Great Ormond Street Hospital
Biomedical Research Centre

NHS
National Institute for
Health Research

Improving the diagnosis and treatment of childhood diseases

Morgan Stanley Clinical Building

Introduction and Director's report

At Great Ormond Street Hospital (GOSH) and University College London (UCL) Institute of Child Health (ICH) we are working in partnership to improve our understanding of diseases in children.

Funded by the National Institute for Health Research (NIHR), our Biomedical Research Centre (BRC) has a focus on rare diseases and is delivering a programme of scientific and clinical research to improve the prevention, diagnosis and treatment of paediatric diseases. Our NIHR BRC enables us to support a world-class clinical research infrastructure that will accelerate the discovery of the basis of childhood diseases and the development of novel diagnostics and new treatments including stem, cellular and gene therapies.

This mid-term review summarises the progress that our BRC has made to date to support the translation of our laboratory research into better clinical outcomes for children.

The BRC has established and supports state-of-the-art facilities which together with well-established clinical services provide the infrastructure for excellent research. For instance, our Somers Clinical Research Facility (Somers CRF) staffed by highly trained research nurses provides specialist day care and associated facilities for children and young people taking part in clinical research studies.

Our translational research programmes are underpinned by BRC funded facilities which include a Gene and Cell Therapy Facility and two virtual centres funded by the BRC; the Centre for Translational Genomics – GOSgene and the Translational Biomarker Discovery Unit – GOSomics. Together these provide shared facilities, platforms and technologies that underpin the 'first-in-man' and 'first-in-child' research being undertaken within our BRC, and across UCL. Moreover, they are creating many new opportunities for collaboration as well as the ability to leverage substantial research income from funding organisations and the private sector. Our BRC provides the resource to stimulate and sustain many collaborative relationships with international companies including GlaxoSmithKline, Genzyme, BioMarin, Pfizer and Novartis.

Our BRC is part of a nationwide infrastructure network with whom we are working to enhance the competitiveness of the UK in delivering world-class clinical research. To achieve this, we are building our relationships with other NIHR BRCs, Biomedical Research Units and Clinical Research Facilities (CRFs) throughout the country. In addition, we host the NIHR Immunology Rare Diseases Translational Research Collaboration (RD-TRC) which is studying immunological rare diseases in partnership with UCL Hospitals BRC and NIHR/Wellcome Trust Manchester CRF. Our BRC is also collaborating with UCLH BRC and NIHR/Wellcome Trust Birmingham CRF through the neuromuscular and paediatric RD-TRC themes.

"The average citation impact for our experimental medicine publications is 50% higher than the UK average."

Recent analysis by Thomson Reuters provides evidence that research at GOSH and ICH is of high quality. When comparing research being undertaken at GOSH/ICH to other paediatric research centres, we are joint third internationally for publication citation impact and the average citation impact for our experimental medicine publications is 50% higher than the UK average.

We are almost midway through this BRC award term and through our commitments and investments, enabled by NIHR funding, we are focussed on continuing to deliver internationally competitive, innovative, paediatric and rare disease experimental medicine research well into the future.

Professor David Goldblatt
Director, NIHR Biomedical Research Centre

Background to our Biomedical Research Centre

Our BRC combines the paediatric care and treatment strengths of GOSH, with UCL, a world-leading biomedical research university. Together, we are translating fundamental biomedical research into treatments that save and improve the lives of children with rare diseases. We are ideally positioned to deliver this as GOSH is the largest recipient of nationally commissioned NHS services for rare diseases in the UK.

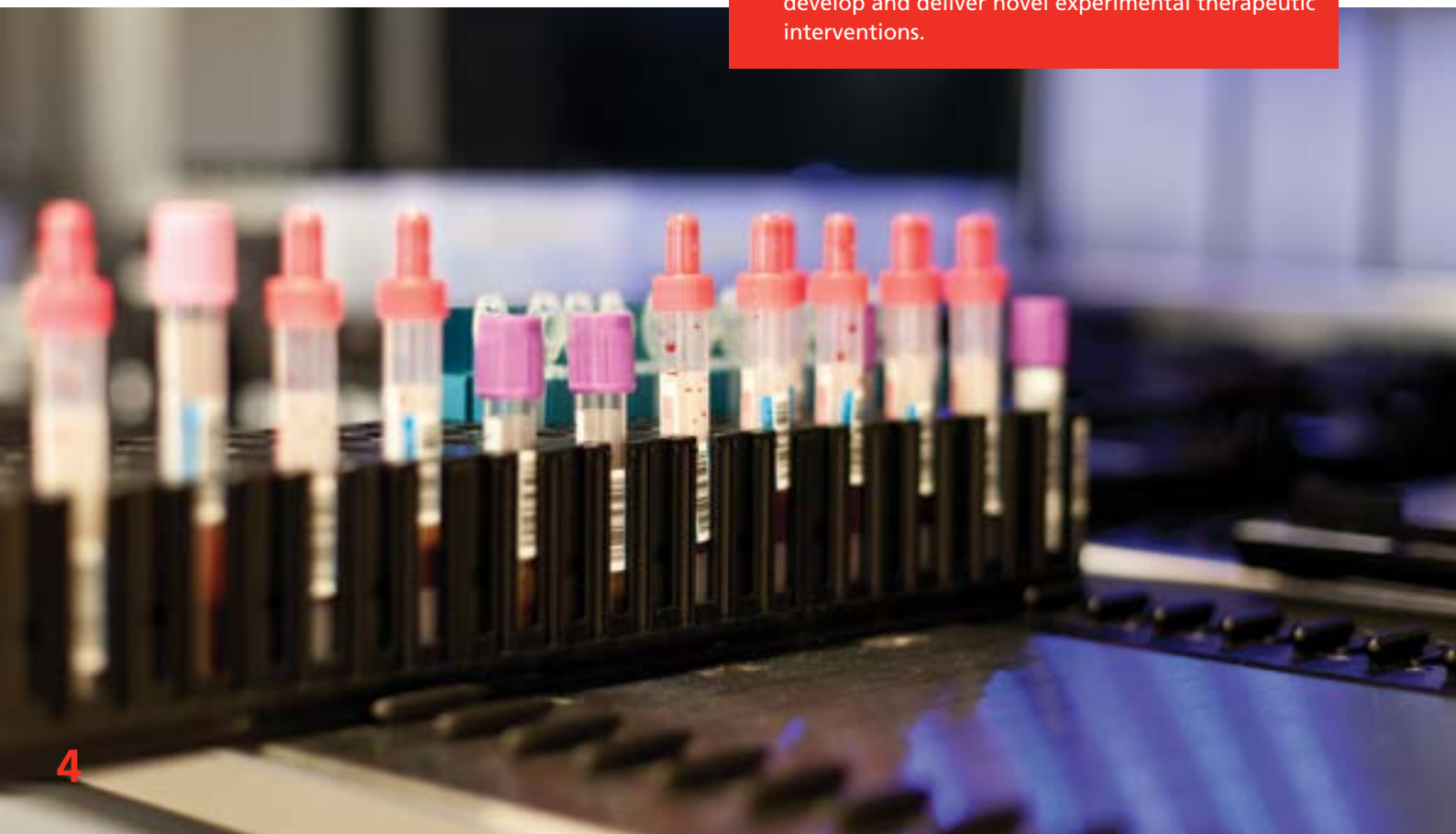
On 1 April 2012, our BRC entered a second 5-year term following a successful application for NIHR funding of over £35 million. Together with other NIHR BRCs, NIHR funding will help to deliver the Government's initiative to improve the translation of basic scientific developments into clinical benefits for patients, and to reinforce the position of the UK as a global leader in healthcare related research.

NIHR funding enables the translation of basic scientific discoveries in laboratories into 'first-in-man' or 'first-in-child' clinical studies. Our research aims to accelerate discoveries into the basis of childhood rare diseases and to develop novel diagnostics, imaging techniques and new treatments, including cellular and gene therapies.

Funding from the NIHR is being invested in research infrastructure across GOSH and ICH, including staff, facilities and training so that we can achieve our goal to deliver patient benefit worldwide.

Our research is organised into four themes:

- Molecular basis of childhood diseases: Clinicians and researchers from all disciplines are working towards understanding the molecular causes of childhood diseases, including rapid gene identification in uncharacterised genetic diseases.
- Diagnostics and imaging in childhood diseases: Our BRC funds research to improve diagnostics for rare diseases and the development of new biomarkers and novel imaging strategies to monitor disease progression and response to experimental therapies.
- Gene, stem and cellular therapies: Funding has been committed to the development of somatic gene and cellular therapies for immunodeficiency disease and a wide range of rare inherited and acquired disorders.
- Novel therapies for childhood diseases: Through access to some of the largest paediatric cohorts in the UK, we undertake novel 'first-in-man' studies to develop and deliver novel experimental therapeutic interventions.



BRC Research themes and programmes

Theme 1: Molecular basis of childhood disease – identification of disease-causing genes

We use state-of-the-art genomics and bioinformatics tools to identify the genetic mutations that cause rare childhood diseases. The discovery of the molecular basis of childhood diseases will translate into better diagnosis, carrier testing and the identification of targets for therapeutic agents.

Centre for Translational Genomics – GOSgene

To facilitate rapid gene identification in uncharacterised genetic diseases, in 2010, NIHR BRC funding created the opportunity for the development of the Centre for Translational Genomics – GOSgene. GOSgene brings together clinicians and researchers from all disciplines to work towards understanding the molecular causes of uncharacterised congenital disorders and other rare diseases as the first step in improving clinical outcomes for people with these diseases. The success of GOSgene demonstrates the power of genomics as a tool to identify novel rare disease causing genes.

The GOSgene team has performed whole-exome sequencing on over 320 samples covering a spectrum of 46 clinical phenotypes since April 2012. This work has resulted in the identification of 32 disease genes, 19 of which are novel. The current gene identification success rate is 59%, which is very high for a gene discovery programme of this nature.

GOSgene combines a multidisciplinary team of scientists including laboratory scientists, genomic analysts, bioinformaticists and software programmers. The support provided by GOSgene to clinicians and researchers is helping to improve diagnostic testing, supports genetic counselling, and will guide further functional analysis aimed at understanding the pathogenesis of disease. BRC investment into GOSgene has provided the support to build a team of researchers that are expertly skilled in the utility of next generation sequencing and will be ready to become the future leaders in translational genomic science. They will also be poised to utilise the large data sets being generated by national genomics initiatives such as Genomics England.

Collaborations with industry are increasing the value of our investment into GOSgene. GOSgene has formed collaborative partnerships with industrial partners including Illumina and Complete Genomics through links with the North East Thames Regional Genetics Service based at GOSH. GOSgene has also established a strong collaborative link with one of the leading software companies in the field of genomic data analysis, Qiagen Ingenuity Systems. The company provides intuitive web-based applications for quickly analysing and accurately interpreting the biological meaning in our genomics data. The collaboration with GOSgene has led to further software development which has enhanced data quality and interpretation times. This in turn has allowed GOSgene to adopt more projects and support more researchers across GOSH and UCL.

GOSgene is well positioned to combine the genomics and proteomics expertise from across UCL. The BRC has committed over £1 million in a new high definition, in-depth phenotyping at GOSH (HIGH-5) programme which will deliver an all-inclusive OMICS approach to inform diagnostic and treatment options of rare diseases. Genomics, proteomics or metabolomics approaches in partnership with the UCL Translational Biomarker Discover Unit – GOSomics will help to achieve a better understanding of the basis of genetic variation in human disease (and health) by characterising molecular endophenotypes. The aim is to identify molecular endophenotypes, modifiers of disease and therapeutic targets to personalise patient care.

Theme 2: Diagnostics and imaging in childhood diseases

The diagnostics and imaging theme brings together BRC experimental medicine activities across laboratory and clinical medicine and imaging.

The core strategy is to develop our 'Biomarker discovery' approach incorporating genomics, proteomics, metabolomics and expression profiling, to maximise translational NHS hospital capability for new diagnostic tests.

Our imaging research utilises our world class computer modelling and device design platform, which complements cardiovascular and neurological imaging strategies across UCL.

UCL Translational Biomarker Discovery Facility – GOSomics

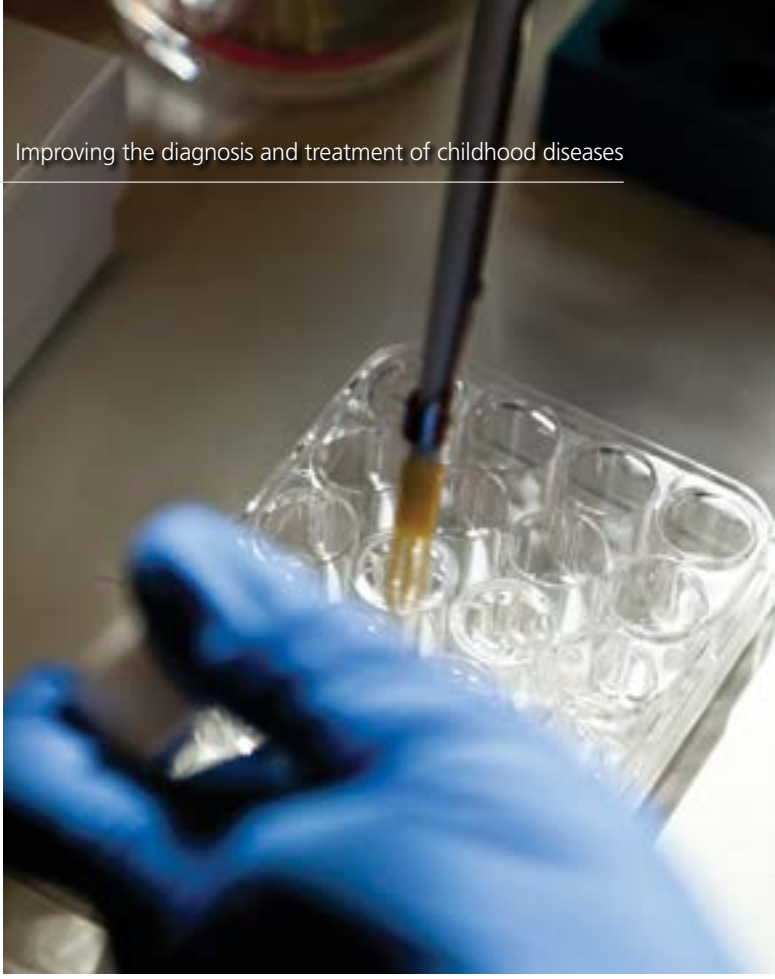
The establishment of the UCL Translational Biomarker Discovery Facility – GOSomics was made possible by BRC funding in 2012. The investment of over £1 million provides support for GOSomics researchers, including the facility lead, Dr Kevin Mills, as well as an academic clinical lecturer in Translational Biomarker Discovery and a non-clinical research associate.

GOSomics supports the discovery of novel biomarkers and their translation into clinical tests for use by diagnostic laboratories by analysis of protein expression in human tissues and fluids. The BRC investment enables the facility to undertake pilot studies, including set-up, sample preparation, analysis and bioinformatics. This is important to achieve proof of concept that can then be used by researchers applying for funding to translate discovery to patient benefit. Projects undertaken by GOSomics often involve collaborations between departments within GOSH and UCL, including; the Institute of Ophthalmology, Molecular Immunology, the Clinical Medical Genetics Unit, the Surgical Unit at GOSH and the Institute of Neurology.

The facility has already resulted in the discovery of novel sensitive biomarkers that can predict presymptomatic kidney disease, which are being developed into a rapid, multiplexed urine test for use by the GOSH chemical pathology department. There is great potential to use this non-invasive test to look at other types of kidney disease, for example as a non-invasive test for early detection of renal dysfunction that currently requires a kidney biopsy. Another benefit to patients from GOSomics work includes the ability to rapidly and accurately monitor enzyme replacement therapy in patients with Fabry disease.

Other highlights are the development of a new rapid and more specific maternal serum test for Down's Syndrome and, in close collaboration with our sister hospital the National Hospital for Neurology, the discovery of potential new biomarkers of neurodegenerative diseases (Alzheimer's, Parkinson's disease and Lewy body dementia).

GOSomics is already supporting over 20 collaborative projects across UCL and plan to expand their operations to include both metabolomics and matrix-assisted laser desorption / ionisation tissue imaging during 2014 to increase capability and opportunities for collaboration.



Radiography – dedicated research scanner support

BRC funding is being used to provide dedicated MRI and CT-PET scanning resource to support our imaging experimental medicine research. Imaging appearances can be considered as biomarkers of disease, and better imaging markers can improve our disease diagnoses. Our dedicated scanning support is being utilised to develop markers predict prognosis in epilepsy and define cell density and structure of childhood tumours. In addition, we are expanding our pioneering work developing novel imaging markers of tumour response to therapy.

Development of less invasive autopsy in children has been identified as a Department of Health priority and we have been world leaders in this area over the last five years. We are extending this ground-breaking work to optimise specific MRI protocols for clinical use at GOSH and other NHS centres. We have recently expanded this work to pioneering 'first-in-man' demonstration of laparoscopic-assisted minimally invasive autopsies with targeted tissue biopsy. We are continuing to develop this approach to provide a complete less-invasive post-mortem service that can be offered to parents of foetuses or children who die as an alternative to conventional autopsy. This minimally invasive technique is more acceptable for parents which will increase the uptake of post-mortem assessment. Such patient-centred developments are particularly relevant to user involvement in the delivery of healthcare in the NHS.

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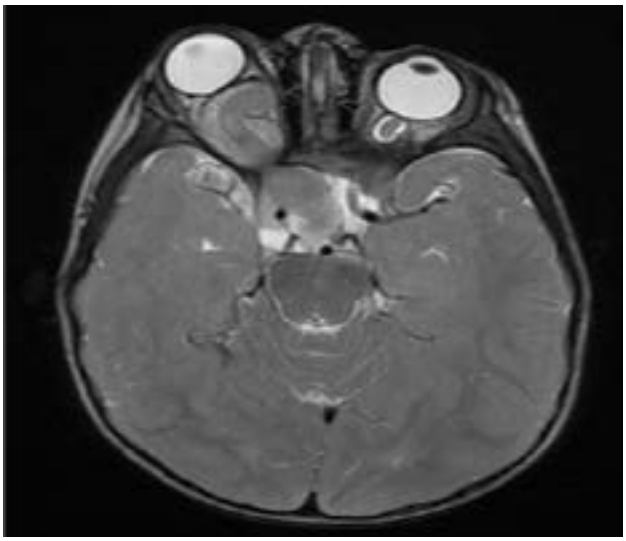
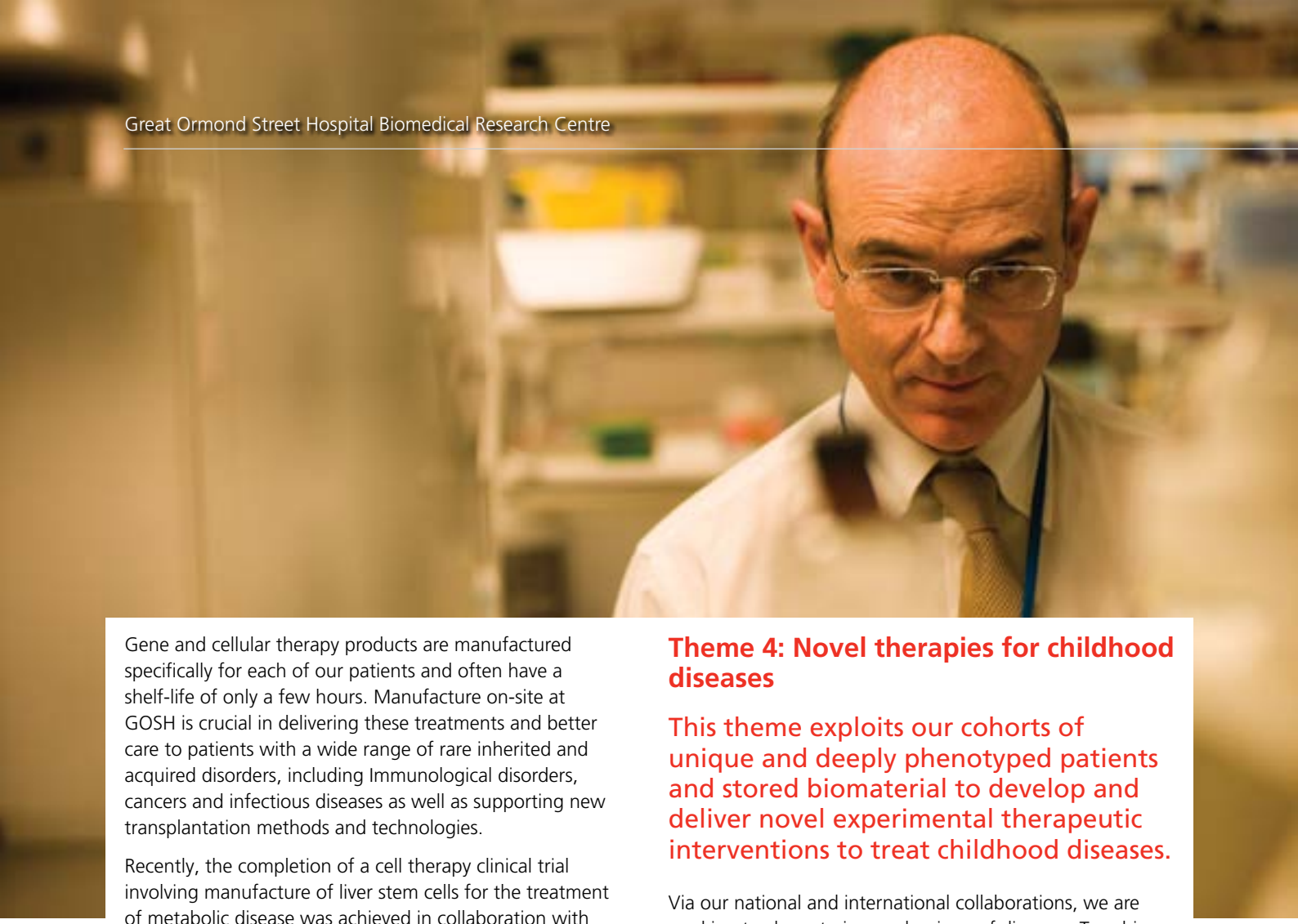


Theme 3: Gene, stem and cellular therapies – driving therapeutic innovation in rare disease

Our work in this theme addresses limitations of current medical treatments for a wide range of rare inherited and acquired disorders, and will combine with comprehensive training systems to allow dissemination of this expertise. Building on experience with our existing phase I/II gene therapy trials we have established infrastructure to support complex cell and gene therapy clinical trials. The aim is to develop new gene, stem and cellular therapies in adult and paediatric patients both locally, nationally and internationally.

Gene and Cell Therapy Facility

The discovery and development of innovative treatments for rare diseases is supported at GOSH and ICH by the Gene and Cell Therapy Facility. This facility is comprised of two clean rooms for the manufacture of gene and cell therapy medicinal products and supports research by allowing rapid translation of cutting-edge research into clinical treatments as part of phase I/II clinical trials and 'off-trial' treatments for children with rare diseases. This work is labour intensive, highly regulated and innovative. Funding from the BRC allows this work to continue at a high pace.



Gene and cellular therapy products are manufactured specifically for each of our patients and often have a shelf-life of only a few hours. Manufacture on-site at GOSH is crucial in delivering these treatments and better care to patients with a wide range of rare inherited and acquired disorders, including Immunological disorders, cancers and infectious diseases as well as supporting new transplantation methods and technologies.

Recently, the completion of a cell therapy clinical trial involving manufacture of liver stem cells for the treatment of metabolic disease was achieved in collaboration with a leading European biotech company and Birmingham Children's Hospital. This is improving health outcomes for children with liver diseases. Gene therapy trials for Netherton Syndrome, using genetically modified skin cells have also recently opened, made possible by the expertise that is supported and developed by our BRC.

The Gene and Cell Therapy Facility provides a quality-assured interface with industrial partners that require the manufacture of gene modified cell products for specific gene therapy studies. It also acts as a local and national hub for specialised training and for manufacture of personalised cell and gene therapies. BRC funding is securing further expansion of the facility to six clean rooms in the new Centre for Children's Rare Disease Research which will allow the Facility to support a greater number of early-phase clinical trials in future. Professor Adrian Thrasher, who leads the facility, says that 'Thanks to BRC investment the Gene and Cell Therapy Facility is in a unique position to be able to quickly translate novel innovative therapies to patients'.

The BRC has committed a further £750,000 to investigate strategies to improve the efficiency and effectiveness of T-cell modification with co-funding from Miltenyi Biotech. This collaboration will drive investigation and development of an automated or part-automated process for production of gene and cellular therapy products for use in patients. The project will support the evolution of the Gene and Cell Therapy Facility in becoming a major European hub for the delivery of cell and gene therapy technologies.

Theme 4: Novel therapies for childhood diseases

This theme exploits our cohorts of unique and deeply phenotyped patients and stored biomaterial to develop and deliver novel experimental therapeutic interventions to treat childhood diseases.

Via our national and international collaborations, we are working to characterise mechanisms of diseases. To achieve this we are using clinical tools, cellular models as well as existing biobanks containing samples from well phenotyped and stratified cohorts available at GOSH, in collaboration with other UCLH BRCs, the NIHR BioReseource and the EuroBiobank (a network of which we are members).

The wide range of conditions assessed at GOSH, underpinned by expertise in biomedical science and BRC support, is enabling us to proceed along the translational pathway from the laboratory to proof of concept first-in-man/-child studies. Our strong clinical base has allowed us to translate and refine validated outcome measures to allow better and larger confirmatory studies, thereby maximising translational impact for our patients.

Through this research theme, the BRC is currently supporting phase I and II studies in haematology and oncology, and rheumatological, neuromuscular, metabolic and renal disorders. There is a high degree of complementarity with the other BRC themes and with other national and international initiatives; in particular the evaluation of an intervention often requires access to unique imaging or diagnostic facilities as well as disease stratification based on novel biomarkers. A number of interventions are complementary with the stem cell and gene therapy approaches, underpinned by collaborative funding between individual areas of research.

Investing in Neuromuscular Experimental Medicine Research

Our BRC provides essential support to the work of the MRC Centre for Neuromuscular Diseases (CNMD) in collaboration with Newcastle and UCLH BRCs. The CNMD's mission is the development of a highly visible national strategic focus for translational research in NMD. This will be achieved through the following two routes: 1) by interdisciplinary cross institution partnerships linking scientists, clinicians, hosts, industry and patients; by developed core translational tools including muscle MRIs; 2) by linking independently funded science programmes to existing clinical research projects and inform our translational research.

"Our specific contribution to the biobanks in the CNMD has enabled over 60 projects which resulted in over 20 high impact papers."

We have committed £350,000 to supporting the CNMD in this BRC award term in partnership with UCLH and Newcastle BRCs. This will contribute to supporting neuromuscular early phase experimental medicine studies and clinical trials by providing funding for staff resources.

Our specific contribution to the biobanks in the CNMD has enabled over 60 projects which resulted in over 20 high impact papers. These manuscripts range from research and development in the diagnosis of neuromuscular diseases, to cutting edge gene discoveries. For example, one of the studies has confirmed that flow cytometry analysis of skin fibroblasts can be used as an alternative method for screening for collagen VI deficiency at the protein level, avoiding invasive muscle biopsy. This is a major advance in the diagnosis of rare congenital muscular dystrophies. This quantitative, time and cost-effective method has been adopted by the National Specialist Commissioning Team as a diagnostic service. Similarly, quantitative assessment of alpha dystroglycan glycosylation using flow cytometry analysis has been shown to complement existing immunohistochemical assays in skeletal muscle.

BRC investment in the CNMD also supports the neuromuscular tissue bank, which has received over 450 samples since 2012. This is already yielding important information and promoting work with industry, for example the quantification of utrophin upregulation in muscle biopsies in collaboration with Summit plc. This information is crucial for the development of the Summit drug aimed at upregulating utrophin production in Duchenne muscular dystrophy children. Our collaborative phase Ib clinical trial with Summit plc, has already recruited its first patients at GOSH. Along similar lines we have also recruited the first DMD patients into two Prosensa sponsored studies, aimed at skipping exons 45 and 53, in which DMD boys will receive systemic administrations of the relevant antisense oligonucleotide therapy, developed by Prosensa. In addition to clinical outcome measures, the restoration of dystrophin expression to improve muscle condition in DMD patients will be measured using technology developed in collaboration with the MRC UK Biobank.

Somers Clinical Research Facility – supporting research nursing and clinical trials

The Somers Clinical Research Facility (Somers CRF) provides space for specialist day care accommodation and the nursing staff to care for the children and young people taking part in research studies.

The expert Somers CRF team works closely with dedicated support staff to provide a tailored service to investigators undertaking all types of clinical research within GOSH. There are currently over 80 active clinical studies being conducted within the Somers CRF.

The Somers CRF opened at GOSH in December 2008 and is central to our delivery of high impact clinical studies. Generous support towards the capital costs for this development was given by Mrs Somers and the JN Somers Charitable Wills Trust and Friends of Great Ormond Street Hospital.

Another key aim of the Somers CRF is to provide a management and career development structure for all children's research nurses working within GOSH/ICH. The CRF Children's Research Nursing team works in concert with other clinical research staff supported by the NIHR Local Comprehensive Research Network, including nurses and data managers, who are also based in the Somers CRF.

The Somers CRF is the central point of contact for commercial partners looking to undertake clinical trials at GOSH. Over 40% of the studies hosted by the CRF are commercially sponsored.

Recent successes include the Summit plc trial, to which the first two global patients were recruited in the Somers CRF. The Somers CRF has supported a BioMarin Pharmaceuticals sponsored clinical trial that has led to the clinical development and approval of a drug called VIMIZIM as a treatment for Morquio A syndrome. These successes demonstrate the importance of support for the CRF in continuing to deliver high quality and complex clinical studies for paediatric and rare diseases.

BRC success stories

BRC input into Genomics England

In collaboration with other UCL Partner BRCs at UCL Hospitals and Moorfields Eye Hospital, we have contributed over 800 samples to the pilot of the Genomics England (GEL) project.

To achieve this, the Somers CRF was opened during weekends between November 2013 and January 2014 for patient appointments and sample collections. The GEL project is a national initiative aiming to sequence genomes of 100,000 patients in the next five years. The knowledge gained will lead to improved diagnosis and personalised care.

Achievement of first global recruits to clinical trials

Research teams at GOSH have recruited the first global patients to four multi-centre studies, including the first two patients to a Summit plc sponsored phase Ib study.

The treatment under investigation is a compound capable of upregulating the production of utrophin, a protein that can compensate for the lack of dystrophin, which cannot be produced in boys with DMD. This treatment could slow or even stop the progression of this devastating condition. This demonstrates the continued success of GOSH in utilising its established cohorts and commissioned services to recruit patients to global clinical rare disease studies.

"These global firsts are a significant achievement and shows the dedication of the research and research support teams at Great Ormond Street Hospital."

Professor Dame Sally C Davies, Chief Medical Officer and Chief Scientific Adviser at the Department of Health

'Off-trial' treatment in rare diseases

The careful use of 'off-trial' treatment is particularly important for patients with rare diseases when treatment options are limited or when patients do not fully meet the criteria for entry into a clinical trial or when a clinical trial is not available.

GOSH has a special licence allowing certain gene and cell therapies to be used in 'off-trial' treatment. When we treat patients 'off trial', we adhere to a local ethical framework approved by a clinical ethics committee with the aim of protecting the patient interests and patients are treated just as if they are on a trial. The patient is provided with as much available information on the treatment as possible and, naturally, their consent is obtained. Beyond the initial hoped-for patient benefit, there are also wider reaching results. Information obtained during such treatment can inform the design of future trials and their implementation. In summary, this means that by treating small numbers of patients 'off-trial' there are potentially significant benefits for the patient (access to potentially life-saving treatment), academia and the pharmaceutical industries (lessons learned for future trial design or implementation of a trial).

In a recent clinical trial of lentiviral vector mediated gene therapy for adenosine deaminase (ADA) severe combined immunodeficiency (SCID), two patients were treated prior to the trial being opened. Both patients were in need of treatment and were poor candidates for a mismatched bone marrow transplant which was their other option. Both patients were treated 'off trial' and have had excellent outcomes two years later. The patients received significant benefit but the experience gained from their treatment also informed the design of the subsequent formal clinical study. Since 2010, we have treated seven patients 'off-trial', for a range of genetic disorders including ADA SCID, chronic granulomatous disorder (CGD), and X-linked SCID.

Reliable, Accurate Prenatal non-Invasive Diagnosis (RAPID)

The RAPID programme is investigating non-invasive prenatal diagnosis (NIPD) using cell free DNA in maternal plasma and is transforming the way we offer prenatal diagnosis to families at high risk of genetic disorders or chromosomal problems.

This new technology allows safer prenatal diagnosis by taking a maternal blood sample to analyse the cell free fetal DNA which circulates in the mother's blood. The patient benefits of our efforts are already being seen as the number of invasive tests required diminishes, reducing the risk of miscarriage to the women involved. We have led the evaluation of a number of tests and several tests for single gene disorders have now been implemented into routine prenatal genetic practice in the NHS, a service delivered from our laboratory at GOSH. We are also leading the evaluation of NIPD for aneuploidy in the NHS. Having developed this test in our Regional Genetics Laboratory at GOSH we have launched the evaluation study in four NHS maternity units, with roll out to more units in Scotland and the South of England imminent. We are working closely with the National Screening Committee to develop the standards required for more widespread implementation.

"The success of the RAPID programme demonstrates that experimental medicine research at GOSH is leading to better clinical outcomes in the NHS."

The success of the RAPID programme demonstrates that experimental medicine research at GOSH is leading to better clinical outcomes in the NHS. With evidenced support from healthcare professionals and service users for non-invasive testing and diagnosis to be implemented into clinical practice, work will continue on developing testing for aneuploidies and further single gene disorders. The success of the RAPID Programme has been due to the phenomenal support from over 40 centres UK-wide, as well as some key collaborations across UCL and with industry.

The RAPID programme is also supported by an NIHR Programme Grant for Applied Research award.

Patient and public involvement and engagement in research

Involving and engaging patients and the public in the research conducted within the BRC is one of our key aims. We are bringing together researchers and the public to improve our understanding of the childhood diseases that are treated at GOSH. We continue to develop creative approaches to include the opinions, insights and experiences of our patients and the public in our research.

Our updated BRC Patient and Public Involvement and Engagement (PPI/E) Strategy was launched in 2013. As part of this strategy, the BRC now offers a programme of support to researchers with PPI/E including training on PPI/E in paediatric research and support for researchers in establishing a meaningful PPI programme informing and disseminating their research activities. The BRC also hosts events for school students to offer them a unique insight into the research conducted across the BRC.

One of the National Institute for Health Research Clinical Research Network Children's theme's Young Persons Advisory Groups meet regularly in our Somers CRF to help shape our BRC strategic objectives. The group consists of approximately a dozen young members, aged between 8-18 years who have personal or indirect experience of living with a childhood condition, illness, or disability, and many have participated in clinical trials. The group meets frequently either at weekends, or during school holidays. The views and opinions expressed by the group feeds into the BRC Scientific Board.

Patient and public engagement events held over the last year include International Clinical Trials Day in May, the Big Bang Festival in July, and the Bloomsbury Festival in October 2013. These events have successfully raised awareness among families and visitors, students, and the local community about paediatric clinical health research.

"Patient and public engagement events… include International Clinical Trials Day in May, the Big Bang Festival in July, and the Bloomsbury Festival in October."

Industrial partnerships and enterprise

The development of new medical treatments and techniques very much depends on partnership with Industry, particularly pharmaceutical companies, medical devices companies and SMEs (small and medium enterprises).

The BRC continues to work closely with UCL Business (UCLB) to raise awareness of the importance of the creation and development of intellectual assets for the translation and dissemination of innovation and clinical practice. We continue to see an upward trend in activity and creation and development of intellectual assets remains a focus of our long term BRC strategy.

The Somers CRF continues to play a key role in delivering a growing volume of clinical and especially experimental medicine research within GOSH in partnership with industry. Since 2012, the number of industry sponsored clinical trials supported by the Somers CRF has increased by 25% and has facilitated clinical projects with over 20 pharmaceutical companies in this period.

Professor Adrian Thrasher is co-inventor on a patented gene therapy technology for Haemophilia A that was recently licensed to BioMarin and is led by Professor Amit Nathwani from the UCL Cancer Institute.

A fruitful collaboration between Professor Simon Heales and Vitaflo Ltd (part of Nestlé) has resulted in two patent filings on dietary compositions that effect mitochondrial bio-genesis and have the potential to control epileptic seizures. UCLB is currently in negotiations with Vitaflo to grant access under the patents for the development of nutritional products with potential to replace the ketogenic diet. Vitaflo funding for the collaboration is likely to be extended for an additional three years.

Through our industry collaborative awards, the BRC has provided over £300,000 to support five projects which build on existing partnerships between BRC researchers and commercial organisations in the UK and abroad. These awards will help to quicken the speed of delivery of these research projects as well as the translation of the results into improved clinical outcomes.

Through the organisation of industry engagement seminars and events, our BRC has been helping to encourage clinicians and academics across GOSH and ICH to think more about the value of partnering with commercial organisations in the translation of their research to clinical benefit.

Training the next generation of researchers

Our BRC is totally committed to delivering a successful strategy to train the next generation of paediatric researchers having allocated £3 million to a range of different training initiatives across different career stages and professions.

PhD studentships in translational research

We are funding 12 translational research PhD studentships across UCL, in research areas aligned with the objectives of BRC, including a jointly-funded UCL medical devices studentship scheme with UCLH and Moorfields BRCs.

Francis Crick Institute fellowships

Our BRC has already started building collaborative relationships with the Francis Crick Institute (FCI) by funding two clinical research fellowships, in partnership with other BRCs across London, to harness the multidisciplinary strengths of the FCI in the delivery of our research objectives.

Experimental medicine research fellowships

We are funding 13 postdoctoral experimental medicine scientists and clinical fellows that we support in their development into leading paediatric researchers as well as the attainment of higher fellowships in translational research.

Academic clinical fellowships and lectureships

Through our links with GSK, we are piloting a joint BRC-GSK Academic Clinical Fellowship scheme to begin to expand paediatric clinical pharmacology resource in partnership with industry. We are also supporting research career development through our commitment to an Academic Clinical Lecturer working within Translational Biomarker Discovery Facility – GOSomics.

Nurses and allied health professionals

We are working to engage more nurses and Allied Health Professionals (AHP) in experimental medicine research being undertaken in our BRC. We are supporting a leadership position at GOSH who is working with the GOSH Centre for Outcomes and Experience Research in Children's Health, Illness and Disability to develop clinical and academic research leadership for nurses and AHPs at GOSH and UCL. By funding MSc level courses and covering study leave, we have started to work with and mentor our nurses and AHPs so that they can be successful in being awarded NIHR training fellowships.

Working with other centres for nursing and AHP-led research across the country, our initiative seeks to encourage individual and institutional development that will create a base of research led by nurses and AHPs.

Academic training weekend

Developed and supported by the our BRC, this weekend will provide an excellent opportunity for 40 academic paediatric trainees from across the country to develop their research skills in a residential weekend course at Ashridge Business School. The innovative programme includes facilitated interactive work in small groups on grant proposals, the peer review process, ethics review, and presentation skills. Seminars will also be given on experimental medicine and public health research in children.

Great Ormond Street Hospital for Children
NHS Foundation Trust

Design: Susan Rentoul Design

National Institute for Health Research Great Ormond Street Hospital Biomedical Research Centre is a partnership between Great Ormond Street Hospital NHS Foundation Trust and University College London (UCL) Institute of Child Health.

www.gosh.nhs.uk/research-and-innovation/biomedical-research-centre

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