal in 1998, working with Professor Anthony Costello's team, our main focus was on the survival of newborn babies. At the time, this was a relatively invisible health area.

"Over the past 10–15 years, I've learned something big. If you keep saying that 'something's unknown', how are you going to change policy? With enough people trying to raise attention, change can begin to happen. Nepal now has a national newborn health policy."

Dekha Undekha (Seen Unseen) was a multimedia collaboration between slum-dwelling artists, gallery artists and health experts in Mumbai, India. Supported by The Wellcome Trust, the project led to an exhibition in Dharavi, Asia's most well-known slum.

"Improving the health of women and children in low-income countries isn't about 'intensive care' as we know it in Western models of healthcare – it's much simpler than that," explains Dr David Osrin. For most of the year, he works in India as a Wellcome Trust Senior Research Fellow in Clinical Science, affiliated to the Centre for International Health and Development at the UCL Institute of Child Health (ICH). "You can do quite basic things such as breastfeeding your baby immediately after birth, keeping your baby close to you, keeping things clean and loving your baby. The outcomes of these social behaviours have medical consequences."

Dr Osrin's early research formed part of a joint initiative led by Professors Anthony Costello at the ICH and Dharma Manandhar in Nepal, under the banner of Mother and Infant Research Activities (MIRA). This group took on the challenge of improving the survival of newborn babies through a large trial in the rural Nepalese district of Makwanpur, using a somewhat novel intervention: groups of women talking to each other.

"At the time, there was scepticism whether women's groups would be a suitable vehicle for reducing infant mortality," says Dr Osrin. "But MIRA saw newborn survival increase by 30 per cent. These unconventional social interventions, which involve mobilising women to take ownership of their health, can achieve significant results."

These trials have since been replicated, with similar successes in rural Bangladesh, India and Malawi. Recently, Dr Osrin has been working with the Society for Nutrition, Education and Health Action (SNEHA) in the slums of Mumbai. It became obvious that the additional stressors of the urban environment required the team to take into account other factors, over and above newborn survival, to further their aims of improving women's and children's health.

"We wanted to continue working with women's groups and communities," explains Dr Osrin. "However, we appreciated the need to engage municipal corporations, governmental agencies and public health services, as well as to broaden our remit to address childhood nutrition, violence against women, and sexual, reproductive and mental health. In other words, be more holistic in our approach."

In the Mumbai project, information and outreach is provided to families via community workers based at a SNEHA resource centre – a nodal point within the slum similar to a Citizens Advice Bureau. The teams then measure their success by looking at a range of health indicators. These include giving birth in a hospital, family planning, better child growth, and support for domestic violence. Communities benefit from the answers to the research questions being proposed, while an evidence base develops for future interventions.

Dr Osrin explains the methods underpinning this: "We've fused research elements into this study by setting up SNEHA resource centres in 20 target areas, as part of a cluster-randomised trial. We then carry out simple surveys – a census taken initially, and then after two years. School leavers with Android phones collect and transmit the data in real-time. In this particular project, we're reaching out to over 100,000 people, whose lives we hope to demonstrably improve."

By taking on this work, Dr Osrin and his colleagues are confronting challenging issues. These include the delivery of global healthcare, the ethics of participatory research in complex societies, and how to deliver sustainable and effective aid. In a world where science increasingly competes with political and social agendas, it is encouraging that the research models he and his colleagues are applying have the potential to unite communities, as well as national policymakers, in their efforts to improve people's lives.

Clinical tests that are appropriate for children and acceptable for families lie at the heart of research to improve today's treatments and tomorrow's cures.

Our business is improving patients' lives; uniting specialists who understand children's medical problems with core experts at the forefront of scientific research.

Working with UCL Business PLC (UCLB)

UCLB is responsible for the management and exploitation of intellectual property arising from both the UCL Institute of Child Health (ICH) and Great Ormond Street Hospital (GOSH).

Through the protection of intellectual property, proof of concept funding, leverage of translational grant funding and partnership with industry, our work at UCLB aims to maximise the positive social, health and economic benefits of ICH and GOSH discoveries. We have continued to see an upward trend in activity and interest in leverage of intellectual property for the translation and dissemination of ICH and GOSH research and clinical practice. Most notably, the number of ideas submitted to UCLB through the invention disclosure process has almost doubled from the previous year. Patent filings have been submitted for several of these and we look forward to supporting the development of these new approaches over the coming months and years.

A particularly successful commercial partnership has been an ongoing clinical collaboration between Abbott and Professor Atul Singhal, exploring the effects of early nutrient intake on growth and body composition. The study has achieved an interim milestone. The ICH continues to work closely with Abbott to develop an infant formula which matches infants' nutritional requirements during their first weeks of life. The team hope that by mimicking the initial period of natural weight loss which occurs after birth when breastfeeding, infants will have a reduced risk of obesity as they grow into childhood.

New patents have been filed for a number of projects, many of which have received proof of concept funding from UCLB. This funding supports the essential work required to develop and transform an idea into a proven innovation, demonstrating performance and confirming suitability for commercialisation and leverage of translational funding.

As part of the work of the Dubowitz Neuromuscular Centre, Professor Francesco Muntoni and collaborators at University of Western Australia have filed a patent for an improved antisense oligonucleotide approach for the treatment of spinal muscular atrophy.

Exploring ways to prevent birth defects, Dr Nick Greene and Professor Andrew Copp have discovered novel supplements that, if taken in pregnancy, can reduce the chance of the baby having neural tube defects such as spina bifida or anencephaly. These are important in pregnancies where folic acid and other supplements are not as effective. A new patent has been filed and through our proof of concept funding we are supporting work to validate and test combination in animal models.

Two new patents have been filed by Professor Tessa Crompton and colleagues, who have discovered that modulating the hedgehog signalling family proteins has implications for certain cancers and inflammatory diseases.

With support from Vitaflo, Professor Simon Heales and colleagues have filed a patent having developed new dietary compositions with the potential to control epilepsy seizures.

Finally, Dr Steve Hart and colleagues have established a new spinout company – Nanogenic Solutions Ltd – based on their lipid-peptide system for targetted drug delivery and associated patents.

Looking ahead, UCLB and the specialist National Institute of Health Research Biomedical Research Centre at GOSH and ICH have recently approved an exciting new joint proof of concept fund, which focuses only on funding innovations developed by local paediatric researchers and clinicians. We hope that this fund will help support the upward trend in activity and interest in leverage of intellectual property for the translation and dissemination of the exceptional research and clinical practice at GOSH and the ICH.

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UCLB Business

Awards, honours and prizes

Staff from the UCL Institute of Child Health and Great Ormond Street Hospital received national and international recognition for their research achievements during 2011/12.

Misbah Arif was awarded an MPhil for her thesis, *The expression of Trp channels responsive to noxious and innocuous temperatures in the developing mouse embryo.*

Indrani Bandyopadhyay was awarded an MD(Res).

The Ekjut trial in Jharkhand and Orissa, led by Dr Prasanta Tripathy and Dr Nirmala Nair of Ekjut, in collaboration with **Dr Sarah Barnett, Professor Anthony Costello** and **Dr Audrey Prost,** was awarded the Trial of the Year Award by the Society for Clinical Trials.

Professor Phil Beales was elected a Fellow of the Academy of Medical Sciences for his contributions to human genetics and birth defects research.

Dr Chiara Beilin was awarded a PhD for her thesis, *Common cytokine receptor y chain deficiency in dendritic cells: implications for immunity.*

Giovanni Biglino was awarded a Bogue Research Fellowship. He also won the prize for best poster at the 2nd International Conference on Engineering Frontiers in Congenital Heart Diseases, held in London in March 2011.

Dr Gerben Bouma was the first prize winner in the poster competition at the UCL Centre for Immunodeficiency's 4th One-Day Symposium on Primary Immunodeficiency: Enhancing Immune Function.

Jonathan Bull was awarded an MD(Res).

Dr Sandra Castro was awarded a PhD for her thesis, *Proteomic and molecular analysis of neural tube defects in the mouse embryo.*

Dr Owen Clark was awarded a PhD for his thesis, *The role of protein tyrosine phosphates in neuroblastoma.*

Dr Emma Clement was awarded an MD(Res) for her thesis, *Congenital muscular dystrophy in 2010.*

Dr Jonathan Cohen was awarded a PhD for his thesis, *Colonisation-induced protection against Streptococcus pneumonia disease.*

Dr Jane Collins, Chief Executive of Great Ormond Street Hospital for Children NHS Foundation Trust, was awarded a University College London Honorary Fellowship.

Professor Andrew Copp was elected a Fellow of the Society of Biology and was appointed to the Medical Research Council Career Development Awards and Senior Non-Clinical Fellowships Panel.

Dr Mario Cortina Borja was appointed statistical editor of *The Psychiatrist.*

Daria Cosentino won the prize for best oral presentation at the 2nd International Conference on Engineering Frontiers in Congenital Heart Diseases, held in London in March 2011.

Professor Anthony Costello was awarded the James Spence Medal, the highest honour of the Royal College of Paediatrics and Child Health, for outstanding contributions to paediatrics and child health.

Dr Elin Davies was awarded a PhD for her thesis, *Developing markers of neurological manifestations in neuronopathic Gaucher disease.*

Dr Simon Eaton received the award for most valued lecture of 2010 from the London Mass Spectrometry Discussion Group.

Dr Christin Eltze was awarded an MD(Res) for his thesis, *Epilepsy in infancy study: a population based study on epilepsies with onset in the first two years of life.*

Dr Shiuh-Bin Fang was awarded a PhD for his thesis, *Early interactions of non-typhoidal Salmonella with intestinal epithelium.*

Dr Bruno Ferraz de Souza was awarded a PhD for his thesis, *Analysis of novel steroidogenic factor-1 targets in the human adrenal gland.*

Dr Jonathan Fishman was awarded a Research Training Fellowship from Sparks (Sport Aiding Medical Research for Kids) for his PhD project, *A tissue-engineered approach to cranial neuromuscular regeneration.* He was also awarded a Medical Research Council Clinical Research Training Fellowship.

Dr Lucy Freem was awarded a PhD for her thesis, *The development of the neural crest-derived intrinsic innervation of the lung.*

Professor David Gadian was invited to join the Expert Review Group of the Technology Transfer Division of the Wellcome Trust. He was also awarded an Honorary Professorship by the School of Physics and Astronomy at the University of Nottingham.

Professor Ruth Gilbert was appointed Chair of the Medicines for Children Research Network Methodology Clinical Studies Group.

Dr Paul Gissen was awarded a Wellcome Trust Senior Clinical Research Fellowship for his project, *The role of two key intracellular trafficking genes, VPS33B and VIPAR, in development and disease.*

Dr Anna-Lise Goddings was awarded a Medical Research Council Clinical Research Training Fellowship.

The National Institute for Health Research (NIHR) awarded the NIHR Biomedical Research Centre (BRC) at Great Ormond Street Hospital for Children NHS Foundation Trust and UCL Institute of Child Health £36 million to support research for the next five years. The BRC is led by **Professor David Goldblatt,** alongside **Professor Bobby Gaspar, Professor Phil Beales, Professor Francesco Muntoni, Professor Adrian Thrasher** and **Professor Neil Sebire.** It is managed by **Dr Julian Hughes,** and **Dr Rachel Joynes** contributed to the success of the application.

Awards, honours and prizes
continued

Dr Astrida Grigulis was awarded a PhD for her thesis, *Lives of Malawian nurses: stories behind the statistics.*

Dr Mike Grocott was awarded the BOC Chair of Anaesthesia award of the Royal College of Anaesthetists.

Dr Yeong-Lih Hiew was awarded a PhD for her thesis, *Examining the biological consequences of DNA damage caused by irradiated 3T3-J2 fibroblast feeder cells and HPV16; characterisation of the biological functions of Mll.*

Dr Aya Hoshino was awarded a PhD for her thesis, *The role of TBX22 in craniofacial development.*

Dr Stephen Hughes was awarded a PhD for his thesis, *Antigen presentation and dendritic cell function in severe malnutrition.*

Dr Thomas Jacques was awarded the Cavanagh Prize of the British Neuropathological Society.

Jacqueline Jonuschies was awarded a poster prize at the second Summer School for Myology in Berlin.

Dr Marko Kerac was awarded a PhD for his thesis, *Improving the treatment of severe acute malnutrition in childhood.*

Dr Rachel Knowles was awarded a PhD for her thesis, *Modelling childhood survival with serious congenital heart defects.*

Astrid Lammers was awarded an MD(Res).

Dr Wen-Hsin Lee was awarded a PhD for her thesis, *A model to study the function of NPM-MLF1 in myelodysplasia.*

Dr Sonia Lewycka was awarded a PhD for her thesis, *Reducing maternal and neonatal deaths in rural Malawi: Evaluating the impact of a community based women's group intervention.*

Dr David Long received a Medical Research Council New Investigator Award for his work on thymosin-beta4 in glomerular development and disease.

Dr Mairéad MacSweeney was awarded £5.5 million by the Economic and Social Research Council for the continuation of the Deafness Cognition and Language Research Centre for the next five years, alongside her fellow directors Professor Bencie Woll, Professor Gary Morgan and Professor Gabriella Vigliocco.

Dr Eleanor Main was awarded a Fellowship of the Chartered Society of Physiotherapy.

Dr Marina Martinos was awarded a PhD for her thesis, *The consequences of convulsive status epilepticus in children.*

Dr Hannah Mitchison was appointed to the editorial board of the journal, *Cilia.*

Dr Dale Moulding was the second prize winner in the poster competition at the UCL Centre for Immunodeficiency's 4th One-Day Symposium on Primary Immunodeficiency: "Enhancing Immune Function".

Alice Neal was awarded a poster prize at the Neuromuscular Translational Research Conference 2011.

Dr Kiran Nistala was awarded a PhD for his thesis, *The biology of Th17 and regulatory T cells in juvenile idiopathic arthritis.*

Dr Pascal Odent was awarded a PhD for his thesis, *Early infant feeding and neonatal survival in Nepal: breastfeeding, colostrum and discarding of the first milk.*

Dr Hikari Osaki was awarded a PhD for her thesis, *Investigation of the leukaemic activity of MLL-fusions in human haematopoietic cells.*

Maurizio Pacilli was awarded an MD(Res).

Dr Jenny Papakrivopoulou was awarded a Wellcome Trust Postdoctoral Training Fellowship for MB/PhD Graduates for her project, *Planar cell polarity in glomerular development and disease.*

Dr Anna Pearce was awarded a PhD for her thesis, *Will policies in the early years reduce inequalities in health? A synthesis to inform policy.*

Dr Pamela Phillips was awarded a PhD for her thesis, *Monitoring blood stream infection in neonatal intensive care units.*

Dr Edmund Poon was awarded a PhD for his thesis, *Assessment of TWIST1 as an immunotherapeutic target of cancer.*

Dr Weerapong Prasongchean was awarded a PhD for his thesis, *Somatic stem cells: properties and potential for regenerative medicine.*

Professor Jugnoo Rahi was appointed to lead the Royal College of Ophthalmologists' Quality Standards Group work to develop the first quality standards for paediatric ophthalmology. She was also appointed to serve on the Medical Research Council Clinical Fellowships Panel.

Dr Helen Rees was awarded a PhD for her thesis, *The role of MET receptor tyrosine kinase and hepatocyte growth factor signalling in paediatric rhabdomyosarcoma.*

Dr Valeria Ricotti was awarded the Elsevier Poster Prize at the World Muscle Society Congress in Portugal in October 2011.

Dr Andreas Roposch was offered the Chair of the Scientific Committee of the European Paediatric Orthopaedic Society. He also won the 2010 best clinical research award from the European Paediatric Orthopaedic Society for his work on newborn hip dysplasia.

Dr Alexander Rossdeutsch was awarded an MBPhD for his thesis, *The role of Thymosin beta4 in vascular development.* He was also awarded the 2010 Cordwainer's Prize for the Best Thesis of the Year in the MBPhD Course.

Hemant Sahni was awarded a Graduate Research Scholarships for Cross Disciplinary Training by University College London.

Dr Julie Sanders was awarded a PhD for her thesis, *Development and validation of a scoring system to assess post-operative morbidity following cardiac surgery. The cardiac post-operative morbidity score.*

Dr Miriam Schmidts was awarded a Clinical Training Fellowship by Action Medical Research for her PhD project, *Molecular genetic analysis of asphyxiating thoracic dystrophy (Jeune syndrome).*

Dr Delali Sefe was awarded an MBPhD for her thesis, *T cell kinetics in HIV infected children.*

Dr Harriet Shannon was awarded a PhD for her thesis, *Safety and efficacy of paediatric on-call respiratory physiotherapy in intensive care.*

Dr Lola Solebo was awarded the AMO Prize 2011 by the Royal College of Ophthalmologists for her paper, *UK and Ireland study of primary intraocular lens implantation in children ≤2 years old with congenital/infantile cataract (IOLunder2).*

Emanuela Spadoni was awarded an MPhil for her thesis, *An investigation of genetic variation in complex disorders of the pituitary gland.*

Dr Joseph Standing was awarded a Medical Research Council Methodology Research Fellowship for his project, *Mathematical and statistical modelling of CCR5 inhibitor effects in adults and children with HIV-1 infection.*

Dr Sanja Stanojevic was awarded the 2011 Paediatric Respiratory Research Award by the European Respiratory Society for outstanding contributions to the field.

Dr Giorgio Stefanutti was awarded a PhD for his thesis, *Novel experimental therapies for intestinal ischaemia and reperfusion injury.*

Professor Janet Stocks was awarded the 2011 Paediatric Assembly Lifetime Achievement Award by the European Respiratory Society for her achievements in the field of paediatric respiratory medicine.

Dr Viktoria Tchetchelnitski was awarded a PhD for her thesis, *Regulation of neurotrophin receptors by receptor-type protein tyrosine phosphatases.*

Professor Adrian Thrasher received the European Society of Gene and Cell Therapy's Outstanding Achievement Award 2011.

Dr Sophia Varadkar was awarded a PhD for her thesis, *Kynurenine pathway metabolites in childhood brain diseases.*

Dr Eileen Vizard was awarded a MD(Res) for her thesis, *Developmental trajectories towards sexually abusive behaviour and emerging severe personality disorder in childhood.*

Dr Martin Weber was awarded an MD(Res) for his thesis, *Retrospective analysis of post-mortem investigation of sudden unexpected deaths in infancy.*

Dr Caroline Williams was awarded a National Institute for Health Research Doctoral Fellowship.

Grants and donations

The UCL Institute of Child Health and Great Ormond Street Hospital continue to receive grants towards research from the following individuals and organisations:

A
Abbott Laboratories
Abbott Nutrition
Karim and Raya Abdel-Motaal
The Academy of Medical Sciences
Actelion
Action Duchenne
Action Medical Research
Action on Hearing Loss (trading name of the Royal National Institute for Deaf People)
Christine Adcock and her friends
Malcolm Hardy Addison
Paul and Debbie Adler
AFM Téléthon (Association Française contre les Myopathies)
Age UK (combining Ace Concern and Help the Aged)
Alcon
Alder Hey Children's NHS Foundation Trust
Alexion
Alzheimer's Research UK (formerly Alzheimer's Research Trust)
AMAG Pharmaceuticals
Ambu
Anatomical Society
Angelina Our Star Appeal
Antisoma
Arthritis Research UK (formerly Arthritis Research Campaign)
Aspreva
Association for International Cancer Research
Association Monégasque contre les Myopathies
The Association of Anaesthetists of Great Britain and Ireland
Association of Paediatric Anaesthetists of Great Britain and Ireland
Astellas Pharma
Asthma UK
AstraZeneca
Autism Speaks
AVI BioPharma
Avidex

B
Barts and The London Charity
Batten Disease Family Association
David Baum International Foundation
Baxter
Bayer Schering Pharma AG
Beaufour Ipsen Pharma
Mrs Heather Beckwith
Becta (formerly British Educational Communications and Technology Agency)
BIAL
Horst-Bickel-Stiftung (Horst Bickel Foundation)
Big Lottery Fund
Bioenvision
Biogen Idec Hemophilia
BioMarin
Biophage Limited
Biotechnology and Biological Sciences Research Council
Birds Eye Wall's
Bliss
Bone Cancer Research Trust
BPL (Bio Products Laboratory)
Bristol-Myers Squibb
British Academy
British Academy of Childhood Disability
British Association for Paediatric Nephrology
British Council
The British Dietetic Association
British Heart Foundation
British Journal of Anaesthesia
British Lung Foundation
British Medical Association
British Neuropathological Society
British Skin Foundation
The British Society of Audiology
Mr Brian Bunce
Bupa Foundation
Tiggy Butler

C
The C P Charitable Trust
Cambridge University Hospitals NHS Foundation Trust
The Sophie Cameron Trust
Canadian Institutes of Health Research
Cancer Fund
Cancer Research UK
Canterbury Christ Church University
Fondation Milena Carvajal-ProKartagener
Central and East London NIHR Comprehensive Local Research Network
Cerebra
The CgD Research Trust
Channel Four Television Corporation
Charité – Universitätsmedizin Berlin (Charité University Hospital Berlin)
Chartered Society of Physiotherapy Physiotherapy Research Foundation
CHDI
Child Growth Foundation
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CHILDREN with CANCER
Children's Brain Diseases Foundation (A Batten Disease Resource)
Children's Cancer and Leukaemia Group
Children's Hyperinsulinism Fund
Children's Liver Disease Foundation
Children's Memorial Hospital
The Children's Research Fund
The Children's Trust, Tadworth
Childrens Arthritis Trust
Chiron
Cincinnati Children's Hospital Medical Center
Ashton and Tilly Clanfield
CLAPA (Cleft Lip and Palate Association)
Miss A E Clarke
CLEFT
CLIC Sargent
Climb (Children Living with Inherited Metabolic Diseases)
Clinique
Coeliac UK
Cold Spring Harbor Laboratory
Colgate-Palmolive
The Colt Foundation
The Company of Biologists
Cord Blood Charity
CORDA
Mrs Janet Crawford
CREA
Crohn's in Childhood Research Association
CSL Behring
CURE
Cyberonics
Cystic Fibrosis Trust
Cystinosis Foundation
Cystinosis Foundation Ireland
Cystinosis Research Network

D
Daiichi-Sankyo
Danone Baby Nutrition
James Datnow
Dr Genevieve and Mr Peter Davies
Hywel Davies
Deafness Research UK
DEBRA
Mrs Audrey Dell
Department for Business, Innovation and Skills
Department for Education
Department for International Development
Department of Health
Diabetes UK
Samantha Dickson Brain Tumour Trust
Dimbleby Cancer Care
Down's Syndrome Association
The Dromintee Trust
Duchenne Parent Project
Lord and Lady Dundas

E
Economic and Social Research Council
Eisai
Elimination of Leukaemia Fund
EMBO
Emergency Nutrition Network
Engineering and Physical Sciences Research Council
Epilepsy Research UK
European Commission
European Parliament
European Respiratory Society
European Society for Immunodeficiencies
European Society for Paediatric Endocrinology
European Union

F
Fidelity International
Fiduciary Asia Corporate Services Pte. Ltd
Fight for Sight
Mr and Mrs Fitzpatrick
Food and Agriculture Organization of the United Nations
Foundation for the Study of Infant Deaths
Mrs Thelma Fox
Fundação para a Ciência e a Tecnologia (Portuguese Foundation for Science and Technology)

G
Bill & Melinda Gates Foundation
General Charitable Trust of the Institute of Child Health
Généthon
Genex Biosystems
Genzyme
Gilead
GlaxoSmithKline
GlaxoSmithKline Biologicals
Raisa Gorbachev Foundation
The Gosling Foundation Limited
The Shauna Gosling Trust
Mrs Dorothy Graham
Rob Gray
Great Ormond Street Hospital Children's Charity
Mr Hugh Greenwood
Grifols
The Guide Dogs for the Blind Association
Deborah Gulperin
Guy's and St Thomas' NHS Foundation Trust
Cynthia Rose Gwilliam

H
Charles and Kaaren Hale
Paul Hamlyn Foundation
Guy and Julia Hands
The Charles Hawkins Fund for Handicapped Children
Cathal Hayes Research Foundation
The Health and Care Infrastructure Research and Innovation Centre
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Health Protection Agency
Healthcare Infection Society (formerly Hospital Infection Society)
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Jane Hodge Foundation
Olivia Hodson Cancer Fund
Paul and Jane Hollway
Hospital Dona Estefania
The Hospital for Sick Children (SickKids)
HSA Charitable Trust
Human Early Learning Partnership
Human Frontier Science Program
D B Hussey
Hyperinsulinism Parent Support Group

I
Ichthyosis Support Group
ICN Pharmaceuticals
Ideal Shopping Direct Plc
ID Greening Budget
Imperial College Healthcare Charity
Imperial College Healthcare NHS Trust
Imperial College London
Inspiration Biopharmaceuticals
Institute for Fiscal Studies
Institute of Biomedical Science
International Association for the Study of Pain
International Insulin Foundation
Investec
Ipsen
Isis Pharmaceuticals

J
Janssen
Janssen Biotech, Inc. (formerly Centocor Ortho Biotech Inc.)
Jeans for Genes
The Jenner Institute
The Jennifer Trust for Spinal Muscular Atrophy
JISC (Joint Information Services Committee)
The Johns Hopkins University
The Johns Hopkins University School of Medicine

K
Kellogg's
The Kay Kendall Leukaemia Fund
Kidney Research UK
Kids Company
Kids Kidney Research (formerly the Kidney Research Aid Fund)
Kevin Kitching and Sinead O'Shea
The Mary Kitzinger Trust

L
Fondation Leducq
Eugène and Stephanie Léouzon
Leukaemia & Lymphoma Research
Leukaemia & Lymphoma Society
The Leverhulme Trust
The Bernard Lewis Family Charitable Trust
Eli Lilly and Company
Lilly Foundation
Ruth Lilly Philanthropic Foundation
The Enid Linder Foundation
Fondazione Eugenio Litta
Longview
LV= (Liverpool Victoria)

M
Macular Disease Society
Macula Vision Research Foundation
The Man Group plc Charitable Trust
March of Dimes Foundation
Marie Curie Cancer Care
Marks and Spencer plc
The Nancy Lurie Marks Family Foundation
Masimo
Mason Medical Research Foundation

Grants and donations
continued

Annabel McEnery Children's Cancer Fund
Allison and Harvey McGrath
Mead Johnson
Scott and Suling Mead
Medical Research Council
Medical Research Society
Medtronic
The Gavriel Meir Trust
MEND
Meningitis Research Foundation
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MRC Clinical Trials Unit
MSS Research Foundation (Marshall-Smith Syndrome)
Mundipharma
Mundipharma Research Limited
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Myositis Support Group

N
National Academy of Education
National Eczema Society
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National Institute for Health Research (NIHR)
National Institute of Academic Anaesthesia
National Institute of Mental Health (part of the National Institutes of Health)
National Institute of Neurological Disorders and Stroke (part of the National Institutes of Health)
National Institutes of Health
National Patient Safety Agency
National Specialised Commissioning Team (DH)
Nederlandse Organisatie voor Wetenschappelijk Onderzoek (Netherlands Organisation for Scientific Research)
Nelsons
The Neuroblastoma Society
Newcastle University
Newlife Foundation for Disabled Children (formerly Birth Defects Foundation (BDF)/ BDF Newlife)
Nexen Petroleum UK Ltd
NHS Blood and Transplant
NHS Innovations London
The NIHR Biomedical Research Centre at Great Ormond Street Hospital for Children NHS Foundation Trust and UCL Institute of Child Health
NIHR Biomedical Research Centre for Ophthalmology
NIHR Health Technology Assessment programme
North Bristol NHS Trust
North Central London Innovation Hub
North Thames Regional Cleft Lip and Palate Service
Novartis
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O
Ms Dorothy Oakley
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P
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Philips AVENT
Mrs Irene Philips
Nick and Miranda Pink
PRA (Pharm Research Associates)
Primary immunodeficiency Association
PTC Therapeutics
Public Health Laboratory Service (DH)
Public Health Research Consortium (DH)

Q
Queen Mary, University of London
Quintiles

R
Rank Bequest
The Rank Prize Funds
Alexandra Raphael and family
Rays of Sunshine Children's Charity
Research Autism
Research Into Childhood Cancer
Rho
Rijksinstituut voor Volksgezondheid en Milieu (Dutch National Institute for Public Health and the Environment)
Dr Sara Riley
Roche
Ronald McDonald House Charities
Ross Products Division
Fondation Genevoise de bienfaisance
Valeria Rossi di Montelera
The Royal Academy of Engineering
The Royal College of Ophthalmologists
Royal College of Paediatrics and Child Health
The Royal College of Radiologists
The Royal College of Surgeons of Edinburgh
The Royal College of Surgeons of England
Royal National Institute of Blind People
The Royal Society

S
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Smiths Medical
Society for Mucopolysaccharide Diseases (the MPS Society)
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Sparks (Sport Aiding Medical Research for Kids)
St George's, University of London
St Peter's Trust for Kidney, Bladder and Prostate Research
Stanford University
Sir Hugh and Lady Stevenson
Sir Halley Stewart Trust
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Summit
Sweets for Life
The Walter Swindon Charitable Trust
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Jean-Michel and Isabel Sylvestre

T
Takeda
Tanita
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The Baily Thomas Charitable Fund
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Trophos
The True Colours Trust

U
U.S. Department of Health & Human Services
UBS
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UCL Business PLC
UCL Futures
UCL Grand Challenge of Global Health
UCL Institute of Education
UCL Institute of Neurology
UK Clinical Research Collaboration
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UK National Screening Committee
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The Ulverscroft Foundation
UNHCR (United Nations High Commissioner for Refugees)
UNICEF (United Nations Children's Fund)
Università degli Studi di Milano (the University of Milan)
Universität Hamburg (University of Hamburg)
University College London
University College London Hospitals Charity
University College London Hospitals NHS Foundation Trust
University College London Hospitals/ UCL NIHR Biomedical Research Centre
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University of Bristol
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The University of Iowa
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The University of Manchester
The University of Nottingham
University of Oxford
The University of Sheffield
University of Southampton
The University of York
USAID (U.S. Agency for International Development)

V
Valid International
Vitaflo
Vitol Foundation
VolkswagenStiftung (Volkswagen Foundation)

W
Miss Dorothy Wall
Mr and Mrs John Walton
Sir Siegmund Warburg's Voluntary Settlement
Mrs Eluned Watkins
Wayne State University
Wellbeing of Women
WellChild
Wellcome Trust
The Welton Foundation
Wockhardt UK
The Charles Wolfson Charitable Trust
World Health Organization
Richard Wright
Wyeth
Wyeth-Lederle Vaccines

Y
Yorkhill Children's Foundation

Senior academic staff

Cancer Theme

Theme Leader
Professor Kathy Pritchard-Jones

Molecular Haematology and Cancer Biology Unit

Reader in Molecular Neurobiology and Head of Unit
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Professor of Paediatric Haematology and Oncology
Professor Ian Hann
Hugh and Catherine Stevenson Professor of Cancer Biology
Professor Kathy Pritchard-Jones
Professor of Paediatric and Developmental Pathology
Professor Neil Sebire
Emeritus Professor of Haematology and Oncology
Professor Judith Chessells
Reader in Paediatric Oncology
Dr John Anderson
Reader in Cancer Biology
Dr Arturo Sala (until August 2011)
Senior Research Fellow
Dr Martino Barenco
Senior Lecturer
Dr Mike Hubank
Honorary Senior Lecturers
Dr Julia Chisholm
Dr Nicholas John Goulden
Dr Darren Hargrave (from August 2011)
Walport Lecturers
Dr Daniel Morgenstern (from December 2011)
Dr Karin Straathof (from March 2011)
Lecturer
Dr Owen Williams
Honorary Lecturer
Dr Tanzina Chowdhury

Cardiorespiratory Sciences Theme

Theme Leader
Professor Janet Stocks (until June 2012)
Professor Christopher O'Callaghan (from June 2012)

Cardiac Unit

The British Heart Foundation Vandervell Professor of Congenital Heart Disease and Head of Unit
Professor John Deanfield
Professor of Cardiothoracic Surgery
Professor Martin Elliott
Honorary Professors
Dr Dominique Bonnet
Professor Marc de Leval
Emeritus Professor of Paediatric Cardiac Morphology
Professor Robert Anderson
Honorary Reader
Dr Michael Burch
Senior Clinical Research Fellow
Dr Vivek Muthurangu
Honorary Senior Lecturers
Dr Katherine Brown
Dr Alessandro Giardini
Dr Tain-Yen Hsia
Dr Sachin Khambadkone
Professor Martin Kostolny (from April 2011)
Miss Isabelle Russell-Eggitt
Dr Ingram Schulze-Neick
Dr Ian Sullivan
Walport Lecturer
Dr Marietta Charakida (from February 2011)

Patient Care Research and Innovation Centre

Honorary Professor
Professor Faith Gibson
Honorary Clinical Senior Lecturer
Dr Debbie Sell

Portex Unit of Paediatric Anaesthesia, Pain Research, Critical Care, Respiratory Medicine, Physiology and Physiotherapy

Head of Unit
Professor Janet Stocks (until June 2012)
Professor Christopher O'Callaghan (from June 2012)
Smiths Medical Professor of Anaesthesia and Critical Care
Professor Michael (Monty) Mythen
Professors of Respiratory Physiology
Professor Christopher O'Callaghan (from June 2012)
Professor Janet Stocks
Emeritus Professor of Paediatric Anaesthesia
Professor David Hatch
Honorary Reader in Paediatric Intensive Care
Dr Quen Mok
Honorary Reader in Respiratory Paediatrics
Dr Colin Wallis
Senior Research Fellow
Dr Sook-Yuen Lum
Senior Lecturer
Dr Eleanor Main
Clinical Senior Lecturers
Dr Mark Peters
Dr Suellen Walker
Honorary Senior Lecturers
Dr Paul Aurora
Dr Robert Bingham (until February 2012)
Dr Ann Black (until June 2011)
Dr Phil Cunnington
Dr David de Beer
Dr Robert Dinwiddie (from January 2012)
Dr Hilary Glaisyer
Dr Louise Harding
Dr Jane Herod
Dr Richard Howard
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Dr Ian James
Dr Paula Lister
Dr Adrian Lloyd-Thomas
Dr Angus McEwan (until March 2011)
Dr Caro Minasian
Dr Reema Nandi
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Dr Andy Petros
Dr Christine Pierce
Dr Padmanabhan Ramnarayan
Professor Andrew Shaw (from July 2011)
Dr Jonathan Smith
Dr Mike Sury (from November 2011)
Dr Mark Thomas (from March 2012)
Dr Isabeau Walker
Dr Hugo Wellesley (from September 2011)
Dr Glyn Williams
Dr Sally Wilmshurst (until October 2011)
Honorary Lecturers
Dr Sarah Barnett (until March 2012)
Dr Denny Levett (from December 2011)

General and Adolescent Paediatrics Theme

Theme Leader
Professor Terence Stephenson

General and Adolescent Paediatrics Unit

Nuffield Professor of Child Health and Head of Unit
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Professorial Research Associate
Professor Helen Roberts
Professor in Adolescent Health
Professor Russell Viner
Emeritus Professor of Paediatrics
Professor Mark Gardiner (from January 2011)
Emeritus Professor of Community Child Health
Professor Brent Taylor
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Honorary Reader
Dr Deborah Christie
Clinical Senior Lecturer
Dr Eddie Chung
Honorary Senior Lecturers
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Dr Zaib-Un-Nisa Sadiqah Davids (until February 2011)
Dr Haitham Elbashir
Dr Deborah Hodes (until March 2011)
Dr Sanjay Kinra (from March 2011)
Dr Camilla Salvestrini (until February 2011)
Dr Ananth Shankar
Dr Franco Torrente
Dr Emma Weisblatt (from July 2011)
Dr Daniel Wood
Lecturer
Dr Sophie Khadr

Louis Dundas Centre for Children's Palliative Care

True Colours Chair in Palliative Care for Children and Young People and Head of Unit
Professor Myra Bluebond-Langner

Genes, Development and Disease Theme

Theme Leader
Professor Pete Scambler

Clinical and Molecular Genetics Unit

Professor of Clinical and Molecular Genetics and Head of Unit
Professor Gudrun Moore
Professor of Paediatric Metabolic Disease and Hepatology
Professor Peter Clayton
Professor of Paediatric Endocrinology
Professor Mehul Dattani
Professor of Computational Biology
Professor Peter Hammond
Professor of Clinical Chemistry
Professor Simon Heales
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Professor Peter Hindmarsh
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Professor Susan Malcolm
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Professor Marilyn Monk
Emeritus Professor of Biochemistry
Professor David Muller
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Professor Marcus Pembrey
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Professor Michael Preece
Emeritus Professor of Biochemistry
Professor Bryan Winchester
Reader in Paediatric Endocrinology and Wellcome Trust Senior Research Fellow in Clinical Science
Dr John Achermann
Reader in Clinical and Molecular Genetics
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Reader in Paediatric Endocrinology
Dr Khalid Hussain
Reader in Paediatric Metabolic Medicine
Dr Shamima Rahman
Senior Clinical Research Fellow
Dr Peter Adlard
Senior Research Fellow
Dr Kevin Mills
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Dr Caroline Brain
Dr Stephanie Grünewald
Dr Gill Levitt
Dr Alison Male
Dr Richard Scott (from January 2011)
Dr Helen Spoudeas
Dr Richard Stanhope
Dr Ashok Vellodi (until March 2011)
Senior Clinical Research Associate
Dr Paul Gissen (from March 2011)

Medical Molecular Biology Unit

Professor of Human Genetics and Head of Unit
Professor David Latchman
Reader in Molecular and Cell Biology
Dr Anastasis Stephanou
Senior Research Fellow
Dr Mattia Calissano (until May 2011)
Honorary Senior Lecturer
Dr Richard Knight (from May 2011)
Lecturer
Dr Vishwanie Budhram-Mahadeo

Molecular Medicine Unit

Professor of Molecular Medicine and Head of Unit
Professor Pete Scambler
Professor of Medical and Molecular Genetics and Wellcome Trsut Senior Research Fellow
Professor Phil Beales
Honorary Professor in Molecular Cardiology
Professor Paul Riley (from October 2011; Professor of Molecular Cardiology until September 2011)
Senior Lecturer
Dr Hannah Mitchison

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Reader in Nephrology and Head of Unit
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Professor Robert Kleta
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Dr Daljit Hothi
Dr Stephen Marks
Mr Imran Mushtaq
Dr Rukshana Shroff
Dr Kjell Tullus
Honorary Lecturer
Mr Divyesh Desai

Senior academic staff
continued

Infection and Immunity Theme

Theme Leader
Professor Christine Kinnon

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Professor of Vaccinology and Immunology, Director of Clinical Research and Development and Head of Unit
Professor David Goldblatt
Professor of Immunology
Professor Robin Callard
Professor of Experimental Immunology
Professor Tessa Crompton
Honorary Professor of Paediatric Dermatology
Professor John Harper
Emeritus Professor of Molecular Immunology
Professor Malcolm Turner
Lecturer
Dr Wei-Li Di

Infectious Diseases and Microbiology Unit
Professor of Infectious Disease and Immunology and Head of Unit
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Professor Alan Phillips
Senior Lecturer
Dr Mona Bajaj-Elliott
Clinical Senior Lecturer
Dr Paul Brogan (jointly with Rheumatology Unit)
Honorary Senior Lecturers
Dr Garth Dixon (from June 2011)
Dr Susan Hall
Dr John Hartley (until January 2011)
Dr Valerio Novelli
Dr Delane Shingadia (from June 2011)
Dr Martin Weber
Academic Clinical Lecturer
Dr Jonathan Cohen
Honorary Lecturer
Dr Kathryn Harris (from June 2011)

Molecular Immunology Unit
Professor of Molecular Immunology and Head of Unit
Professor Christine Kinnon
Professor of Transplantation Immunology
Professor Persis Amrolia
Professor of Paediatrics and Immunology
Professor Bobby Gaspar
Professor of Paediatric Immunology and Wellcome Trust Senior Clinical Fellow
Professor Adrian Thrasher
Reader in Molecular Biology
Dr Kenth Gustafsson
Reader in Molecular Genetics
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Honorary Reader in Paediatric Immunology
Dr Graham Davies
Honorary Reader in Stem Cell Transplantation
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Walport Lecturer
Dr Paul Turner (from March 2011)

Rheumatology Unit
Professor of Paediatric Rheumatology and Head of Unit
Professor Lucy Wedderburn
Honorary Senior Lecturer
Dr Clarissa Pilkington (from April 2011)
Senior Clinical Lecturer
Dr Paul Brogan (jointly with Infectious Diseases and Microbiology Unit)
Senior Lecturer
Dr Bin Gao
Honorary Lecturer
Dr Charles Li (from January 2011)

Neurosciences and Mental Health Theme

Theme Leader
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Professor of Developmental Psychopathology
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Professor Philip Graham
Senior Teaching Fellow
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Senior Research Fellow
Dr Jessica Hobson
Senior Clinical Research Fellow
Dr Robert Senior
Honorary Senior Lecturers
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Dr Danya Glaser (from October 2011)
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Visiting Professor
Dr Mortimer Mishkin
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Dr Michelle de Haan
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Dr Alexandra Hogan
Dr Margaret Mayston
Dr Peter Rankin
Lecturer in Developmental Cognitive Neuroscience
Dr Frédérique Liégeois

Dubowitz Neuromuscular Centre
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Honorary Professor
Professor Caroline Sewry
Reader in Cell Biology and Wellcome Trust University Award Holder
Dr Jennifer Morgan
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Dr Stephanie Robb (until March 2011)
Honorary Lecturer
Dr Matthew Wood (from April 2011)

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Rank Professor of Biophysics
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Dr Kieran McHugh
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Professor Andy Copp
Reader in Developmental Neurobiology
Dr Nick Greene
Reader in Neurobiology and Wellcome Trust University Award Holder
Dr Juan Pedro Martinez-Barbera
Great Ormond Street Hospital Children's Charity Reader in Craniofacial Developmental Biology and Genetics
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Lecturer
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Dr Robert Goold

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Professor Sheena Reilly
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Dr Rod Scott
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Dr Vijeya Ganesan
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Dr Carlos de Sousa
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Dr Sophia Varadkar
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Ms Katie Price

Nutritional and Surgical Sciences Theme

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Professor Atul Singhal (from October 2011)

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continued

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Dr Elizabeth Isaacs
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Emeritus Nuffield Chair of Paediatric Surgery
Professor Lewis Spitz
Honorary Reader
Dr Andreas Roposch
Clinical Senior Lecturer
Dr Paolo de Coppi
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Dr Robert Evans
Mr Thirloshan Kangesu
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Dr Keith Lindley
Dr Michael Mars
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Dr Virpi Smith
Mrs Michelle Wyatt (from January 2011)
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Population Health Sciences Theme

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Professor Catherine Law (from April 2012)

Centre for International Child Health
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Reader in International Child Health and Wellcome Trust Senior Research Fellow in Clinical Science
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Miss Sandra Lang
Senior Research Fellows
Dr Andrew Seal
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Professor Catherine Law (from April 2012)
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Zhen is three years old and is staying on Lion Ward while he is treated for B-cell lymphoma. He has been here for two months, but recently got to go home for the day to celebrate his third birthday!

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Written by Adam Kamenetzky. Designed and produced by Doulla Theodorou.

Photography by Adam Laycock and Candice-Joelle Wordley.

Printed by Hill and Garwood, using vegetable-based inks on Heaven 42.

Thank you to everyone who was interviewed for, or gave permission for their picture to be used in this review, as well as the many members of the UCL Institute of Child Health and Great Ormond Street Hospital staff who helped during its production.

Please visit www.ucl.ac.uk/ich/research-ich or www.gosh.nhs.uk/research-and-innovation for an online version of this review.

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Bengali
অনুরোধ করলে নিম্নলিখিত ঠিকানায় থেকে এই লেখার অনুবাদ, বড় অক্ষর, ব্রেল বা অডিও বিবরন পাওয়া যাবে|

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Polish
Tłumaczenia są do uzyskania na żądanie pod podanym powyżej adresem. Dokumenty w formacie 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,பெரய்லி அல்லது ஒலி பதிப்புகள் விண்ணப்பித்தால் கீழ்கண்ட 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 آڈیوپردرج ذیل پتے سے حاصل کی جا سکتی ہے۔