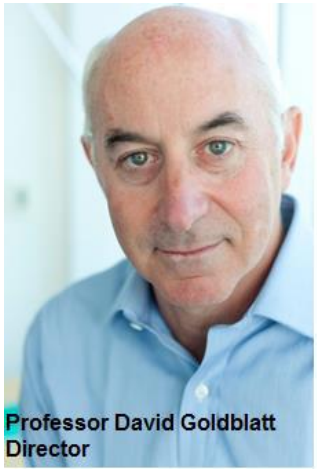
National Institute for Health Research

News from the
NIHR Biomedical Research Centre at
Great Ormond Street Hospital for Children NHS Foundation Trust
and University College London

Director's introduction


Professor David Goldblatt
Director

Welcome to the March 2016 edition of our newsletter, highlighting the activity and achievements of our National Institute for Health Research Biomedical Research Centre (BRC) at Great Ormond Street Hospital for Children NHS Trust and University College London.

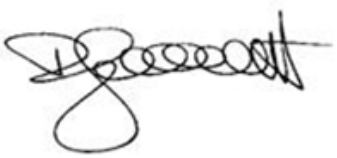
Our Special Feature this issue is on the success of the 100,000 Genomes. At the recently held open meeting we were able to share findings and stories from some of the first families at GOSH who have received a diagnosis through the pilot study. The 100,000 Genomes project has been supported by the National Institute of Health Research Great Ormond Street Biomedical Research Centre.

I would like to congratulate Lesley Katchburian, a Clinical Specialist Physiotherapist at GOSH on being awarded a Clinical Doctoral Research Fellowship from the NIHR, Lesley's project is entitled 'Understanding clinical and patient reported response of children and young people with Cerebral Palsy to Botulinum Toxin A: a longitudinal observational study'. Congratulations also to Dr Elaine Cloutman-Green an Infection Prevention and Control Practitioner at GOSH who has been awarded a NIHR Clinical Lectureship entitled 'Application of molecular typing in a routine clinical setting for the detection of cross transmission events linked to Gram-negative bacteria'. Both Lesley and Elaine received a one-day-a-week BRC internship to allow them to develop and enhance their applications.

I would also like to congratulate Dr William Van't Hoff on his recent appointment as NIHR Clinical Research Network (CRN): Clinical Director for NHS Engagement. Congratulations to both Dr Ri Liesner and Dr Anna Martinez who have been recognised for their significant contribution to commercial research within the NHS. Dr Rhi Liesner has been recognised for recruiting the first global patient in a haemophilia study designed to evaluate the safety and efficacy of a recombinant fusion protein. Dr Anna Martinez was recognised for recruiting the first European patient into a phase 3 Epidermolysis Bullosa trial. They join over 70 other leading NIHR commercial Principal Investigators who have been celebrated for their crucial role in the NIHR CRN's substantial growth in the delivery of commercial contract research in the NHS.

The official competition for NIHR Biomedical Research Centre funding has been launched, which will provide funding to successful centres for the next five years, from the 1st of April 2017 to 31st of March 2022. We have submitted the pre-qualifying questionnaire to the NIHR and these are now under review, we expect to hear whether we have been successful at this stage during April. I would like to invite you to a series of open meetings that we are holding to allow staff across GOSH and ICH to come and share their ideas for the full BRC application. For more information, please see the Upcoming events section.

I hope you enjoy reading this newsletter and as always welcome any thoughts and contributions you would like to make. Please e-mail BRC Newsletter with any future contributions to the newsletter you would like to include.



David Goldblatt, Director NIHR Biomedical Research Centre Director, Clinical Research and Development, Great Ormond Street Children's Hospital,

Professor of Vaccinology and Immunology NIHR Senior Investigator

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SPECIAL FEATURE

First patient's to receive diagnosis through 100,000 genome project

At the end of 2013 clinicians from GOSH, Moorfields and the National Hospital for Neurology and Neurosurgery enrolled patients with rare diseases into the 100,000 Genomes Pilot study. The aims of the Pilot were twofold: to find out whether Whole Genome Sequencing would be a feasible diagnostic tool for patients in the NHS, and to test the pipelines and processes for patient recruitment and sample collection in anticipation of the main 100,000 Genomes Programme. Supported and funded by the GOSH BRC, over 1000 patient samples were provided by GOSH and our UCL partners, contributing to 22% of the total samples included in the pilot study.

Researchers are now getting results back on some of the early patient recruits. On January 12th 2016 in the Kennedy Lecture theatre at ICH, GOSH Chief Executive Peter Steer hosted an open meeting to share findings from some of the first families with results from the Pilot study.

Two families have spoken about their experiences of participating in the study. Jessica is a 4 year old girl with epilepsy and developmental delay who was found to have a mutation in a gene, *SLC2A1*, coding for a glucose transporter.

In Jessica's case, glucose cannot be transported efficiently across the blood-brain barrier meaning that her brain cannot get the energy it needs. The genetic result has significant implications for Jessica; this type of epilepsy responds well to a 'ketogenic diet', a diet rich in fat which the brain can use as an alternative energy source to glucose. This means that Jessica's anti-convulsant medication can be reduced.

The second patient was 4 year old Georgia, who has developmental delay, visual and renal problems. She was found to have a mutation in a gene which modifies histones, the proteins which package DNA affecting the expression of many important genes. Her parents spoke very movingly about the long time it had taken to get a diagnosis for Georgia and what this diagnostic uncertainty had meant for them. Since getting their results from the study Georgia's parents now know that her condition has not been inherited, but has arisen for the first time in Georgia. Knowing that the chance of having a child with similar problems to Georgia is very low, they now feel able to extend their family and have another child.

The audience included staff from GOSH and our partner Trusts, which together make up the North Thames Genomic Medicine Centre. Guests included Professor Sue Hill, Chief Scientific Officer for NHS England, Professor Mark Caulfield, Chief Scientist for Genomics England and the UCL Vice Provost for Health, Professor David Lomas, who all participated in a Q&A session with the audience.

GENERAL NEWS

Dr Tom Jacques co-authored update of national guidelines

Dr Tom Jacques has co-authored the fourth edition of the Dataset for tumours of the central nervous system, including the pituitary gland. These datasets provide prognostic information allowing clinicians to provide high standards of care for patients and appropriate management for specific clinical circumstances.

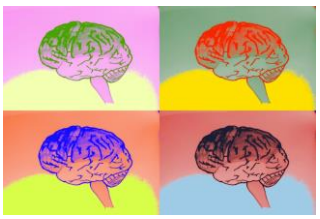
'Under the microscope' exhibition launched

Artist Sofie Layton has been supported by the BRC along with the Wellcome Trust and the Blavatnik Family Foundation, to investigate the work of rare disease specialists. Inspired by families 'under the microscope', the project looked at gene therapy and the experiences of families on the cardiac ward which involved 3D printing of hearts. Sofie has reinterpreted these ideas in a creative and captivating way, to enable children and their families to engage with the world of the researcher. Sofie's work includes an exhibition called 'making the invisible visible' which combines a series of multidisciplinary artworks about the heart, translating medical aspects of cardiology as well as gene therapy. The exhibition is currently being held in the Winter Garden Gallery Space in ICH and in various other sites in the hospital. For more information about Sofie's work click here or contact GO Create!

External Advisory Board visit-January 2016

The third BRC External Advisory Board (EAB) visit took place on the 6th and 7th of January 2016. The aim of the visit was to give feedback for the upcoming BRC renewal this year. The visit went really well, highlighting our strengths in integrating early career researchers into all aspects of the BRC, and above average return on investments, whilst also providing valuable advice on areas for future focus.

The London Brain Project has secured further funding from Arts Council England



The London Brain Project has been awarded a £25,000 grant from Arts Council England (ACE), which will support two years of programming in their 'beyond' series, exploring issues related to neurological and psychological disorders. This ACE grant was made possible by matched funding from GOSH BRC, who have provided funds to support the 'Beyond my Brain' workshop and exhibition, where we will explore the realities of living with a brain injury in childhood. This will be starting in early summer 2016 with the Beyond my Brain workshop.

Molecular basis of childhood diseases theme news

Identification of genetic mutation linked to complications associated with common birthmarks

Research led by BRC-supported Dr Veronica Kinsler, has found that a subset of a common type of birthmark, which is associated with severe complications, is caused by activating mutations in the genes *GNAII* and *GNAQ*. These findings could lead to early identification of infants at risk of serious complications.

Dermal Melanocytosis (also known as Mongolian Blue Spots) are very common birthmarks, and are often dismissed as a normal finding. When they co-occur with vascular birthmarks they come under the diagnostic heading of Phakomatosis Pigmentovascularis (PPV), a condition considered previously to be a separate phenotype. In some cases of Mongolian Blue Spots and PPV, these birthmarks can be associated with congenital glaucoma which can damage vision, and with vascular abnormalities in the brain which can lead to seizures and neurodevelopmental delay. In these cases early detection means treatment can be started for glaucoma and for the brain abnormalities which reduce the risk of these complications. It has not been clear up until now which children with Mongolian Blue Spots are at risk for these problems, and why.

This study found that extensive dermal melanocytosis and PPV are genetic conditions that are associated with post-zygotic mutations in the genes *GNA11* and *GNAQ*. These mutations were undetectable in the blood, however were detected at very low levels in the affected tissue, suggesting that the disorder is caused by a post-zygotic mutation. Therefore, these disorders join a number of other disorders including Sturge-Webber syndrome (SWS) in the group of mosaic heterotrimeric G-protein disorders. This leads the way for accurate clinical molecular diagnosis and identification of neonates at risk of complications associated with these birthmarks. It also allows investigation into potential therapeutic options for these patients.

The study findings were published in The Journal of Investigative Dermatology. The lead researcher on this paper was Dr Veronica Kinsler, a BRC supported researcher and the research was supported by the Wellcome Trust.

Prevention of neural tube defects in women taking additional supplements

BRC supported researchers Professors Andrew Copp and Nicholas Greene have led research which suggests that women who are at risk of having children with neural tube defects (NTD's) such as spina bifida may be able to reduce this risk by taking inositol (Vitamin B8) as well as folic acid during pregnancy.

The study involved 99 women who had previously had a pregnancy affected by a neural tube defect, and who were planning on becoming pregnant within the next year. Half of these women agreed to be randomly assigned to one of two groups; one group were instructed to take 5mg of Folic acid each day and a placebo and the second group were instructed to take 5mg of Folic acid plus 1mg of Inositol daily. Among the women who chose not be randomised many decided to take folic acid and inositol in their next pregnancy.

Overall, fifty-seven of the women became pregnant during the study period. Results showed that none of the women taking both inositol and folic acid had pregnancies with a NTD, compared to 3 of those who were taking only folic acid.

This research suggests that for those women for whom folic acid supplements are not sufficient to prevent a NTD, taking inositol in addition to folic acid, may provide greater protection. A larger-scale study is now needed in order to in order to confirm the beneficial effects of inositol.

The findings were published in the British Journal of Nutrition, and received media coverage from a number of different sources including the BBC.

Novel therapies for translation in childhood disease theme news

Dubowitz Neuromuscular Centre confirmed as Centre of Paediatric Clinical and Research Excellence

The Dubowitz Neuromuscular Centre (DNC) at GOSH and ICH has been confirmed as a Centre of Paediatric Clinical and Research Excellence by Muscular Dystrophy UK. This is one of ten Centres of Excellence and the only Paediatric Centre of Clinical and Research Excellence. This award recognises centres with outstanding levels of specialist care for people living with muscle wasting conditions.

Muscular Dystrophy UK
Fighting muscle-wasting conditions
Paediatric Centre for Research and Clinical Excellence

The DNC is a leading clinical and research centre specialising in neuromuscular disorders affecting children. The DNC provides clinical assessments, diagnostic services and advice on treatment and rehabilitation alongside clinical trials, basic research focusing on causes of neuromuscular diseases in childhood and identifying novel therapeutic interventions.

The status was awarded following a national audit carried out by Muscular Dystrophy UK, aimed at ensuring that high-quality care is provided to patients with muscle-wasting conditions.

BRC Theme Lead Professor Francesco Muntoni is Head of the DNC. Francesco is the lead for the Novel therapies for translation in childhood diseases, and also Head of the Developmental Neuroscience Programme at the Institute of Child Health, and co-director of the MRC Neuromuscular Translational Research at UCL.

GOSH utilising new wearable technology to monitor Niemann-Pick disease

aparito is a smartphone app combined with wearable technology used to monitor patients remotely. Through the use of a motion tracker in a wristband the movements of patients with ataxia and other related ambulatory conditions can be monitored continuously and at home between hospital visits. This data is sent via a smartphone app, where it can be combined with other information such as medication adherence, events such as falls or seizures. Healthcare professionals can have constant access to this information via the web.

BRC-researcher Professor Paul Gissen, has been working closely with aparito, and GOSH is to be the first UK based site to use this new wearable technology in clinical practise. The overall aim of this approach is to allow for patients routine activity to be monitored in a continuous, non-invasive way - resulting in fewer tests and shorter hospital visits.

Gene, stem and cellular therapies theme news

Identification of factors regulating Thymic Epithelial cell

Researchers at ICH led by BRC-supported Professor Tessa Crompton, in collaboration with the Paediatric Department at Oxford University, have shown that Sonic Hedgehog (Shh) is required for normal Thymic Epithelial cell (TEC) development and differentiation. This is one of the very few currently identified factors that regulate TEC differentiation and lineage choice between cortical (c) TEC and medullary (m) TEC. These findings contribute to our understanding of human autoimmune diseases and will inform strategies to replace thymus function in athymic children.

TEC are an essential component of the thymic stroma and are required to support T cell development. Two broad categories of TEC exist; mTEC and cTEC. cTEC are essential for T cell fate specification and TCR repertoire selection, and mTEC are essential for the induction of tolerance to self. Using both foetal and adult TEC, these experiments showed that Shh regulates mTEC differentiation and function, with the potential to alter central tolerance.

These findings are important to our understanding of immunity, autoimmunity and immunodeficiency. In the future it will be important to assess the effects of Shh signalling specifically in TEC differentiation and function on the induction and severity of human autoimmune diseases.
These findings were published in the Journal of Autoimmunity .

Clinical trial of gene therapy to treat patients with Fanconi anaemia to open at GOSH

A new clinical trial is to open at GOSH, trialling the use of Gene therapy in transplantation in Fanconi anaemia. The trial will be led by Dr Claire Booth, Dr Phil Ancliff and Professor Adrian Thrasher, ‘Gene, Stem and Cellular Therapies’ Theme Lead.

Fanconi anaemia is a life-limiting, inherited haematological disorder which causes bone marrow failure in childhood as well as other serious complications. Children can be treated with a bone marrow transplant but the success of this procedure depends on finding a well matched donor for the patient. Gene therapy could offer hope to children lacking a suitable donor for transplant and a clinical trial is due to open here at Great Ormond Street Hospital in the coming months.

The trial will rely on the BRC-funded GMP facility to genetically modify the patient's own stem cells.

Diagnostics and Imaging in Childhood Diseases theme news

Professor Neil Sebire receives NIHR HTA grant to investigate the potential use of the minimally invasive autopsy

Diagnostics and Imaging Theme lead, Professor Neil Sebire and co-applicants Professor Lyn Chitty and Dr Owen Arthurs have been awarded an NIHR HTA grant on behalf of the minimally invasive autopsy team at GOSH. The award of £286,000 is to investigate parental and societal acceptability of standard and less invasive methods of examination after death.

Following the death of a baby during pregnancy, birth or childhood, parents can be left with many questions, which, in many cases can only be answered through post-mortem or autopsy examination. Primarily due to the invasiveness of the procedure, the majority of parents don't agree to an autopsy being carried out and currently there is no alternative available.

It has been shown that a post-mortem MRI along with endoscopic assisted tissue sampling could act as an alternative to the invasive autopsy procedure. This study aims to identify the number and type of patients available for this novel approach and whether parents and families would feel it was more acceptable than the standard autopsy. The data obtained will be used to decide whether a larger study would be appropriate or whether there is sufficient evidence to offer this approach more widely.

Identification of new biomarkers for patients with Mucopolysaccharidoses

BRC supported researchers based in the UCL Biological Mass Spectrometry Research Centre have teamed up with the Centre for Inborn Errors of Metabolism (UCL-GOSH) to identify new urine biomarkers altered in patients with Mucopolysaccharidoses (MPS). These biomarkers were confirmed and validated by a high throughput targeted proteomics assay that has potential for clinical translation.

MPS is a genetic lysosomal storage disorder resulting from the body's inability to produce specific enzymes that degrade large complex sugar molecules also known as glycosaminoglycans (GAGs). Accumulation of GAGs causes progressive cellular damage, multi organ failure and a reduced life expectancy. MPS is currently diagnosed by analysis of urinary GAG species; however

GAG analysis is limited when it comes to discriminating mild and severe phenotypes of MPS I and MPS II.

This study carried out by BRC-funded Dr Wendy Heywood discovered potential surrogate urine markers linked to known pathological features of MPS. The test was able to show a relationship between marker concentrations and disease severity. In mild cases of MPS, markers were not significantly raised in comparison to controls and, 3 markers were able to stratify the severe neurological form of MPS II (Hunter syndrome) and the less severe non-neurological clinical phenotype.

This finding and the development of markers into a translatable assay has the potential to determine patient's severity at the point of diagnosis, where the initiation of treatment is crucial and has further potential to monitor the effect of treatment in MPS patients.

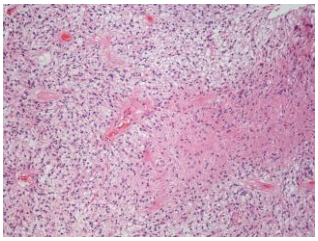
This research was funded as part of the GOSH BRC-supported infrastructure GOSomics and was published in Analytical chemistry.

Identification of molecular drivers behind most common type of paediatric brain tumour

Researchers from Great Ormond Street and UCL/ICH have teamed up with researchers from four other establishments to further knowledge of molecular drivers behind the most common type of paediatric brain tumour.

Pilocytic astrocytomas are the most common type of central nervous tumours in children. They are often slow growing, cystic well-circumscribed tumours that are most commonly situated within the cerebellum, making the removal of the tumour possible and although many are left with significant chronic disabilities this does mean a better prognosis is possible.

This study aimed to further knowledge of molecular drivers of pilocytic astrocytomas. Using brain tumour samples taken from 57 children, detailed investigations were carried out into microRNA (mRNA) and gene expression profiles alongside a pathway analysis. The findings suggested that



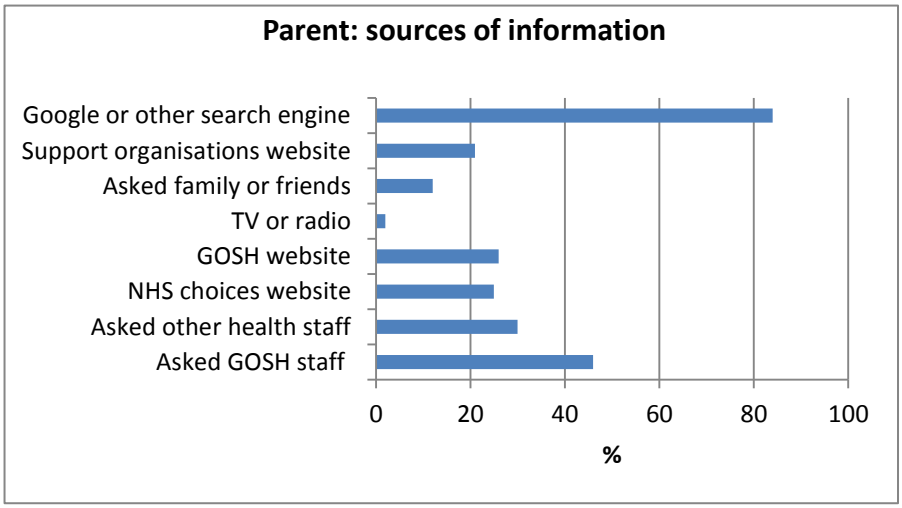
there is a distinctive mRNA and gene expression profile in pilocytic astrocytoma when compared to other paediatric brain tumours. Researchers found mRNA to be up-regulated and the targets of the mRNAs were regulators of the ERK/MAPK pathway. It is believed that ERK/MAPK signalling pathways drive cellular proliferation and trigger senescence. Therefore it is suggested that this up-regulation of mRNA and the resulting effect on the ERK/MAPK pathway contributes to the unique phenotype of pilocytic astrocytoma.

This work was supported by the NIHR GOSH BRC and was published in Acta Neuropathologica Communications.

PATIENTS AND THE PUBLIC

Research awareness survey

In November 2015, 21 patients and 91 parents took part in our survey to let us know what they think about research. 88% of parents were interested in finding out about research into their child's condition, contrasting with young people, where only 19% had looked for research. 7/21 young people, and 38/91 parents reported having been asked to take part in research. Of these, 4 young people and 30 parents said they had taken part. We will be using the survey results to help us improve the way we communicate and engage with patients, families and the public about the research we carry out.



Parent/Carer Research Advisory Group

Our newly formed group had their third meeting on 22 February; three researchers attended to consult with the group and hear parents' perspectives on their projects. We have over 20 group members, with a range of experiences; about 80% have experience of parenting a child with a health condition and/or have had a premature baby, some have research experience and some have been involved in PPI previously. Any researcher at GOSH/ICH looking for PPI can consult with the group. To book a place or discuss your parent involvement needs, please contact Ruth Nightingale

Young Persons Advisory Group

Our Young Persons Advisory Group (YPAG) continues its active involvement in research taking place at GOSH and ICH. The group have recently been involved in advising Research Ethics Committee members via a Health Research Authority training day on the importance of including children and young people in research. We have also consulted with INVOLVE on payment for children and young people involved in research. Recently we welcomed three new members to YPAG, bringing the total number to 17, with children and young people aged between 8-18 years.

Continuing our work with Generation R, we have recently signed up to the Generation R Alliance and look forward to further establishing relationships with key stakeholders in paediatric research

PPI. For anyone wishing to consult with YPAG about any aspects of their research, please contact Erin Walker

PPI/E training
Upcoming PPI/E training masterclasses offered by UCL Partners in PPI/E includes:

Patient Involvement: 6th Sept 2016, 2.30-4.30pm. Boardroom, UCLPartners.
Person-centred care: 15th Jun 2016, 9.30-11.30am. Boardroom, UCLPartners.
Person-centred care: 29th Nov 2016, 2.30-4.30pm. Boardroom, UCLPartners.

If you would like any information or advice about PPI/E, please get in touch with Erin Walker and Ruth Nightingale, Joint Leads for PPI/E via research.ppi@gosh.nhs.uk

ARUK Adolescent Rheumatology Symposium 2016

On 28 January 2016, the second national Arthritis Research UK Adolescent Rheumatology Symposium was held. It was a free public event held at ICH and GOSH, with national and international speakers, who spoke on a wide range of novel and exciting topics related to adolescent rheumatology. There were 170 attendees including students, researchers, clinicians, healthcare professionals, industry and charity representatives, as well as members of the public. Sessions included talks on imaging, biomarkers and disease activity, management of rheumatic disease in adolescents and young adults, and young person involvement in research.

It was this last session that was most popular, as it allowed the audience to hear experiences of involving young people in research from academic teams, but the highlight of course was hearing from the young people themselves. Our Young Person Representatives, Dr Cat Wade and Ms Phoebe Rushe gave emotive and powerful speeches on some of their experiences as young people with a rheumatic disease and what it's like to be involved in research. They portrayed to the audience how important it is to involve patients in research development to improve the quality of the research conducted and how it empowers young people to ensure their voice is heard.

For more information on the Centre for Adolescent Rheumatology which is supported by both the GOSH and UCLH BRCs, please visit ARUK or follow @Rheum_Research on Twitter.

The London Engagement Network

The new London Engagement Network, has been set up to support those interested in public engagement. This network aims to foster cross-sector and inter-disciplinary work for those interested in creative and cross-disciplinary public engagement and is available for people from any sector, as well as providing early support and opportunities for early-career researchers to get experience in public engagement.

For more information or to join the network please click here

Click here to sign up to the mailing list

TRAINING

Child Health Research Summer Vacation Studentship scheme

The NIHR GOSH BRC will be funding two summer studentships this year, which will be part of the Child Health Research summer vacation studentship scheme. These studentships will be for projects relating to areas of translational clinical research/experimental medicine, and are available to undergraduate students studying science or medicine. Students will receive maintenance support of £190 per week for up to eight weeks.

Two supervisors per studentship are required; Postdocs and senior PhD students are particularly encouraged to get involved, as this is a great opportunity to add to your teaching and research output.

For more information and to download an application form click here

Doctoral Training Support fund 2016

The GOSH BRC will be continuing its support for doctoral trainees within GOSH and ICH by providing a consumables allowance to augment the existing non-pay funding available within their studentship awards**.** This is for translational clinical research/experimental medicine projects being undertaken by doctoral students within GOSH and ICH.

Visit our website for more information and to download an application form

EVENTS

Upcoming Events

BRC open meetings

As part of the renewal application for our BRC we are holding a series of open meetings. These will include an introduction from Professor Thomas Voit (Director Designate of the BRC) about the new BRC. This will be followed by an open discussion for people to share their ideas and thoughts to help shape the application for the renewal. There will also be an opportunity to have an informal discussion with individual theme leads after the meeting. We would love to see as many people from ICH and GOSH there as possible and are looking forward to hearing your thoughts

The dates and locations of the meetings are as follows:

Thursday 24th of March 12:00-13:00- Charles West Room, Level 2, Paul O'Gorman Building.

Tuesday 5th of April 14:00-15:00- Leolin Price Lecture Theatre, UCL ICH

Tuesday 12th of April 14:00-15:00- Leolin Price Lecture Theatre, UCL ICH

Introduction to Applied Genomics in the Clinic

The UCL institute of Child Health is holding a one-day course on the 7th of April, aimed at explaining how genomic medicine is becoming part of the day-to-day work of clinical laboratories and is based on real clinical cases. The course is open to general and specialist paediatricians and physicians, trainees in paediatric medicine and surgery, and genetic nurses and counsellors.

The BRC is sponsoring a limited number of free places for this course for GOSH staff. Please enquire about availability on booking.

Click here for more information and to register for the course.

Health and Care Innovation Expo 2016

The Expo is one of the NHS' biggest events, celebrating world leaders in science, research and innovation and will be returning to Manchester on 7th and 8th of September 2016. Early-Bird tickets are now available at a discounted price, for more information or to register please click here

'Spotlight on Research' May 9th-20th

To celebrate International Clinical Trials Day, staff from the GOSH BRC and ORCHID are arranging a series of research awareness events to put the 'spotlight on research'. The fortnight will involve a variety of activities for patients, their families and staff to raise the profile of the research at GOSH, showcase examples of the work we do and encourage all staff to engage with research. Highlights include a Research Trail around the hospital and a photo booth.

As part of the fortnight ORCHID are holding a one day conference showcasing research by nursing and allied healthcare professionals on the 9th of May from 9am-5pm, to book your place please click here. Other Staff activities will include a pub quiz (with a specially-developed research round) held at the Rugby on Thursday the 19th of May and a workshop led by artist Sofie Layton.

NIHR Research Training Camp

The seventh NIHR Infrastructure Doctoral training camp is due to take place on the 6-8th of July 2016 at Ashridge Business School. It is an intense three day annual event for NIHR trainees offering workshops, guest speakers and opportunities for networking and mentoring, the focus of this year's camp will be on 'A call for proposals'. Attendance is by invitation only.

2nd National Residential Training Weekend

The 2nd National Residential Training Weekend organised by the GOSH BRC will be held on the 1st-2nd of October at Ashridge Business School. The weekend is for clinical academic trainees working in child health and is open to medical, nursing and allied health professionals. The event is a great opportunity to network with fellow clinical trainees and academic leaders in paediatrics, providing a unique opportunity to develop research skills and gain career advice. Confirmed guest speakers include Professor Terrence Stephenson, Dr Shelley Dolan, Professor Jonathan Grigg, Professor Peter Callery, Professor Clare Lloyd and Dr Jack Kreindler.

The weekend is open to academics from training centres all over the UK and last year it was a great success. Subsidised attendance cost per individual is £75.

Celebration of ICH/GOSH translational excellence April 8th

To celebrate the translational excellence of ICH/GOSH there will be a series of talks from ICH scientists about how they have tackled moving their project from research in the clinic to commercial readiness. Please join us on the 8th of April 10.00-12.00 in the Leolin Price Lecture Theatre. Speakers will include Chris O'Callaghan, Persis Amrolia, Bobby Gaspar and a Panel consisting of representatives from the Translational Research Office, the R&D office and UCLB.

There will be opportunities to meet and discuss projects with the TRO, R&D and UCLB during the afternoon and in the following months.

New HRA Approval for research

The Health Research Authority (HRA) Approval is the new process for research in the NHS in England, bringing together the assessment of governance and legal compliance. This replaces the need for local checks of legal compliance and related matters by each participating organisation in England. Three cohorts of studies are currently eligible for the HRA approval process (currently not Clinical Trials, single site/sponsor and student studies). Further information on this can be found here.

Governance staff in the R&D office (2nd Floor, Philip Ullman Wing, ICH) will be available on **Wednesday afternoons** from **Wednesday 9th March** for researchers to drop in to ask questions and obtain further guidance on the new process.

Past Events

ICH Rare Disease Elevator Pitch event- Feb 25th

The first ever ICH Rare Disease Elevator Pitch was held on the 25th of February. The event was modelled on the theme of an elevator pitch, with members of the panel and audience voting for the pitch they would most likely invest in. Two prizes were awarded for the presentations; The panel winner was Dagan Jenkins for 'Drug treatment of Craniosynostosis', and the audience winner was Chris O'Callaghan for 'Translating findings from rare to common respiratory illness: disease models'.

BRC Trainee Day-March 10th

The annual NIHR GOSH BRC trainee day was an opportunity for BRC-funded trainees to meet other trainees and give a short presentation on their progress and future plans. The day saw a range of presentations from nurses, allied health professionals, those from a basic science background and some from a medical background. The trainees fed back that the day was really useful, particularly to hear about other opportunities open to them and that other trainees were facing the same problems as they were.

Rare Disease day-Feb 29th

The ninth National Rare Disease day was held on the 29th of February. To celebrate Rare Disease day at GOSH, a display was set up in the Lagoon where individuals were encouraged to share their thoughts and feelings about rare diseases. The display was really well-received and we have included some of the contributions made by patients and carers. To read more about the day, please visit the Rare Disease website.