UCL INSTITUTE OF CHILD HEALTH

Great Ormond Street Hospital for Children
NHS Foundation Trust

Research Review 2012/13

The child first and always

Contents

Research

People

Cover: Chloe, age 11, has cystic fibrosis and is receiving treatment on Badger Ward.

Left: Jordell-Talib, age 12, is an outpatient at the department of Child and Adolescent Mental Health Service (CAMHS).

Six-year-old Luca on Walrus Ward. Luca has come all the way from Malta to receive treatment.

Director's report

The UCL Institute of Child Health (ICH), with its partner Great Ormond Street Hospital (GOSH), 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.

It has been a great privilege for me to join, in October 2012, the ICH. The mission of the ICH, which is to 'improve the health and wellbeing of children and the adults they will become through world-class research, education and public engagement' is inspirational and, appropriately, puts the child at the centre of what we do. This mission has established a tremendous legacy of excellence in research and teaching, which has been benefiting children in the UK and throughout the world.

In the last year, I have been planning, with colleagues, a new academic strategy to build on the superb position that the ICH holds with its clinical partner, GOSH. To develop that strategy I spent most of my first three months meeting with staff within the research units and learning about what they do. It has been very interesting and humbling to appreciate the tremendous contributions made by colleagues in the ICH. The second three months focused on making external visits with at least one of the ICH's senior academics to other world-leading centres for children's health research and education. We visited the Hospital for Sick Children (SickKids) in Toronto, the Department of Paediatrics at Stanford University and Lucile Packard Children's Hospital, Boston Children's Hospital, Cincinnati Children's Hospital, the Children's Hospital of Philadelphia, and the Necker Children's Hospital and Imagine Research Institute in Paris. We always knew that these visits would be important, but I don't think any of us appreciated the tremendous value of the insights they provided and the exciting opportunities for collaboration presented by other leading children's institutions.

It became very clear that there were certain features that are unique to the ICH. These include having not only world-class basic science research but also superb paediatric epidemiology. We need to develop the interdisciplinary nature of the research spectrum, which spans a huge range, from very basic science research, through to population research. We have articulated some key principles that should underpin and guide our academic strategy, which are:

- interdisciplinarity
- accelerating translation
- national partnership and leadership
- developing our academic leaders

We have identified five programmes of research, which encompass all of the ICH's current strengths. These are:

- genetics and genomic medicine
- population, policy and practice
- infection, inflammation and immunity
- developmental biology and cancer
- developmental neurosciences

This will form the basis of our strategy and we will be working jointly with GOSH to develop a joint strategy for clinical research.

These new programmes will offer exciting opportunities to further develop research-led teaching. Our educational portfolio continues to grow. We have developed new modules and courses, which fit in with our paediatric and child health remit, for example, the new MSc/Diploma/Certificates in Paediatric Physiotherapy and the new pathway for Paediatric Gastroenterology. We are also starting to expand our offerings for continuing professional development to clinicians and allied health professionals interested in paediatric and child health-related subjects by developing an expanded portfolio of courses.

We continue to attract substantial funding for important new initiatives. In October 2012, HRH The Princess Royal opened the Newlife Birth Defects Research Centre, funded by the Newlife Foundation for Disabled Children, Great Ormond Street Hospital Children's Charity and other benefactors. November 2012 saw the opening of The Arthritis Research UK

Director's report
continued

Centre for Adolescent Rheumatology, with funding from Arthritis UK, Great Ormond Street Hospital Children's Charity, GOSH and UCL. This is the first of its kind and includes staff from across UCL, GOSH and University College London Hospitals, as well as the launch of a national network for research into adolescent rheumatology. Our researchers are also in receipt of substantial funding from the NIHR through the GOSH UCL Biomedical Research Centre. You can read more about their work in the Division of Research and Innovation report.

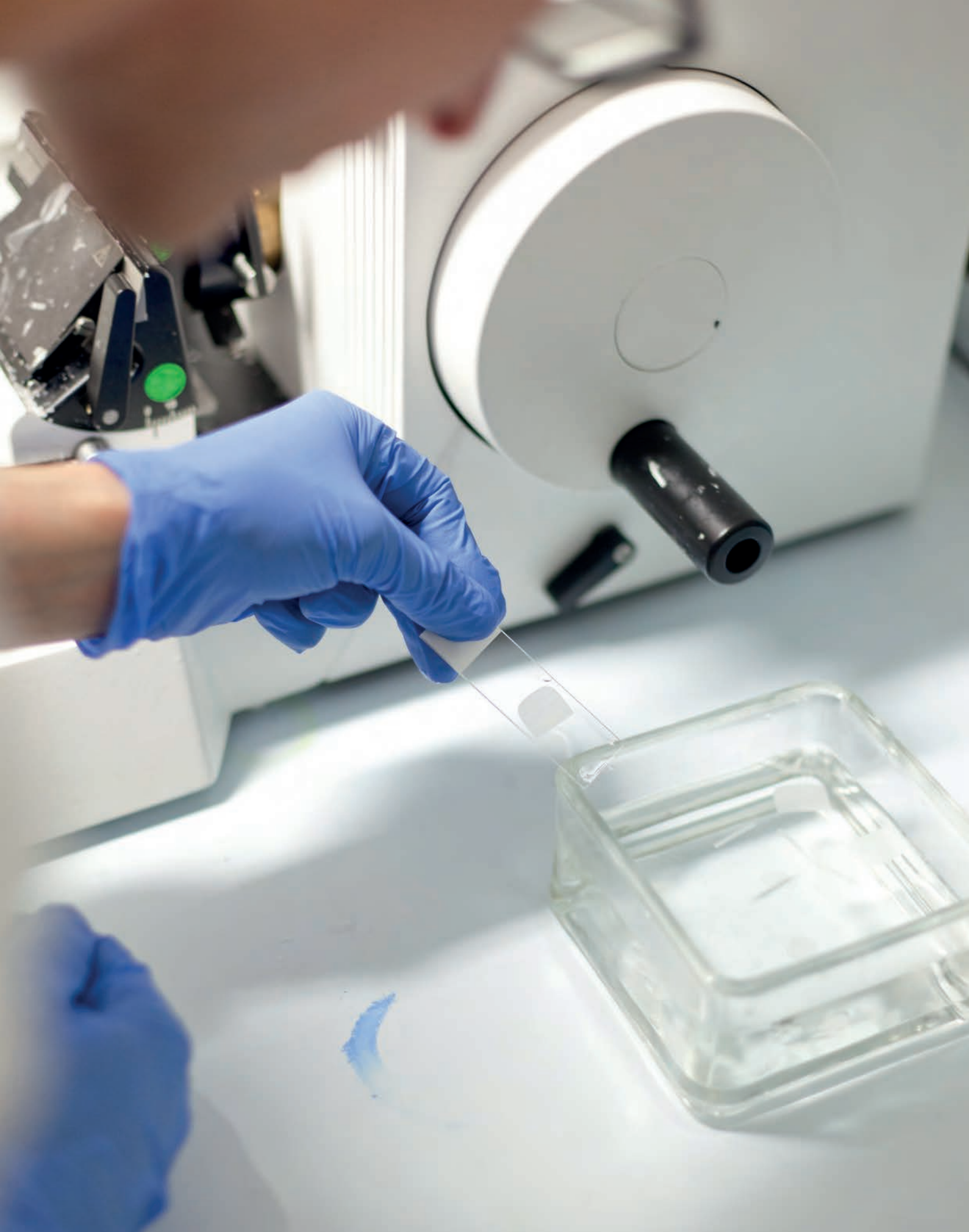
Our plans, in partnership with GOSH and Great Ormond Street Hospital Children's Charity, for a Centre for Children's Rare Disease Research, received a tremendous boost at the end of 2012, with the announcement of an award of £10 million towards the centre from the Higher Education Funding Council for England, under their UK Research Partnership Investment Fund. Finally, at the end of 2012, the Centre for International Health and Development within the ICH became a separate institute within the Faculty of Population Health Sciences at the Institute of Global Health, which is evidence of its continued and growing success.

A number of our academic staff have achieved notable positions. Professors Kathy Pritchard-Jones and Marie-Louise Newell were among the 46 individuals from across all of biomedical sciences in the UK to be elected to the Academy of Medical Sciences. Professor David Goldblatt received a National Institute for Health Research Senior Investigator Award, while Professor Terence Stephenson has been elected Chair of the Academy of Medical Royal Colleges. We have also had a tremendous clutch of successes in the senior promotions. Those promoted to Professor included John Anderson, Paul Gissen, John Achermann, Mary Fewtrell and Nick Greene. A number of other staff were promoted to Senior Lecturer and Reader. Our warmest congratulations go to them all. We have also been very pleased to appoint Professor Chris O'Callaghan to the position of Professor of Respiratory and Paediatric Medicine. Chris joins us from the University of Leicester where he has developed a very successful programme of research addressing ciliopathies. This work complements the ongoing studies here at the ICH with Professor Phil Beales and Dr Hannah Mitchison.

I am delighted to report that teams within GOSH and the ICH are continuing to report their important discoveries in top scientific journals. We have had two publications in the journal *Science* from the Africa Centre for Health and Population Studies where Professor Marie-Louise Newell has been Director for the past several years. These publications have addressed the important impact of therapies for HIV on adult life expectancy in Africa. The birth cohort studies, in which investigators from the ICH are involved, continue to report important findings. For example, Dr Alastair Sutcliffe from the General and Adolescent Paediatric Unit has looked at data from the Millennium cohort, and the National Evaluation of the Sure Start study showed better outcomes for a number of different measures in children of older mothers. Within genetic research, work from Dr Hannah Mitchison and Professor Phil Beales has continued to elucidate the genetic basis underlying ciliopathies. And *Nature Genetics* featured Professor Jugnoo Rahi's publication on novel candidate genes for myopia. Meanwhile, Professor Francesco Muntoni and colleagues in the Dubowitz Neuromuscular Centre are reporting important discoveries about pathways involved in congenital muscular dystrophy.

In summary, much has been achieved through dedication, hard work and collaboration. We need to build on this fantastic platform and I am confident that the ICH's new academic strategy will enable us to drive forward research that will be even more impactful on the lives of children and the adults that they will become.

Professor Rosalind Smyth
Director
UCL Institute of Child Health



Six-month-old Lehreen is receiving total parenteral nutrition (TPN) on Squirrel Ward.

Chief Executive's report

Conducting translational research has always been central to Great Ormond Street Hospital's (GOSH) mission to put 'the child first and always'. Today, our position as a leading paediatric research centre is a result of our close relationship with University College London, our principal academic partner, the UCL Institute of Child Health (ICH) and the support provided by Great Ormond Street Hospital Children's Charity.

Throughout this review, you will read about how our joint ventures are pioneering new and novel ways of treating children with complex and rare diseases. For example, our Director of Clinical Research and Development, Professor David Goldblatt, highlights the importance of our flagship award with UCL (funded by the National Institute for Health Research) as a Biomedical Research Centre (BRC), the only BRC in the UK with a paediatric focus.

Our review also highlights the breadth of our research. The ground-breaking gene therapy treatments developed by Professors Bobby Gaspar and Adrian Thrasher are leading global advances in treating children whose conditions mean they are unable to fight infections – with truly revolutionary results.

In this review, we also highlight the work of Dr Darren Hargrave, Consultant Paediatric Oncologist, who is leading a strategy to translate scientific discoveries into better therapies for children with brain tumours where the current prognosis remains poor. You can also read how Dr Veronica Kinsler, academic lead for paediatric dermatology at GOSH, and the ICH, identified the genetic cause of a rare skin condition, which now paves the way for developing better treatments and helping the understanding of other conditions in particular skin cancers.

GOSH sees more children with rare diseases than any other centre in Europe, and so, along with the ICH is uniquely placed to conduct research in this area. In last year's Research Review, we talked about our plans with the ICH and Great Ormond Street Hospital Children's Charity to open the new Centre for Children's Rare Disease Research. I am pleased to say that this project is now well underway. Our appointed architects are currently coming up with designs for the site, due to open in 2017, which will occupy a space just a few steps from the main hospital.

We are also very excited to be launching a joint research strategy with the ICH. This will give a clear focus for how we will work together, to develop our core research strengths including recruiting and retaining the best researchers in their fields supported by state-of-the-art infrastructure.

But there is still much more work that can and should be done. As the UK's leading children's hospital and centre for research, we should be continuously looking to exploit the opportunities technological and scientific advances can bring to improve the lives of children.

We are currently developing an information strategy that aims to enable critical patient information to be accessed wherever the patient is being treated throughout their lives. What's more, we are working to create powerful phenotype information that can be cross referenced with emerging genetic data to really drive forward treatments and cures for diseases caused by gene defects.

After reading this review I hope you will agree that GOSH and its partners are on an exciting journey of discovery to meet the challenge of improving the health and well-being of children not just in the UK

Mr Jan Filochowski
Chief Executive
Great Ormond Street Hospital
for Children NHS Foundation Trust

Division of Research and Innovation report

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

During 2012, we secured a second term of funding for the GOSH UCL Biomedical Research Centre (BRC). The National Institute for Health Research funds 11 BRCs across the country and is the flagship scheme designed to support experimental medicine and the translation of research into patient benefit.

GOSH and UCL are the recipients of the only dedicated paediatric BRC funding and have been awarded £36 million over five years (2012–2017). The focus of the BRC is rare diseases and will help maintain our status globally as a leading centre for child health research. Our focus on rare diseases leverages GOSH's role as a provider of more nationally commissioned services for rare diseases than any other NHS Trust.

During our first round of BRC funding (2007–2012), we conceived and funded the development of GOSgene to facilitate rapid gene identification in uncharacterised genetic disease. GOSgene brings UCL and ICH research expertise to principal investigators and clinicians working with congenital disorders. This enables the discovery of new genes and provides an infrastructure and step-by-step guidance for selected projects.

This initiative ultimately helps improve diagnostic testing, supports genetic counselling, and guides further functional analysis aimed at understanding the pathogenesis of disease and improving patients' management or developing novel therapies. Continued funding has allowed GOSgene to increase its capacity, and GOSgene is now able to process more samples through its exome sequencing pipeline. This expansion has allowed the enterprise to mature and rebrand as the Centre for Translational Genomics, incorporating the genomics and proteomics expertise from across UCL so that it can become the leading children's centre internationally in functional genomics. The continued success in translation is represented by the successful projects in which the centre is able to identify the causative gene in the different disorders. Currently, their overall success rate is 40 per cent, which is very high for a gene discovery programme of this nature.

GOSomics, established in 2012 as part of the BRC in collaboration with the Biological Mass Spectrometry Unit within the ICH, is a translational biomarker diseases facility that supports the investigation of potential biomarkers in rare childhood diseases. Using state-of-the-art technology, GOSomics will aid the design of diagnostic tests and help elucidate the disease mechanisms involved in rare diseases. The team are currently involved with 18 different projects, seven of which are collaborative with other groups and BRCs at UCL.

In 2012, BRC funding also allowed us to expand capacity and manufacture gene and cell therapy medicinal products more flexibly. This facility underpins the gene therapy programme at GOSH, which has pioneered gene therapy for primary immune deficiency. The programme, thanks in part to BRC funding, is now expanding its focus, targeting treatments for multiple inherited diseases, cancers and infectious disease, as well as aiding transplantation technology.

Our Somers Clinical Research Facility has delivered a growing volume of clinical and experimental medicine research within the Trust. During 2012, the number of clinical trials conducted in the CRF grew by 25 per cent and the CRF is now working with 18 industrial partners who are supporting clinical trials.

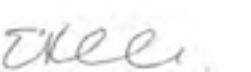
Through the BRC, we have developed a strategy for patient and public involvement and engagement in research, which builds on the success of activities led, for example, by the Medicines for Children Network. Our strategy aims to involve patients and the public in our research at all stages. Patients and the public will be embedded in our research decision-making to influence our strategy and ensure our research results in patient benefit.

These examples provide a snapshot of the activity made possible through our BRC funding, and you can read about other excellent examples of our research in this report.

The Division of Research and Innovation continues to grow to ensure research is effectively supported across the organisation. Our focus for the forthcoming year is to work closely with our clinical divisions to further embed research within the Trust.

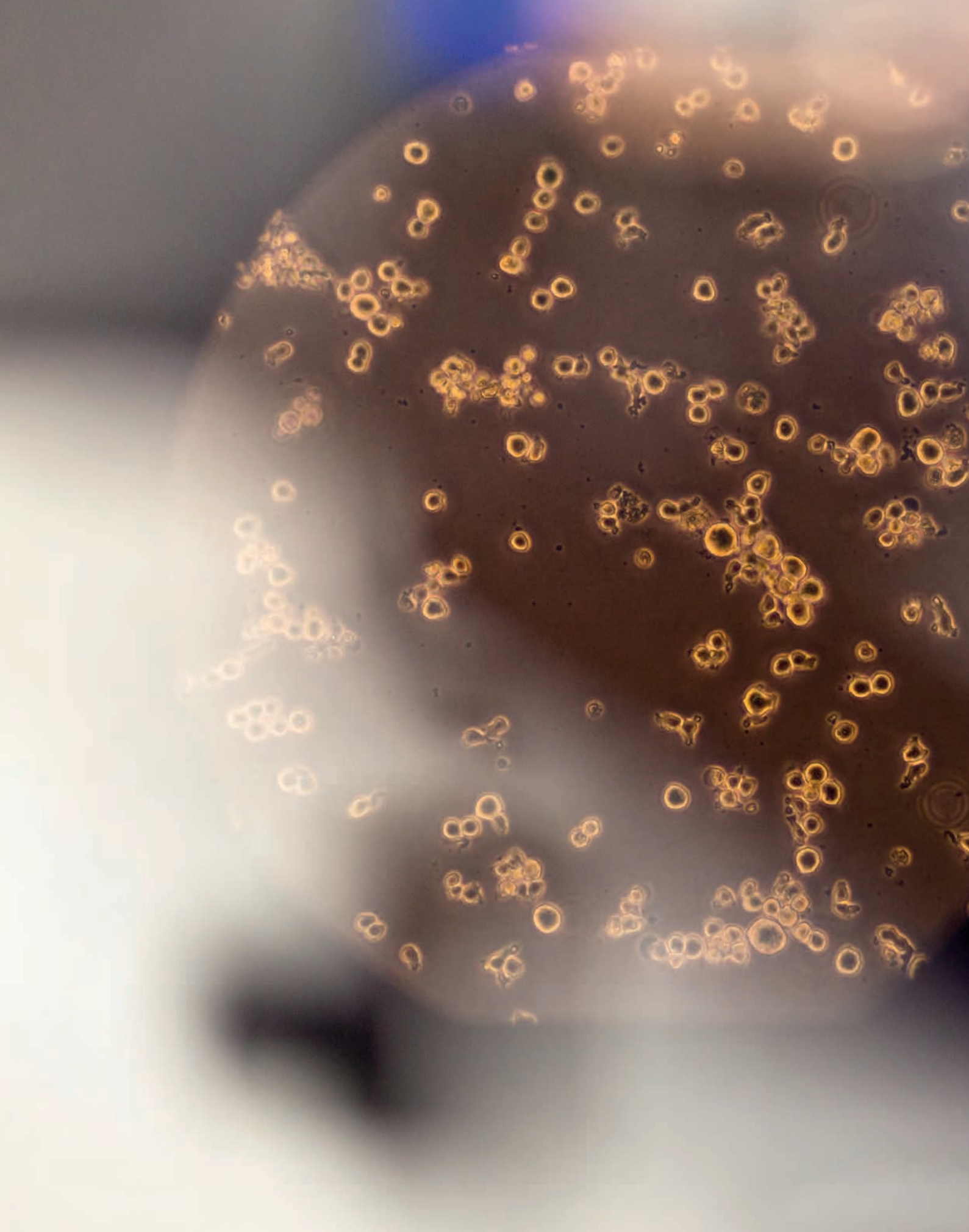
We look forward to reporting on the success of these new initiatives in future years.

Professor David Goldblatt
Director of Clinical Research and Development



Ms Emma Pendleton
Deputy Director of Research and Innovation

Freddie is having tests on Kingfisher Ward. He has to fast for 18 hours, which he isn't looking forward to!




The heart of our
effort is translating
the work into
patient benefit.

Europe's first research centre to tackle birth defects

One in 33 children in the UK is born with a birth defect, and around 40 per cent of children treated at Great Ormond Street Hospital (GOSH) have a disorder present from birth. Of the 4,000 known birth defects, some are very rare, while others such as spina bifida, cleft palate and Down's syndrome, are more common. At the UCL Institute of Child Health (ICH), we have created Europe's first centre dedicated to research into birth defects.

Professor Andrew Copp

"I began my career as a scientist with a BA in Zoology and a Doctorate of Philosophy in Experimental Embryology from the University of Oxford. I began to see how my scientific training could be clinically relevant to healthcare, but I needed to gain a deeper understanding of health problems. Hence, after my Doctorate, I applied for medical training at Guy's Hospital, London.

"Throughout the clinical course, I moonlighted in research within the Paediatric Research Unit at Guy's Hospital. I began working on a mouse model of spina bifida, which I am still studying today! I found the period of medical training both varied and stimulating, as I moved seamlessly between learning how to manage patients on the ward and studying mouse embryos that failed to close their neural tube in the lab. I think this combination of scientific and clinical training was the best possible preparation for the work I am doing today."

The Newlife Birth Defects Research Centre is a state-of-the-art laboratory, which opened in 2012. The centre brings together teams of researchers who were previously housed in separate buildings within the ICH. "By co-locating scientists in superb new labs, we are providing an exciting opportunity for collaborative research," says Professor Andrew Copp, Head of the Newlife Birth Defects Research Centre. "We hope the centre's research will enable significant strides in understanding the causes of birth defects, and then developing new ways to treat or prevent them."

Professor Copp highlights how research in the centre is inspired by clinical experiences at GOSH. "Working alongside leading clinicians ensures that children's needs remain at the heart of our research. The clinical cases of birth defects seen at the hospital are vital in shaping our research questions. At the same time, researchers have the opportunity to go back to the hospital to translate their major research findings for patient benefit."

Work at the new centre is broadly divided into three specialist areas. Some researchers are working to identify the genes responsible for birth defects, particularly for rare genetic conditions. Others are studying the complex processes involved in the development of embryos and using this information to discover the causes of conditions such as spina bifida. A third group of researchers are devising novel therapies to treat or prevent some of these conditions, for example using stem cells and tissue engineering.

Professor Copp's own team, which is co-directed by Professor Nick Greene, is investigating the early events that lead to spina bifida and related disorders. "We can only really move forward with new treatments or prevention if we understand how and why the embryo fails to close its spinal cord in the early weeks of pregnancy," says Professor Copp. Some years ago, the team found that spina bifida could be prevented effectively in mice using a supplement called inositol. "It was very exciting to identify a possible new approach to prevention that might in future be added to folic acid."

This early research culminated in a clinical trial (the PONTI study), which is being conducted throughout the UK, thanks to support from the Medical Research Council and SPARKS, a children's medical research charity. Women with a previous pregnancy affected by spina bifida are invited to join, and are then randomly allocated to receive inositol plus folic acid during their next pregnancy, or else folic acid alone. Professor Copp adds: "The preliminary PONTI study will finish in late 2013, and we are now planning a larger scale, Europe-wide clinical trial. Our aim is to discover whether inositol is effective in preventing some cases of spina bifida, alongside folic acid."

While research into birth defects remains the core focus of the centre, training and public engagement are also high priorities. "Ensuring our young scientists become world-class experts will allow the centre to lead the way in birth defects research for years to come," says Professor Copp. He also recognises the role the centre could play in engaging with the public. "Families with children who have birth defects have little access to information on the latest research. In the future we would like to create a portal that provides them with sensible and accessible information about the latest discoveries. Sadly, only a very small number of birth defects can currently be prevented. We hope research conducted in the Newlife Birth Defects Research Centre will aid in developing future treatments for children with these conditions."

Professor Adrian Thrasher and Professor Bobby Gaspar

"We started to work independently on gene therapy in the mid-90s when we were PhD students at the ICH. We began collaborating on one of the severest forms of immunodeficiency, and developed the first study in the UK – and only the second worldwide – to show that gene therapy can correct a genetic disease. We could also see the potential of gene therapy to correct other immune disorders and inherited diseases, but we also understood that to make the difference we needed to have a really broad research programme that stretched from basic science in the laboratory to clinical implementation in the hospital.

"The last decade has been an extremely exciting time, building up our laboratory and clinical team and seeing the programme expand to treat a number of different conditions. We have also been able to build significant relationships with other major international centres and have pioneered the adoption of multicentre trials. This aspect has been particularly stimulating and clinically very rewarding. We are fortunate to be in such an exciting and cutting-edge area of medicine."

Gene therapy

Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (ICH) have led global advances in treating children born with an inability to fight infections. As one of the first centres worldwide to offer this remarkable treatment, Professor Adrian Thrasher and Professor Bobby Gaspar's research has transformed the lives of children with these conditions.

"Just over 10 years ago, we became one of the few centres in the world to begin trials of a revolutionary new form of therapy for children born with a genetic disorder called X-linked severe combined immunodeficiency (X-SCID)," explains Adrian Thrasher, Professor and Honorary Consultant in Paediatric Immunology at GOSH and the ICH. Known as gene therapy, the team has successfully treated a group of children with X-SCID. This success has also been extended to children with three other rare immune system disorders: adenosine deaminase-SCID (ADA-SCID), X-linked chronic granulomatous disorder (CGD) and Wiskott-Aldrich syndrome.

Children with these conditions have a compromised immune system, which means they are unable to fight infection and have to live in a sterile environment. This is the result of a mutation in a gene that affects either the development or the function of cells in their immune system. The conventional treatment for these conditions is bone marrow transplantation. This can have excellent results when a fully matched donor is found, but results are less successful with an unmatched donor.

The team have been trialling a revolutionary new treatment, gene therapy, which uses the child's own cells to fix the genetic defect. A vector – a disabled virus – acts to place a working copy of the gene, manufactured in the laboratory, into a child's bone marrow cells. These modified cells are then reintroduced to the body. Now armed with the vital genetic instructions that they previously missed, these bone marrow cells grow into the full spectrum of immune cells, which are crucial for fighting disease. "We hope the promising results of our gene therapy programme mean it will now be seen as an alternative to standard treatment," says Bobby Gaspar, Professor of Paediatrics and Immunology at the ICH and Honorary Consultant in Paediatric Immunology at GOSH.

While the first generation of gene therapy trials proved remarkably effective, there were still many things to learn. This has enabled the development of much more sophisticated vector and trial designs. "In collaboration with other international groups we have recently begun several new clinical studies. Early indications are extremely promising," says Professor Thrasher, who is supported by the Wellcome Trust and Great Ormond Street Hospital Children's Charity.

As a world-leading centre for gene therapy, the team are also developing new approaches to fix the faulty gene itself. "At present we're able to efficiently place a new working copy of the gene into a child's cell. Ideally we would like to fix the gene at its own site using a cut-and-paste approach," explains Professor Gaspar, who is supported by Great Ormond Street Hospital Children's Charity and the Medical Research Council. The team are developing an alternative genetic method in which molecular 'scissors' cut the faulty gene away and replace it with the correct gene.

The team hopes to make gene therapy available to more patients with immune disorders in the future. "Our aim is to scale this up to more patients here and nationally so they too can benefit from the significant clinical improvements. Dedicated gene therapy facilities will give us the infrastructure to develop this form of genetic medicine for more patients," says Professor Thrasher.

The work exemplifies the unique partnership between GOSH and the ICH, combining scientific breakthroughs with medicine for patient benefit. Professor Gaspar adds: "A decade on, gene therapy has improved the life and health of many children with life-threatening immune diseases. With the tools we have available, we are broadening this therapy to treat other conditions such as metabolic disorders, HIV and skin disorders."

Nina's story
by her mum Aga

"When Nina was born, everything seemed fine, but after a period of time we noticed she was not feeding properly and was sleeping more than usual. We started to become concerned and doctors soon discovered she had developed multiple infections. Soon afterwards she was placed in isolation to stop her picking up infections. We had to scrub down and wear masks and gowns before we could see her.

"Doctors confirmed she had a life-threatening immune disorder and we were told that Nina would need a bone marrow transplant. As Nina's health deteriorated she was moved to GOSH.

"Within weeks of being transferred doctors diagnosed her with ADA-SCID. Looking back, this was a turning point. Nina started to receive vital treatment to replace a key enzyme her body was not producing. She started to put on weight and the improvements she was making offered the possibility of gene therapy as a treatment.

"Professor Bobby Gaspar discussed this option as an alternative to a bone marrow transplant and earlier this year she was given the treatment. Since the treatment, Nina's health has shown encouraging signs. However, we're still very cautious around her. We continue to have a strict regime of cleaning hands, surfaces and even sterilising toys to stop her coming into contact with infection as her immune system starts to build up.

"The doctors and nurses at GOSH have been amazing. They're always available to answer all our questions and their approach to treating Nina has been extremely reassuring. This has given us the hope that one day Nina will be able to lead a good quality of life."


How's life?
Take part in Life
Life Study will track the lives of b
and their families from pregnancy
through the early years.
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generations to come by joining Life
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antenatal clinic or checking out
www.lifestudy.ac.uk.

Professor Carol Dezateux
"My passion for paediatrics developed as a medical student. My interest in research came later, as a junior doctor looking after babies with acute bronchiolitis. I realised that if I wanted to satisfy my curiosity about its causes, I would need to research healthy babies before they became ill. A Wellcome Trust fellowship enabled me to develop my research ideas and bring laboratory science into populations.

"My research focuses on children's health in the early years and its improvement through prevention or early detection. My experiences of leading research to collect biological samples from young children in the UK Millennium Cohort Study taught me the importance of bringing together biomedical and social scientists at the outset when designing such studies.

"My ambition to develop a cohort with this cross-disciplinary approach was realised when our team was selected to design and lead the UK's new birth cohort study. I am privileged to direct this exciting study and look forward to working with parents to chart the early lives of their babies."

Life Study: Understanding lives now and for the future

Birth cohort studies follow the same group of children and their families from birth to adult life. These studies enable us to understand children's early family, social, cultural, economic and physical environments and development and how, when taken together, these factors influence future health, wellbeing and life chances. Professor Carol Dezateux is leading the largest UK birth cohort study to date, Life Study.

"We know from research based on the UK's earlier birth cohort studies that a child's experiences and their family and wider environment in the early years has a major impact on their future health, wellbeing and education," explains Professor Carol Dezateux, Director of Life Study[1], Professor of Paediatric Epidemiology at the UCL Institute of Child Health (ICH) and Honorary Consultant Paediatrician at Great Ormond Street Hospital (GOSH). "We need to understand this in more detail by focusing on pregnancy and the first year of life in babies and their parents from different family, social and ethnic backgrounds across the UK. For the first time, we have an exciting opportunity to bring biological, social and environmental sciences into a UK birth cohort study from the outset to understand how these influences combine to shape children's health and development."

Life Study will be the fifth in a series of world-renowned UK birth cohort studies. It builds on this exceptional legacy but at the same time has been designed with new features to ensure its future as a powerful and unique research resource well into the 21st century. It will involve up to 80,000 babies and their families – much larger than earlier studies. The rich and detailed information collected before birth, at birth and during the first year of life will address gaps in knowledge about these very early stages of life.

With this in mind, the study has several research themes chosen to answer important questions that matter to the health and lives of children now. One such theme addresses the rise in childhood obesity and physical inactivity – a major public health challenge. In order to reverse these trends we need to understand more about how eating and physical activity behaviours develop very early on in life.

"We will capitalise on the UK's strengths in all areas of social, environmental, clinical and biomedical science, drawing on advances in a wide number of disciplines. We will work together with leading researchers in many fields ranging from epigenetics to economics," says Professor Dezateux.

"Another exciting part of the study is the links we are developing with local and national research centres, for example through our interests in epigenetics and the early development of the immune system. This requires collecting a range of biological samples, such as placental samples, at the time of birth." Life Study is working with the National Institute for Health Research Biomedical Research Centre at GOSH and UCL to develop protocols for sample collection.

With recruitment to the study due to begin in 2014, Professor Dezateux recognises that partnership with the NHS and the public is key. "We can't do this study without the full support and commitment of clinicians and parents." Working with focus groups of parents and parents-to-be to find out what they think might encourage or discourage parents from taking part, Life Study is taking steps to achieve the participation of the thousands of families needed. "In a study as ambitious in size and scope as Life Study, we are committed to placing children and families at its heart. Families that take part will be supporting research that will make a real difference to our understanding of children's lives now and for the future," she adds.

[1] Life Study is funded by the Economic and Social Research Council, Medical Research Council, UCL and the Wellcome Trust, and benefits from the government's Large Scale Facilities Fund.

Dr Darren Hargrave
"I was inspired to devote my career to treating and researching childhood brain tumours by my mentor and friend Professor Eric Bouffet who I worked for at the Royal Marsden and Hospital for Sick Children in Toronto, Canada. My passion for translating the biology of disease to novel treatments for children with cancer came from my doctoral research. Under my supervisor, Professor Sir Mike Stratton, I learnt about the power of using genetic technology to unravel the driving forces of cancer.

"My research has combined these two influences to lead clinical trials of novel drugs in childhood cancer and specifically brain tumours. Working with Cancer Research UK, I recently completed a first-in-child trial of a new drug in children with relapsed cancer. I am the UK lead of two major international studies investigating targeted treatments for high-risk brain tumours, which are both running at GOSH."

Targeting brain tumours

Brain tumours are the second most common form of cancer in childhood. Great Ormond Street Hospital (GOSH) sees more children with brain tumours than any other centre in the UK. Dr Darren Hargrave, Consultant Paediatric Oncologist at GOSH, is collaborating to build a strategy that translates scientific discoveries into better therapies for children with brain tumours.

"Survival rates in certain types of brain tumour have vastly improved over the last couple of decades," says Dr Hargrave. "This has been achieved through clinical trials using combinations of conventional treatments such as surgery, radiotherapy and chemotherapy. However, for other types of childhood brain tumours, for example high-grade gliomas and diffuse intrinsic pontine glioma, the outcome has not significantly improved."

Dr Hargrave is leading a strategy that aims to develop new treatments for children with brain tumours where the current prognosis is poor. The programme brings together experts and resources from three of the largest paediatric brain tumour research and treatment centres in the UK. "To develop treatments for children with these high-risk brain tumours we have identified a series of collaborative research initiatives. These initiatives combine research into basic science, neuropathology, drug development and clinical investigations," explains Dr Hargrave, who is supported by Great Ormond Street Hospital Children's Charity, Cancer Research UK and the National Institute for Health Research Biomedical Research Centre at GOSH and UCL.

The starting point involves unravelling the causes of high-risk brain tumors. "Advances in DNA technology over the last 10 years have allowed us to improve our knowledge of individual brain tumours at an unprecedented rate and resolution. We can harness the power of this technology to identify specific molecular defects in these tumours," says Dr Hargrave.

At the other end, Dr Hargrave will use his vast experience in clinical trials to translate these scientific discoveries into patient benefit. He points to the recent success of this strategy in a brain tumour called medulloblastoma. "By analysing tissue samples from medulloblastoma, researchers have identified different molecular defects in this type of tumour. Working with a pharmaceutical company, we have developed a 'gene signature test' that predicts which patients will respond to a new targeted therapy and have successfully completed a phase I/II clinical trial." Dr Hargrave is now building on this result by leading a pivotal international phase III clinical trial that will test this novel therapy against conventional chemotherapy treatment for relapsed medulloblastoma.

Another vital aspect of his clinical research is to improve tests that measure the effects of cancer therapies. "Standard brain imaging techniques such as magnetic resonance imaging (MRI) provide you with information on the size and location of the tumour. However, these methods provide little in the way of how active the tumour is. This information is important when managing a child's treatment," he explains.

Working collaboratively with colleagues at University College Hospital, Dr Hargrave is combining these imaging techniques with nuclear medicine. This combination provides functional information about tumour activity such as blood flow and metabolism. "If we have more accurate measurements of tumour activity we will be able to inform children and their families much earlier about the effectiveness of treatment," says Dr Hargrave. Thanks to the support from the Dorothy and Spiro Latsis Benevolent Trust he is also looking to improve the diagnosis and monitoring of children with low grade glioma.

One of the key themes running through Dr Hargrave's clinical research has been the need to extensively collaborate with leading scientists both nationally and internationally. "We want to leverage the best science that already exists out there without reinventing the wheel and combine that with our experience of driving new drugs into clinical trials. At the heart of this effort is translating the work into patient benefit. I am hopeful that by the time I retire we will have introduced better treatments that will significantly improve the outcome for children with high-risk brain tumours."

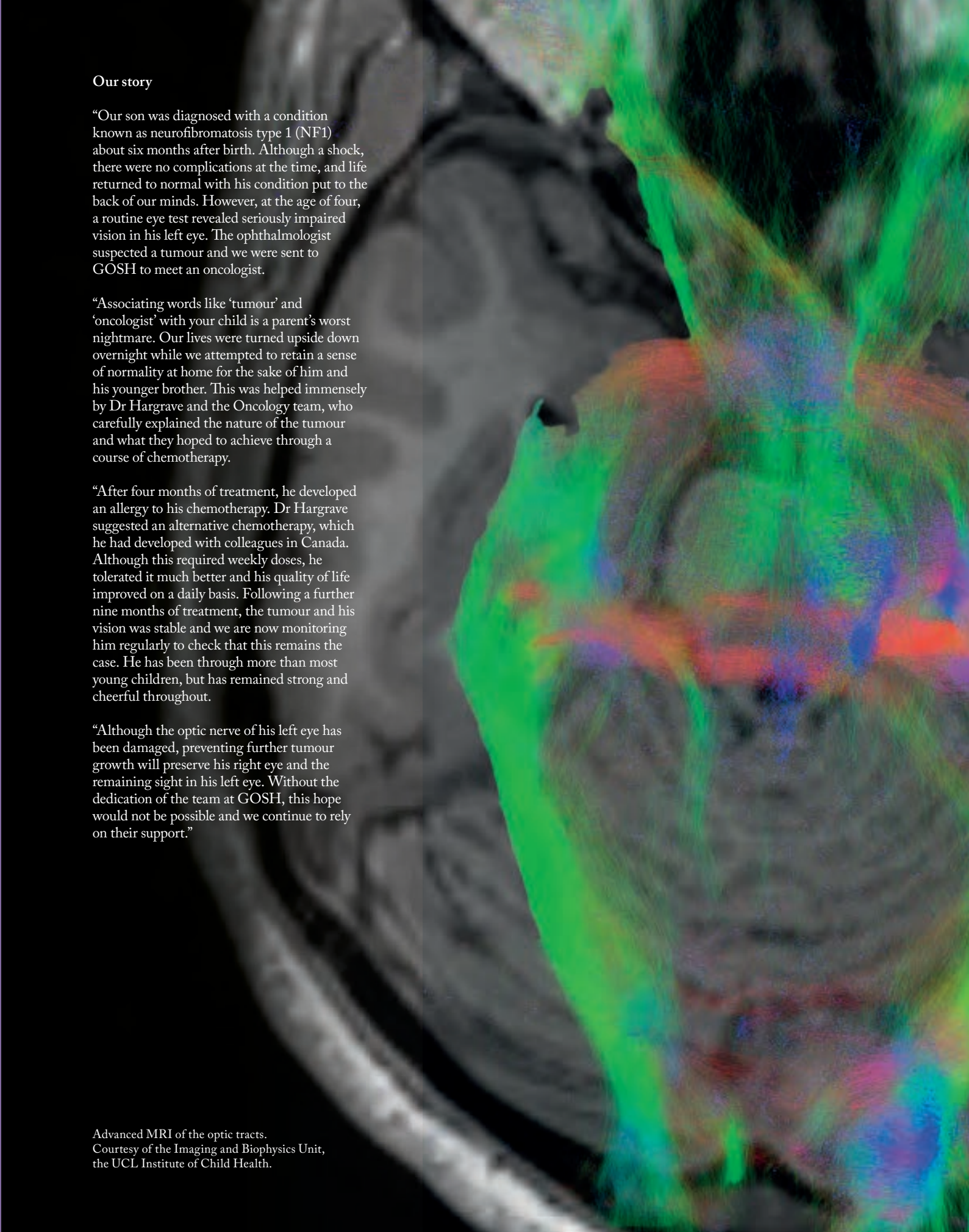
Our story

"Our son was diagnosed with a condition known as neurofibromatosis type 1 (NF1) about six months after birth. Although a shock, there were no complications at the time, and life returned to normal with his condition put to the back of our minds. However, at the age of four, a routine eye test revealed seriously impaired vision in his left eye. The ophthalmologist suspected a tumour and we were sent to GOSH to meet an oncologist.

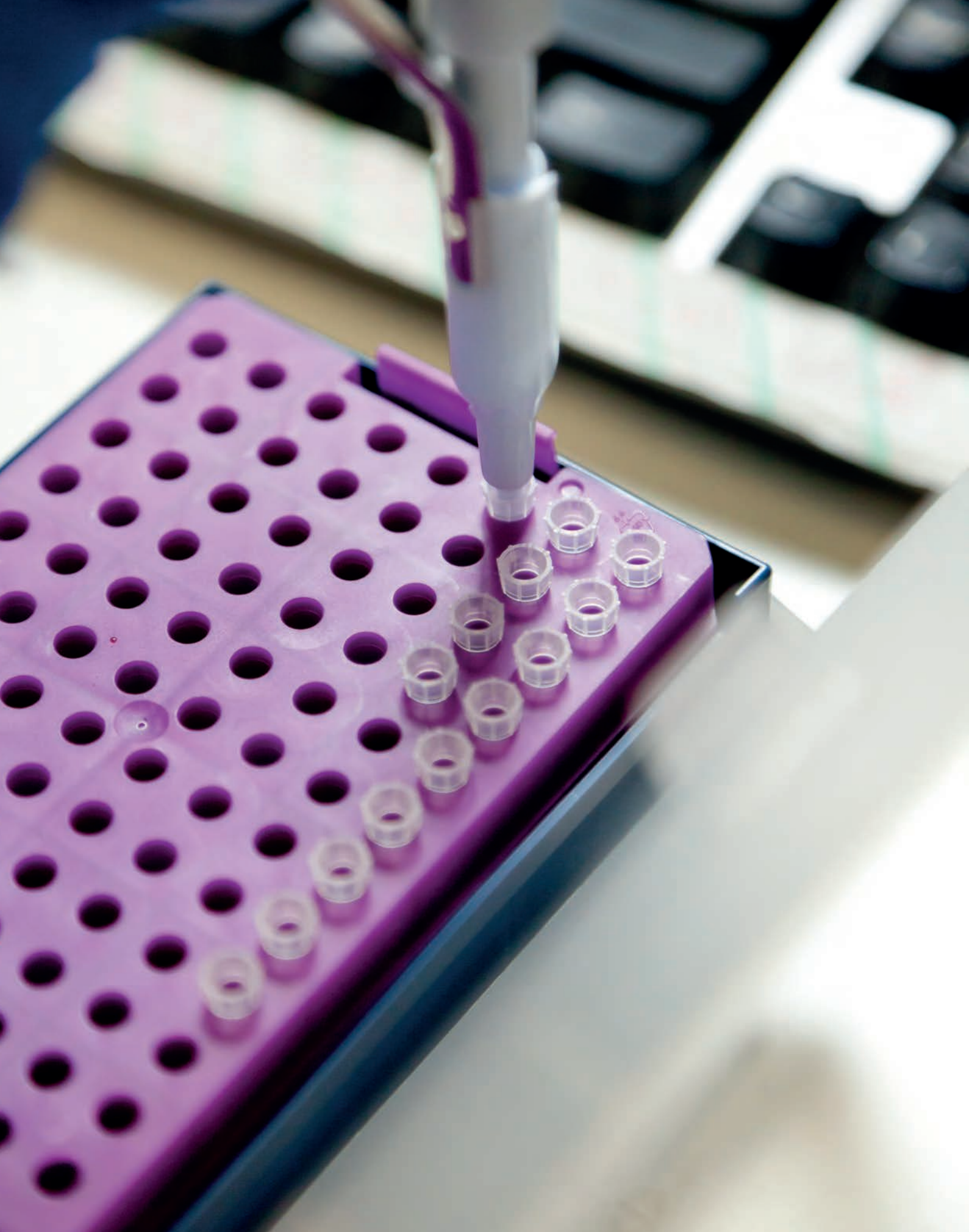
"Associating words like 'tumour' and 'oncologist' with your child is a parent's worst nightmare. Our lives were turned upside down overnight while we attempted to retain a sense of normality at home for the sake of him and his younger brother. This was helped immensely by Dr Hargrave and the Oncology team, who carefully explained the nature of the tumour and what they hoped to achieve through a course of chemotherapy.

"After four months of treatment, he developed an allergy to his chemotherapy. Dr Hargrave suggested an alternative chemotherapy, which he had developed with colleagues in Canada. Although this required weekly doses, he tolerated it much better and his quality of life improved on a daily basis. Following a further nine months of treatment, the tumour and his vision was stable and we are now monitoring him regularly to check that this remains the case. He has been through more than most young children, but has remained strong and cheerful throughout.

"Although the optic nerve of his left eye has been damaged, preventing further tumour growth will preserve his right eye and the remaining sight in his left eye. Without the dedication of the team at GOSH, this hope would not be possible and we continue to rely on their support."



Advanced MRI of the optic tracts.
Courtesy of the Imaging and Biophysics Unit, the UCL Institute of Child Health.



Life-saving vaccines

Immunising children is all about preventing infections and saving lives. As Head of the Immunobiology Unit at the UCL Institute of Child Health, Professor David Goldblatt's work has contributed to the introduction of life-saving vaccines into the UK childhood immunisation programme. His global efforts could also lead to better immunisation strategies for children in developing countries.

Professor David Goldblatt

"Recognising the signs of a disease and providing treatment is important for a medical doctor, but from early on in my medical student days in Cape Town, I was curious about the causes and origins of diseases.

"My desire to pursue research was stimulated when I arrived at GOSH in 1986. I was working as a Senior House Officer on the haematology/oncology ward, where children undergoing bone marrow transplantation were cared for. I was helping to look after a child who had undergone a bone marrow transplant because of an immune deficiency. This experience and contact with the Immunology team, led at that time by the late Professor Roland Levinsky, a fellow South African, stimulated my interest in immunology.

"I always had a natural interest in infectious diseases, and so during my PhD I combined these two interests to look at immune responses to infection. Following this, there was a very clear link to understanding the immune response to vaccines, so in the early 1990s I set up my vaccine evaluation laboratory.

"Seeing the impact of one's vaccine research on children's lives makes this an extremely rewarding area to work in."

"Childhood immunisation programmes have been extremely effective in protecting children from serious infections," says Professor Goldblatt, Director of Clinical Research and Development at Great Ormond Street Hospital (GOSH) and Director of the National Institute for Health Research Biomedical Research Centre at GOSH and University College London. His laboratory has been at the forefront of efforts to introduce new life-saving vaccines into these programmes.

"In the early 2000s, a new vaccine became available with the potential to prevent childhood infections caused by the bacterium, *Streptococcus pneumoniae* (pneumococcus). At that time, pneumococcus was the leading cause of bacterial meningitis in the UK. While the UK was keen to introduce this vaccine, infants in the UK were already receiving two injections at each immunisation visit, and this would add a third injection to the schedule, thought by some to be unacceptable. We therefore tried to devise a way of delivering the new pneumococcal conjugate vaccine (PCV) using fewer doses than recommended on the licence at the time, and thus administering fewer injections," explains Professor Goldblatt.

Working collaboratively with Public Health England (formerly the Health Protection Agency) Professor Goldblatt's team were the first in the world to show that PCV was likely to provide protection with a total of three rather than the recommended four doses. The study provided the evidence needed for the UK government to add this life-saving vaccine to the UK infant immunisation programme in 2006. Now routinely given to all children, the vaccine provides significant protection to children from these devastating infections. Capitalising on the UK experience, countries all over the world have introduced PCV as a routine childhood immunisation with only three doses.

Building on this success, Professor Goldblatt has also focused on studying ways of reducing childhood rates of infectious disease in developing countries. "In some parts of the underdeveloped world, such as Africa, there is a very high exposure to the pneumococcus early in life. Here the World Health Organisation (WHO) immunisation scheme is followed, with infants beginning vaccinations at six weeks of age. However, by six weeks of age in some African countries, 15 per cent of serious pneumococcal disease will have already occurred. We therefore performed a study with colleagues at the Kenya Wellcome Trust unit in Kilifi to assess whether we could protect infants earlier by administering PCV at birth," says Professor Goldblatt.

"Our work showed two things. Firstly, and most importantly, vaccination at birth was safe. Secondly, the dose given at birth was able to induce antibody responses. Giving the first dose of vaccine at birth could provide young infants an earlier window in which to be protected from these serious infections. Furthermore, incorporating immunisation at birth may improve vaccine coverage in countries where access to healthcare facilities is challenging."

Thanks to support from the Department of Health, Wellcome Trust, WHO and the Gates Foundation, Professor Goldblatt reflects on the different global challenges facing immunisations. "As we get better at providing vaccines to the infant population in the Western world, many families thankfully do not see the consequences of infectious disease. The challenges here are to maintain high levels of vaccination, as a reduction in the overall percentage of infants immunised could lead to a resurgence of disease as we have seen recently with measles."

"In contrast, the risk of disease in the developing world is all too evident. Here the challenges are to ensure vaccination is provided and accessible to all children and that new vaccines, such as PCV, are rapidly made available and affordable in resource-poor settings," adds Professor Goldblatt.

Fay's story
by her mum Lucy

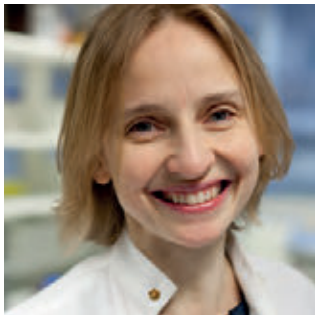
"It was only after Fay was born that we realised something was wrong. She was born with a very large dark and disfiguring mark that covered about 70 per cent of her body. Although we were later informed she had congenital melanocytic naevus (CMN), we knew little about this condition. All I wanted to do was go home and wrap my little family in a cocoon.

"When Fay turned six months she was referred to GOSH. Initially we were seen by Dr David Atherton, and since then we have been under the care of Dr Veronica Kinsler. They helped explain Fay's condition in detail and were able to answer all of our questions. Through their support we have become more understanding of Fay's condition.

"At eight months, Fay had an MRI scan, which thankfully showed no underlying problems beneath the skin. Fay now attends Dr Kinsler's clinic every four months where she is monitored closely due to the extent of her CMN. The doctors at GOSH have been amazing.

"Fay does not let her condition stop her doing what she wants to do. She is a very bright, happy and bubbly girl with a lot of confidence. She particularly enjoys street dancing and has entered competitions up and down the country.

"We are extremely grateful to Dr Kinsler for her commitment and dedication to research into skin disorders. The breakthrough she made in identifying what causes CMN highlights how important research is to us and children like Fay and their families. We never thought such an achievement would be made in such a short space of time."



Getting under the skin of birthmarks

Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (ICH) are leading research into a rare skin condition. Starting in the 1980s, the Paediatric Dermatology department established a database for affected individuals in the UK. Earlier this year, Dr Veronica Kinsler made a breakthrough in identifying the cause of this condition.

Dr Veronica Kinsler

"I was inspired to do this research by my long-term clinical mentor, Dr David Atherton, and by the desperate lack of treatments for children with CMN. The patient support group, Caring Matters Now, generously funded my salary for three years to allow me to start researching, and this helped me to obtain a prestigious research fellowship from the Wellcome Trust.

"I joined Professor Gudrun Moore's team in Genetics at the ICH, and very excitingly we found the gene that causes CMN, for which I received a prize from the Academy of Medical Sciences in 2013, and the CDA trophy from the British Association of Dermatologists in 2013.

"I am very proud to be part of GOSH and the ICH, one of the few places in the world to investigate rare paediatric skin diseases. My ambition is to take this research forward to find treatments for CMN, and to investigate other untreatable skin diseases in children."

"Multiple congenital melanocytic naevi, or CMN, causes large dark moles on the skin that are present at birth. They can vary in size and number and the incidence of larger or multiple moles is approximately one in 20,000 new births per year. The majority of children born with this condition in the UK will be seen at GOSH," says Dr Veronica Kinsler, Academic Lead Clinician for Paediatric Dermatology at GOSH and the ICH.

Such conditions cannot be picked up on ultrasound scans during pregnancy, leaving parents with a considerable shock when their baby is born. Referrals to a place like GOSH, which has the expertise in the diagnosis and management of CMN, mean parents are provided with much-needed information early on. In the weekly clinics run by Dr Kinsler, moles are checked to ensure they are safe and children are assessed to see whether the brain needs to be checked with a scan.

Earlier this year, in a study led by Dr Kinsler, the team made a major breakthrough in identifying the cause of CMN. "This is an important discovery for children who have CMN. The discovery of the genetic cause means we can now start to look for ways to treat this serious condition," she says.

The breakthrough is the discovery that this condition is caused by a mutation in a gene called NRAS. This mutation occurs while the baby is growing in the womb, meaning that parents can be reassured that the condition is not inherited and cannot be passed on to future children. This gene mutation is found in at least 80 per cent of the patients with CMN.

The other 20 per cent are still being investigated to determine the cause of the condition in their cases.

This research could also help understand other conditions. The gene pinpointed in the study is known to be involved in the development of melanomas, a particular type of skin cancer. It explains why patients with CMN have an increased risk of melanoma, and the researchers found that the patients need to develop a second mutation to change the moles into cancer.

Building on this successful finding, Dr Kinsler is keen to accelerate research into other rare dermatology diseases. Earlier this year, she set up the GOSH Rare Dermatology Diseases Resource. "This resource harnesses the enormous potential that exists within the Paediatric Dermatology department at GOSH. It aims to overcome some of the challenges faced when conducting research into these conditions. By systematically collecting samples from patients who attend our clinic, we can accelerate research into identifying the causes of these conditions and work towards developing new treatment options," says Dr Kinsler. Now as part of the Livingstone Skin Research Centre for Children she has also been supported by the Wellcome Trust, Caring Matters Now, Leah Wigmore Melanoma Fund and the Butterfly AVM Charity.

To date, the resource has already recruited 50 families. "It also enables doctors at the hospital to put families in touch with others with the same diagnosis if they wish. This provides support to children and families similarly affected by these conditions," she adds.

Professor John Achermann
"I have always had an inquisitive mind, feeling the need to push the boundaries of our scientific knowledge and combining this with finding better ways to support patients. During my paediatric medical training, I was exposed to the field of endocrinology (hormones). The attraction to endocrinology was the broad spectrum of science, ranging from genetics to psychology.

"After completing a doctorate in London, I undertook a three-year fellowship at Northwestern University in Chicago. This led to a specialist interest in adrenal and reproductive biology.

"When I came back to the UK I was able to obtain a prestigious clinician scientist award from the Wellcome Trust, who have since supported me through their clinician scientist programme. I have been extremely grateful for these opportunities as they allow me to strike a balance between inquisitive research and research that can translate into patient benefit.

"I've always enjoyed working with children. There's a great sense of satisfaction when you are able to help a child and support their family. Being part of a multidisciplinary team is also fulfilling as you learn to appreciate the contributions different healthcare professions make to overall care, and respect everyone's role in the process."

Reproductive development

Disorders of sex development (DSD) are a group of rare conditions affecting the development and function of the reproductive organs. Professor John Achermann, a Wellcome Trust Senior Research Fellow in Clinical Sciences at the UCL Institute of Child Health, combines his clinical expertise in caring for children with DSD and their families with his scientific work aimed at finding the cause of these conditions.

"DSD often come to our attention because of variations in the way a baby's genitals appear at birth. These conditions can also first present when a child doesn't enter puberty at adolescence or in some forms of infertility in later life," says Professor Achermann. He strongly believes that trying to achieve an exact diagnosis early on is an important step in helping children and their families manage these challenging conditions.

Since 2006, as an Honorary Consultant in Paediatric Endocrinology at Great Ormond Street Hospital (GOSH), Professor Achermann has been involved in the hospital's DSD multidisciplinary team. Consisting of specialists in urology, gynaecology, biochemistry, genetics, psychology and endocrinology, the DSD team aims to carefully plan the management of children with these conditions. "Disorders of sex development can have a wide range of causes, each requiring a potentially different approach to their management," explains Professor Achermann. "Working as a multidisciplinary team is essential in pooling everyone's skill and expertise."

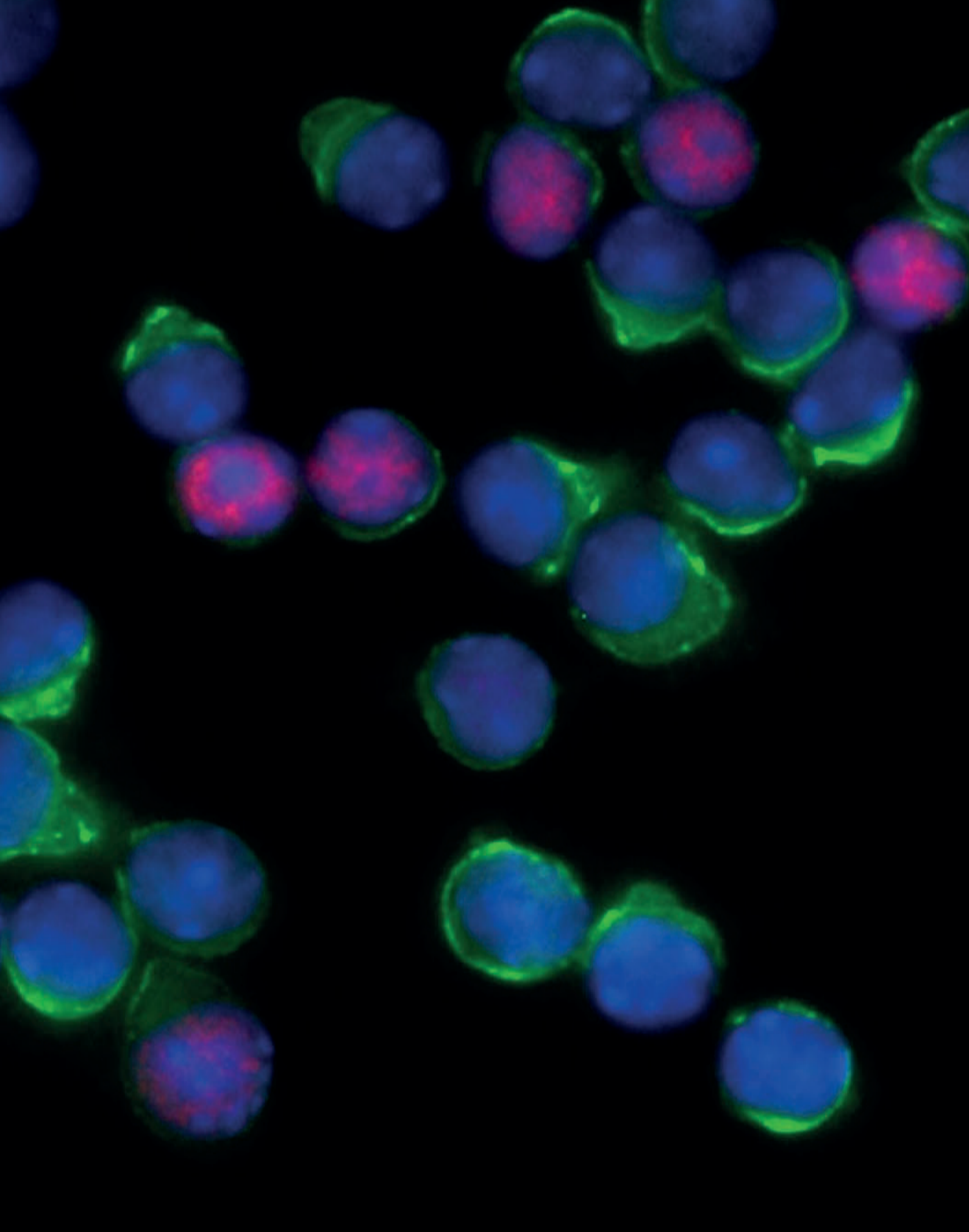
He adds: "The optimal care for each child has to be considered on an individual basis. We gather as much information as possible early on through careful examination, blood tests, genetic tests and biopsies if appropriate, to allow us to make the best recommendations. We take a long-term approach and need to consider factors such as the child's likely gender identity when they grow up, options for fertility and any potential risk of developing tumours. At the core of this is working closely with families to provide care and support that are in the best interests of the child."

Much of Professor Achermann's laboratory research has focused on the role of a transcription factor, responsible for turning genes on and off, called steroidogenic factor-1 (SF-1). His work has established that disruption of SF-1 occurs in around 20 per cent of children with DSD. "We have known for some time that SF-1 plays a key role in the development of the reproductive organs. What is now emerging is that changes in the gene coding for SF-1 are responsible for a spectrum of conditions including some that present later in life," says Professor Achermann.

Working collaboratively with researchers in France, his team discovered that genetic alterations in SF-1 are responsible for some forms of primary ovarian insufficiency, a condition where the ovaries stop functioning normally in women who are younger than 40. The research offers the hope of developing a possible diagnostic test, particularly for women with a family history of premature ovarian failure, or in the evaluation of prospects for treatment of primary ovarian insufficiency.

His team are also investigating cases where a specific diagnosis cannot currently be reached. "One approach our laboratory has taken is to manipulate the levels of SF-1 in cells to identify new genes that might be controlled by SF-1. In parallel, we are collecting samples from patients to see whether these newly identified genes are involved where the cause remains unknown. We hope that by understanding the biology of reproductive development we will be able to improve diagnosis and help us to manage these conditions better, both in the shor-term and long-term," says Professor Achermann.

Reflecting on this clinical work, he recognises that an exact diagnosis is just the first step. "Our DSD clinic means we are able to offer children and their families early support and assessment by an experienced multidisciplinary team. We would now like to define better pathways of management and support for individuals and families with DSD and to provide a seamless transition in long-term support and management as children become adults."



Defending the body while tolerating ourselves

The immune system works to protect us against a variety of infections. At the same time, it is programmed to ignore normal healthy cells. Dr Masahiro Ono, Senior Research Fellow at the UCL Institute of Child Health's Immunobiology Unit, is taking a multidisciplinary approach to understand how a group of disease-fighting cells maintain this fine balance.

Dr Masahiro Ono

"I first trained as a dermatologist in Japan, where I became aware of how certain diseases had limited options for treatment. In those early days, I was involved in collaborations with agricultural scientists to diagnose a rare fungal infection. This study revealed a deficit in a patient's immunity. The experience not only taught me the importance of multidisciplinary research, but ignited my interested in the immune system.

"I later trained as an immunologist and began specialising in research into the regulation of the immune system using genes involved in autoimmune diseases such as rheumatoid arthritis and multiple sclerosis.

"Since being awarded a BBSRC Fellowship, and with the broad expertise I have developed throughout my career, I am aiming to identify a novel way of controlling the immune system, and thereby contribute to the improvement of children's health."

"Our immune system is made up of a complex network of cells that co-ordinate the body's defense against disease-causing organisms", explains Dr Ono. "At the heart of this system are a group of disease-fighting cells known as T-cells. Their importance is highlighted in conditions such as HIV where the virus specifically attacks them, rendering the body susceptible to infections."

"While the immune system plays this vital role in providing protection, it also needs to strike a delicate balance to ensure normal healthy parts of the body remain untouched. For example, in conditions such as multiple sclerosis and rheumatoid arthritis, or following organ transplantation, T-cells can mislead the immune system to attack the body. Understanding how exactly this precise balance is preserved has been a key question in immunology," says Dr Ono.

He believes that by learning how T-cells can activate or suppress the immune system to maintain this balance is essential in understanding how the immune system fights infection while tolerating the body. His research focuses on two subpopulations of T-cells, regulatory T-cells and memory T-cells.

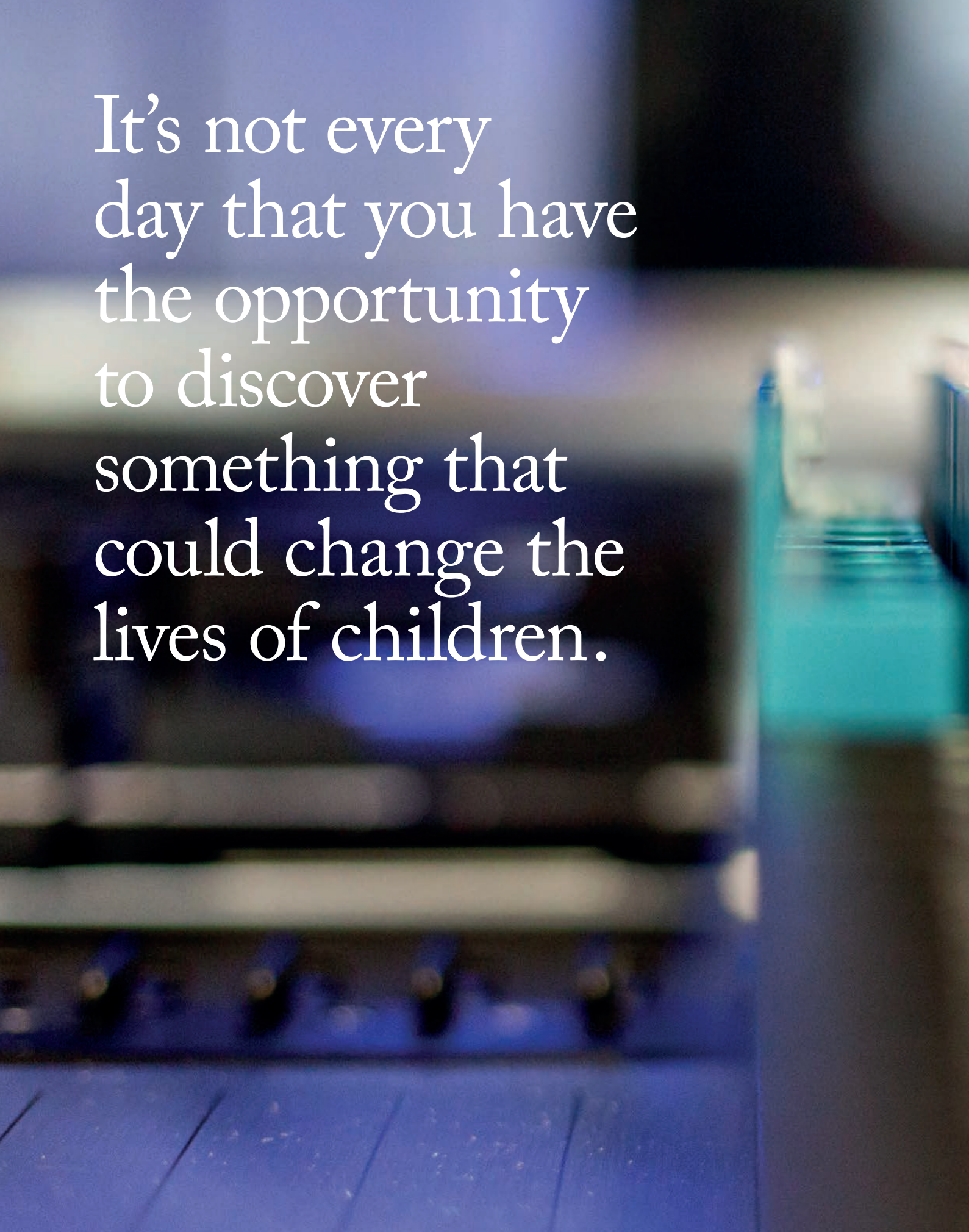
Regulatory T-cells suppress the immune response after eliminating a foreign organism. However, oversuppression of the system can compromise the body's ability to fight an infection. During his early career in Japan, Dr Ono identified a key gene switch by which regulatory T-cells exert their effect. "Our research revealed that a key gene in regulatory T-cells, Foxp3, was hijacking the genetic machinery of normal T-cells and turning them on or off," says Dr Ono.

Building on this discovery he is now investigating the counterpart of regulatory T-cells: memory T-cells. "The immune system has a remarkable ability to 'remember' the strategy it used to defeat a previous infection – a function performed by memory T-cells. This enhances our ability to fight common infections. Nevertheless, it can be a hindrance in autoimmune conditions where it continues to remember healthy parts of the body as a foreign object," says Dr Ono. He is now researching which genes are involved in activating memory T-cells, following the award of a prestigious Biotechnology and Biological Sciences Research Council (BBSRC) David Philips Fellowship.

"One approach we're taking is to see how, when and where memory T-cells are activated," explains Dr Ono. He draws comparison of this work with the neuroscience field. "In neurosciences, visualisation techniques have very much advanced this area. By visualising the activity of the immune system at the gene level, we will be able to identify how memory T-cells are controlled, and how immunological memory is formed."

While understanding individual T-cell functions remains important for Dr Ono, the future lies in piecing together the complex nature of the immune system. He adds: "The immune system is a multidimensional system. It relies on an elaborate and dynamic communications network that exists among the many different kinds of immune cells. In order to effectively manipulate and specifically control the immune system, we need to fully understand the interactions between all T-cells. This will be critical for the future development of new medicines."

Visualising the immune system. Pink cells are regulatory T-cells, while those not in pink include memory T-cells.


It's not every
day that you have
the opportunity
to discover
something that
could change the
lives of children.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Dr Grunewald

Working with UCL Business PLC

UCL Business PLC (UCLB) is responsible for managing and exploiting intellectual property (IP) arising from both Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (ICH).

UCLB's work aims to maximise the positive social, health and economic benefits of discoveries made at GOSH and the ICH. UCLB's team of highly experienced business managers work closely with researchers and clinicians to bring technologies through the journey from invention to market.

This year, UCLB launched an exciting new joint proof of concept fund with the National Institute of Health Research Biomedical Research Centre at GOSH and University College London. This funding supports the essential work required to transform an idea developed by our local paediatric researchers and clinicians into a proven innovation. In addition, a number of new patents were filed and UCLB looks forward to supporting and promoting the development of these new approaches over the coming months and years.

To enable anaesthetists to have ready access to potentially life-saving drug delivery information, Dr Philip Cunnington and his colleagues from the Anaesthetic department at GOSH are developing an iPhone app based on their Drug Administration and Departmental Practice Guidelines. Through proof of concept funding, UCLB is supporting the development of this application as well as licensing the app to UBQO Ltd, a specialist phone app developer, for worldwide distribution.

As part of the Face Value project, funded by Great Ormond Street Hospital Children's Charity, Consultant Paediatric Neurosurgeon Mr Owase Jeelani and Consultant Plastic and Reconstructive Surgeon Mr David Dunaway are developing novel bone distraction devices that will be used during craniofacial surgery. UCLB is providing IP and patent support, and through proof of concept funding, its specialist subsidiary, UCLB Devices Ltd, is supporting the regulatory aspects of the project.

A new patent has been filed by Dr David Long and Professor Paul Winyard at the ICH who are exploring novel ways to treat polycystic kidney disease. They have discovered that targeting the lymphatic system, using growth-factor modulating agents, has a therapeutic effect in laboratory models of the disease. UCLB is currently approaching commercial partners to develop these findings into new medicines for patients.

Professor Simon Heales, in collaboration with colleagues at the UCL Institute of Neurology and Royal Holloway, University of London, continues to develop new fatty acids with the potential to control neurological seizures. Working closely with Vitaflo, a UK company specialising in foods for special medical purposes, they have filed several patents in this area. UCLB aims to develop this innovation into a food formulated for dietary management of epilepsy.

Other notable advancements include the sale of data presentation software, developed by Mr Jez Philips and colleagues at GOSH, to another major NHS trust. In addition, a paediatric patient recorded outcome measure for pain, developed by Dr Anne Hunt (now at the University of Central Lancashire) in collaboration with the Royal College of Nursing, was sold to a major pharmaceutical company conducting clinical studies of new therapies for pain.

In collaboration with GOSH and ICH, UCLB is supporting the development of new social enterprise initiatives. Social enterprise is a vibrant and growing sector, which offers significant potential to strengthen the use of scientific evidence for practice at local and community levels, and to help demonstrate the impact that research can have on the lives of people.

One such project emerging from the research of Professor Atul Singhal and Dr Julie Lanigan is the Trim Tots programme. Reaching out to children and families, Trim Tots aims to promote healthy living within the community. Looking to the future, UCLB is working closely with this team to develop a social enterprise that will aim to maximise the social impact of this obesity intervention for preschool age children.

UCLB looks forward to continuing its work with GOSH and the ICH to ensure that new ideas have the potential to become a real device, diagnostic tool or treatment for the benefit of children.

For additional information, please contact
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UCLB Business

Awards, honours and prizes

Staff from Great Ormond Street Hospital and the UCL Institute of Child Health received national and international recognition for their research achievements during 2012/2013.

Professor John Achermann was awarded a renewal of his Wellcome Trust Senior Clinical Research Fellowship.

Dr Elham Al-Jaaly was awarded a PhD for her thesis, *Factors affecting nutritional status and eating behaviours in Saudi Arabian adolescent girls*.

Dr Maria Alonso-Ferrero received joint first prize in the British Society for Gene Therapy poster competition.

Dr Cynthia Andoniadou was awarded the European Society of Endocrinology Poster Prize at the Joint 15th International Congress of Endocrinology and 14th European Congress of Endocrinology.

Dr Owen Arthurs was awarded a National Institute for Health Research Clinician Scientist Fellowship.

Dr Diane Berry was awarded a PhD for her thesis, *Analysing the association of vitamin D status on selected cardiovascular risk markers using seasonal and genetic variations.*

Dr David Beran was awarded a PhD for his thesis, *Developing a hierarchy of needs for type 1 diabetes.*

Dr Debbie Briggs was awarded a PhD for her thesis, *An investigation into sub-populations of satellite cells and myoblasts.*

Dr Claire Booth was awarded a PhD for her thesis, *Lentiviral vector mediated gene therapy for X-linked lymphoproliferative disease.*

Dr Emma Chan was awarded a PhD for her thesis, *Lentiviral gene therapy for HIV using TRIM-cyclophilin restriction factors.*

Dr Timothy Colbourn was awarded a PhD for his thesis, *Investigating the use of women's groups in Malawi: adapted quality of life measurement, best-worst scaling choice experiments and contingent valuation.*

Professor Anthony Costello was appointed to the role of Pro-Provost for Africa and the Middle East.

Professor Mehul Dattani was elected as Chair of the Programme Organising Committee of the European Society for Paediatric Endocrinology.

Dr Rachel Denholm was awarded a PhD for her thesis, *Investigating the effects of child maltreatment and household dysfunction on child physical development in a British birth cohort.*

Professor Carol Dezateux was awarded WellChild Researcher of the Year.

Dr Robert Edgar was awarded a PhD for his thesis, *Kallikrein inhibition in major surgery.*

Dr Despina Eleftheriou was awarded a PhD for her thesis, *Endothelial injury and repair in childhood arterial ischaemic stroke.*

Dr Jonathan Fisher was awarded a Wellcome Trust/Sparks Research Training Fellowship.

Dr Jonathan Fishman was awarded a Medical Research Council Centenary Fellowship. He won the Wesleyan-Royal Society of Medicine Young Trainee of the Year Award and was also awarded best paper/manuscript of the year by the *Journal of Laryngology and Otology*.

Dr Alessandra Frigiola was awarded a MD(Res) for her thesis, *Right ventricular outflow tract dysfunction following repair of congenital heart disease: surgical solutions and clinical implications.*

Professor David Goldblatt was awarded a National Institute of Health Research Senior Investigator Award.

Dr Michelle de Haan received the Distinguished International Alumni Award from the University of Minnesota.

Dr Dougal Hargreaves was awarded a Harkness Fellowship in Health Care Policy and Practice by the Commonwealth Fund, New York, and supported by the Nuffield Trust and National Institute of Health Research.

Professor Peter Hindmarsh became a member of the Council of Healthcare Professionals, Diabetes UK.

Dr Rebecca Hope won a prestigious Frank Knox Fellowship to undertake The Master of Public Health degree in Harvard.

Dr Miho Ishida was awarded a PhD for her thesis, *The role of imprinted genes in human fetal growth.*

Dr Tomas Jacques became Secretary to the clinical practice group of the British Neuropathological Society.

Dr Sujatha Jayakody was awarded a PhD for her thesis, *Investigating genetic factors underlying hypopituitarism and septo-optic dysplasia in humans.*

Dr Peter Jones was awarded a PhD for his thesis, *Haemodynamic instability during the intubation of critically ill children.*

Dr Jacqueline Jonuschies was awarded a PhD for her thesis, *The assessment of lentiviral vectors for transgene expression in myoblasts.*

Dr Veronica Kinsler was awarded a PhD for her thesis, *Studies of congenital melanocytic naevi.* She was also awarded the CDA trophy from the British Association of Dermatologists and was the winner of a poster competition at the Academy of Medical Sciences.

Dr Jane Kirkby was awarded a PhD for her thesis, *Applications and interpretation of paediatric lung function tests in health and disease.*

Dr Manju Kurian was awarded a Wellcome Trust Intermediate Clinical Fellowship.

Dr Stavros Loukogeorgakis was awarded a Wellcome Trust Postdoctoral Training Fellowship.

Awards, honours and prizes
continued

Dr Panagiotis Maghsoudlou was awarded a Medical Research Council Centenary Fellowship.

Dr Anna Matheson was awarded a PhD for her thesis, *The influence of facial motion on the neural response during emotion perception in typical and atypical development.*

Dr Fiona McElduff was awarded a PhD for her thesis, *Models for discrete epidemiological and clinical data.*

Dr Halima Moncrieffe was awarded an Arthritis Research UK Travelling Fellowship.

Professor Gudrun Moore was elected Fellow ad eundem of the Royal College of Obstetricians and Gynaecologists.

Professor Marie-Louise Newell was awarded the Fellowship of the University of KwaZulu-Natal in South Africa and was also elected to the Fellowship of the Academy of Medical Sciences.

Dr Gemma Northam was awarded the Neonatal Society Young Investigator Prize.

Dr Masahiro Ono was awarded a Biotechnology and Biological Science Research Council David Phillips Fellowship.

Dr Anna Pearce was awarded a Medical Research Council Population Health Scientist Fellowship.

Dr Valerie Pereira was awarded a PhD for her thesis, *The effect of maxillary advancement on speech, nasality and velopharyngeal function in cleft lip and palate.*

Professor Kathy Pritchard-Jones was elected to the Fellowship of the Academy of Medical Sciences.

Dr Shamima Rahman gave the plenary lecture to the Annual Symposium of the Society for Endocrinology.

Dr Saba Raza-Knight was awarded a PhD for her thesis, *The role of Zic2 in spinal neural tube orphogenesis.*

Dr Suzanne Rix was awarded a PhD for her thesis, *The role of IFT80 in the molecular pathogenesis of short rib polydactyly syndromes.*

Dr Michele Rosato was awarded a PhD for his thesis, *How does community mobilisation through women's groups work? Addressing the social determinants of maternal and child health in rural Malawi.*

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

Dr Gayatri Sharma was awarded a PhD for her thesis, *Methods for the measurement of urinary biomarkers of oxidative stress-application to type 1 diabetes mellitus.*

Dr Ameenat Olufunmilola Solebo was awarded a PhD for her thesis, *National study of primary intraocular lens implantation in children aged < two years old with congenital and infantile cataract.*

Professor Lewis Spitz received an honorary Fellowship from the American College of Surgeons. He also received the Ladd Medal of the American Academy of Paediatrics.

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

Dr Holly Stephenson was awarded a PhD for her thesis, *Innate immune recognition of glycosylated surface determinants of Campylobacter jejuni.*

Professor Terence Stephenson was appointed as the new Chairman of the Academy of Medical Royal Colleges.

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

Dr Gilma Olaya Vega was awarded a PhD for her thesis, *Development and evaluation of the effects of new complementary feeding guidelines with an emphasis on red meat consumption on iron and zinc status and growth in infants living in Bogota, Colombia.*

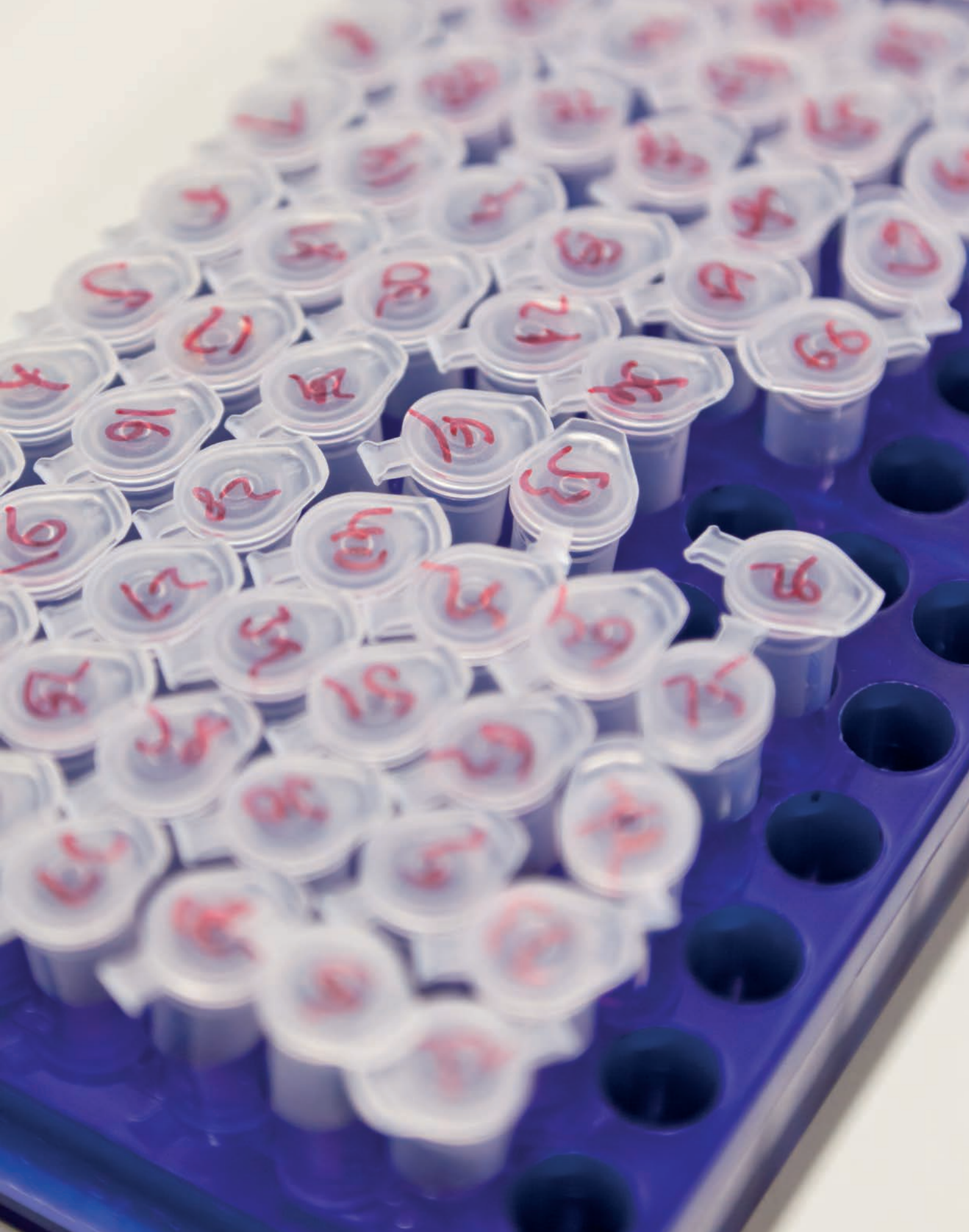
Dr Stefania Vergnano was awarded a PhD for her thesis, *Verbal autopsy for stillbirth and neonatal deaths – comparing population cause specific mortality fraction using two methods.*

Dr Aoife Waters was awarded a Medical Research Council Clinical Research Training Fellowship.

Dr Emma Webb was awarded the Donald Paterson Prize by Royal College of Paediatrics and Child Health.

Professor Bryan Winchester was awarded a 'Clé du Lysosome' by the organisation Vaincre les Maladies Lysosomales.

Dr Michael Yoong was awarded a PhD for his thesis, *Convulsive status epilepticus: prolonged childhood seizures and their consequences*.



Grants and donations

Great Ormond Street Hospital and the UCL institute for Child Health continue to receive grants and donations towards research from the following individuals and organisations:

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AFM Téléthon (Association Française contre les Myopathies)
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D
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Higher Education Funding Council for England
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R
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Rosetrees Trust
The Royal Academy of Engineering
Royal College of Paediatrics and Child Health
The Royal College of Surgeons of England
Royal National Institute of Blind People
The Royal Society

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CANCER THEME

Theme Leader
Professor Kathy Pritchard-Jones

Molecular Haematology and Cancer Biology Unit

Reader in Molecular Neurobiology and Head of Unit
Dr Jonathan Ham

Hugh and Catherine Stevenson Professor of Cancer Biology
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Dr Jessica Bate

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Dr David Michod
Dr Mark Weeks

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Professor Christopher O'Callaghan (from June 2012)

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

Professor of Respiratory Physiology
Professor Janet Stocks

Professor of Respiratory Physiology
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Professor David Hatch

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Dr Mark Peters

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Clinical Senior Lecturer
Dr Suellen Walker

Senior Research Fellow
Dr Sook-Yuen Lum

Senior Research Associate
Dr Rachael Gregson

GENERAL AND ADOLESCENT PAEDIATRICS THEME

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General and Adolescent Paediatrics Unit

Nuffield Professor of Child Health and Head of Unit
Professor Terence Stephenson

Professor in Adolescent Health
Professor Russell Viner

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Emeritus Professor of Paediatric Gastroenterology
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Professor Brent Taylor

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Dr Alastair Sutcliffe

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Dr Sophie Khadr

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Professor Helen Roberts
Professor Linda Franck

Senior Research Associate
Dr Beverley Botting

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

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Professor of Clinical and Molecular Genetics and Head of Unit
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Professor of Paediatric Metabolic Disease and Hepatology
Professor Peter Clayton

Professor of Paediatric Endocrinology
Professor Mehul Dattani

Professor of Clinical Chemistry
Professor Simon Heales

Professor of Paediatric Endocrinology
Professor Peter Hindmarsh

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Professor Susan Malcolm

Emeritus Professor of Molecular Embryology
Professor Marilyn Monk

Emeritus Professor of Biochemistry
Professor David Muller

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Professor Marcus Pembrey

Emeritus Professor of Child Health and Growth
Professor Michael Preece

Emeritus Professor of Biochemistry
Professor Bryan Winchester

Reader in Paediatric Endocrinology and Wellcome Trust Senior Research Fellow in Clinical Science
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Reader in Paediatric Endocrinology
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Professor Peter Hammond

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Professor Martin Barratt
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Reader in Paediatric Nephrology
Dr Detlef Bockenhauer
Dr Lesley Rees

Senior Research Associate
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Dr Eugenia Papakrivopoulou

INFECTION AND IMMUNITY THEME

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Immunobiology Unit

Professor of Vaccinology and Immunology, Director of Clinical Research and Development at Great Ormond Street Hospital and Head of Unit
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Professor of Immunology
Professor Robin Callard

Professor of Experimental Immunology
Professor Tessa Crompton

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BBSRC Senior Research Fellow
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Infectious Diseases and Microbiology Unit

Professor of Infectious Disease and Immunology and Head of Unit
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Senior Lecturer
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Clinical Reader in Vascultis
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Dr Despina Eleftheriou

Senior Research Associate
Ebun Omoyinmi

NEUROSCIENCES AND MENTAL HEALTH THEME

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Behavioural and Brain Sciences Unit

Professor of Behavioural and Brain Sciences and Head of Unit
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National Institute of Health Research Clinician Scientist
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Margaret H Nicholas Professor of Developmental Biology and Genetics
Professor Jane Sowden

Senior Research Associate
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Developmental Cognitive Neuroscience Unit

Professor of Developmental Cognitive Neuroscience and Head of Unit
Professor Faraneh Vargha-Khadem

Readers in Developmental Cognitive Neuroscience
Dr Michelle de Haan

Senior Lecturer
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NUTRITIONAL AND SURGICAL SCIENCES THEME

Nutrition Unit

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POPULATION HEALTH SCIENCES THEME

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Professor of Global Health
Dr Therese Hesketh

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Centre for Paediatric Epidemiology and Biostatistics

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Mr Jan Filochowski (from November 2012)

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Ms Fiona Dalton (until March 2013)

Chief Finance Officer
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Professor Martin Elliott

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Mr Mark Large

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Mr Trevor Clarke

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Right: one-year-old Hollie on Dinosaur Ward.

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