ACADEMIC STRATEGY
2014-2019

Improving lives through excellent child health research and teaching
ICH Research Strategy 2014-2019

Over the course of 2012-13, staff at ICH have developed plans for a new academic strategy aimed at building on the position which we hold, with Great Ormond Street Hospital NHS Foundation Trust (GOSH), as one of the leading centres in the world for child health research and education. This strategy has been informed by the insights gained from visits to six other internationally excellent children’s academic medical centres and by detailed discussions internally and with our external partners, including GOSH, GOSH Children’s Charity, the wider UCL community, UCLPartners and funding bodies.

These ambitious plans require an organisational structure which will deliver them and ICH has been organised into five academic programmes and a cross-cutting theme which addresses Rare Diseases, which is fundamental to much of the activity at ICH/GOSH. This document describes, in outline, the course of our work over the next five years. Our intention is that we will deliver even greater benefits to the health and well-being of children and families we serve – both here, in UCL, and worldwide – they deserve nothing less.

Professor Rosalind Smyth
Director
UCL Great Ormond Street Institute of Child Health

ICH within the Faculty of Population Health Sciences

The Faculty of Population Health Sciences (FPHS) was created in 2011, as one of four Faculties within the School of Life and Medical Sciences. Central to the Faculty’s mission in research, education and enterprise is the study of health and disease throughout the life course. I was therefore delighted that ICH chose to be one of the four founding Institutes within the Faculty, and it is clear that ICH is ideally placed to pursue its new strategy as part of a community of researchers and teachers seeking to understand life course influences on health.

Recently the Faculty has been building upon and harnessing the potential of its intensively characterised clinical and community cohorts, which range from rare disease registries of fewer than a hundred patients to population cohorts of over 100,000 people.

This range is well-represented within the Institute of Child Health. Indeed the Life Study, the largest Birth Cohort Study ever to be conducted, is based within ICH. FPHS will support and enhance the interdisciplinarity of the UCL Great Ormond Street Institute of Child Health, from basic through to clinical and population health sciences. This will help translate the underpinning science and discoveries about the mechanisms of disease into treatments that will result in population health gain.

I warmly commend the new ICH strategy to you. With our colleagues in GOSH, our UCL Partners and other Faculties in UCL we will work together to improve child health, nationally and internationally.

Professor Graham Hart
Dean
UCL Faculty of Population Health Sciences
The mission of the UCL Great Ormond Street Institute of Child Health is to improve the health and well-being of children, and the adults they will become, through world-class research, education and public engagement.

The new academic strategy of ICH will be focussed on five scientific programmes and one cross-cutting theme:

- **Developmental Biology and Cancer**
- **Developmental Neurosciences**
- **Genetics and Genomic Medicine**
- **Infection, Immunity and Inflammation**
- **Population, Policy and Practice**
- **Rare Diseases**

**Four key principles** will underpin these programmes and the academic strategy; these are:

- **Interdisciplinarity**
  ICH will facilitate the best scientists, from all relevant academic and clinical disciplines, to work together to address fundamental questions to improve the health of children.

- **Accelerating translation**
  ICH will support the rapid translation of findings from basic discoveries relevant to understanding of health and disease, to clinical and population studies, more directly aimed at improving clinical care and promoting children’s health. We will also support “reverse translation” from observations in clinical and population studies to basic studies which address mechanisms of health and disease.

- **National and international partnership and leadership**
  National partnership with other UK academic, NHS, charitable, governmental and non-governmental organisations will support and enhance excellent child health research and teaching across the UK. ICH will also promote its mission through strategic international partnerships.

- **Developing our academic leaders**
  Developing our academic leaders, by providing the best possible supportive environment to enable all talented individuals who enter an academic career in child health, to progress and succeed, thus delivering an excellent academic capacity for child health.

Our activities will include active engagement with children and families, to ensure that our work is relevant and appropriate to their needs. We will generate the funding for our research by setting out our proposals in high quality applications to public, charitable and industrial funding bodies and we will disseminate the results of our research by publication in the medical and scientific literature, to practitioners, policy makers and the wider public. We will be highly visible ambassadors for child health at international conferences. Our teaching programmes will address the needs of students and professional groups who are interested in and undertaking work relevant to child health.
Developmental Biology and Cancer

- **Science underpinning Developmental Biology and Cancer**: to study the key processes necessary for normal development and tissue homeostasis that are altered in childhood diseases.

- **Birth Defects Research**: to use techniques of genetics and developmental biology to improve our understanding of, and develop novel treatments and preventive strategies for, clinically important birth defects.

- **Childhood Cancer Research**: to understand the molecular basis of childhood cancers and to develop more effective therapies for children with cancer, by combining basic research in cell and molecular biology with translational cancer research and clinical trials.

- **Stem Cells & Regenerative Medicine**: to promote and expand translational stem cell research at ICH/GOSH and harness the great potential of regenerative medicine for childhood disease.

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Developmental Neurosciences

- **Anatomical, genetic and functional aspects of neurodevelopment**: to foster basic and translational research with the aim of understanding the genetic and pathophysiological roots as well as anatomical and functional correlates of normal and pathological neurodevelopmental processes using innovative technologies.

- **Improving neurodevelopmental outcomes**: to promote early diagnosis and improve the management and treatment of children with developmental or acquired disorders of the brain.

- **Patient benefit**: to undertake clinical trials to investigate the role of innovative treatments to improve outcomes, contribute to the development of national guidelines and policies, and set national/international standards of clinical care for children with neurodevelopmental disorders and diseases affecting the function of the nervous system.
Genetics and Genomic Medicine

- **Genetics (and epigenetics) in Health and Disease:** to use genetics, imaging and biological indicators to understand predisposition to disease and what constitutes health during childhood and throughout the life course.

- **Gene and Protein Function:** to develop tools, skills and resources to elucidate gene function and to inform development of new therapies using state-of-the-art technologies.

- **Translational Genomics:** to create, integrate and maintain data and informatics platforms to support genomic, proteomic and other ‘omic research and its healthcare applications.

- **Personalised Medicine and Patient benefit:** to ensure basic science discoveries of disease mechanisms and patients’ genomes are used to best effect to improve patients’ lives. This will include better diagnostics, identification of biomarkers and targeting of therapies.

Infection, Immunity and Inflammation

- **Molecular basis of immunological and inflammatory disease:** to apply high-throughput genetics and systematic deep phenotyping of patients to understand the molecular basis of these diseases.

- **Basic immunological mechanisms:** to elucidate these mechanisms through experimentation in informative model systems and study of immunological development and function in children.

- **Pathogen action:** to understand molecular mechanisms of pathogen action through study of microbial genetics and host response to challenge.

- **Disease prevention:** to develop effective methods to enhance disease diagnosis in early childhood and utilise effective vaccination strategies.

- **Development of a world-leading translational research programme:** to undertake clinical trials in children with immunological and inflammatory diseases using novel biologic agents, and cell and gene therapies.

- **Respiratory, Critical Care & Pain:** To improve the understanding of early determinants of lung disease and the pathophysiology of children with life threatening respiratory disease, critically ill children on intensive care and children in pain and to develop novel therapeutic and management strategies to improve their outcome.
Academic Programmes: Strategic aims

Population, Policy and Practice

- **Research across the life course**: to promote the health of children, young people and the adults they will become, by: developing life-course methods across generations; testing epidemiological associations; generating hypotheses on causal mechanisms which can be tested through laboratory experiments and clinical trials; and informing public policy.

- **Address burden of disease**: to investigate common, chronic and complex conditions of children and young people, using population-based and clinical approaches.

- **Promote children and young people’s health**: to define and measure what matters for children, young people and their families and use this information to promote children and young people’s health.

- **Children in their contexts**: to carry out research on health services, the environment and other systems and contexts to prevent, diagnose and treat disease and to promote health and wellbeing.
Education

Building on the strengths provided by the academic programmes, we will promote research based education. We will expand our educational provision and student numbers by offering more general and specialist paediatric and child health related programmes. We intend that ICH will be the ‘go-to’ place for education in the world for any aspect of paediatrics or child health.

We aim to attract the best basic and clinical scientists who are interested in pursuing careers in child health related disciplines. Our educational offerings are tailored to their needs. We are also offering vocational courses in paediatric specialities that are designed to attract and educate clinicians, healthcare scientists and allied health professional in all aspects of child health and paediatrics.

Our current post-graduate taught programmes (Masters, PG Certificate and PG Diploma) are:

- Paediatrics and Child Health, with specialised pathways in Advanced Paediatrics, Community Child Health, Global Child Health and Gastroenterology
- Advanced Physiotherapy and Physiotherapy Studies, with specialised pathways in Paediatric and Cardiorespiratory Physiotherapy
- Child and Adolescent Mental Health
- Applied and Clinical Paediatric Neuropsychology
- Cell and Gene Therapy

Our post-graduate research programmes are:

- MRes Biomedicine
- PhD Child Health

In addition to the programmes we already offer we are developing new programmes, including an MRes in Child Health, which will allow both medically and scientifically qualified students to pursue a Masters level research programme. We are also expanding our undergraduate offerings to include an Intercalated BSc in Paediatrics and Child Health that will be open to MB BS/MD students from across the UK and we will contribute to the new UCL BSc programme in Population Health.
Great Ormond Street Hospital
NHS Foundation Trust (GOSH)

Together GOSH and ICH form the largest centre for paediatric research in Europe and one of the largest worldwide. We have established a reputation for world class, innovative research, education and practice that improves the understanding and treatment of childhood disease and child health. We recognise that our reputation and standing is based on our collective strengths and joint expertise in child health. With access to a diverse patient population, our clinical research facility, our excellent scientific infrastructure and facilities and our academic partners and collaborators, we will undertake world-class basic, translational and patient orientated research across the breadth of paediatric disciplines and child health. We will build upon our achievements, the high quality research, training and education that will further enhance our mission to improve the health of children and the adults they will become.

GOSH-UCL Biomedical Research Centre (BRC)

Biomedical Research Centres, the government’s flagship scheme for Experimental Medicine partnerships between NHS Trusts and Universities, were first established in 2007 under the auspices of the National Institute for Health Research. The GOSH UCL BRC, the only BRC to focus exclusively on children, was one of 11 centres in the country successfully refunded for a second 5 year term (2012-17) attracting £36m investment. BRC funding helps 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. GOSH UCL BRC funded research is organised into four themes.

- Molecular basis of childhood diseases
- Diagnostics and imaging in childhood diseases
- Gene, stem and cellular therapies
- Novel therapies for childhood diseases.
Great Ormond Street Hospital Children’s Charity

UCL Institute of Child Health works closely with Great Ormond Street Hospital Children’s Charity to help deliver the charity’s aim of supporting high quality research to drive the development of new therapies, devices and diagnostics to improve the lives of patients treated at Great Ormond Street Hospital. The charity – which is the UK’s largest dedicated charitable funder of paediatric research – has provided significant funding to the ICH over many years for new laboratories and equipment, as well as support for senior research leaders, PhD studentships and research grants. We look forward to continuing our special relationship with Great Ormond Street Hospital Children’s Charity and are especially pleased to be working with the charity and Great Ormond Street Hospital to create a new Centre for Research into Rare Disease in Children (CRRDC).

Centre for Research into Rare Diseases in Children (CRRDC)

For many years, through our research and clinical care, GOSH and ICH have been offering better diagnosis and groundbreaking therapies for children with rare diseases. With the revolution in genomics and innovative stem, cell and gene therapies, our ambition is take our ability to improve the lives of children with rare diseases even further.

We will build a new world-leading Centre for Research into Rare Diseases in Children (CRRDC) on the GOSH/ICH campus that will significantly enhance our research capability and expertise in the diagnosis, understanding, management and care of these conditions. The CRRDC will house a genomics facility that will allow us to understand disease and disease susceptibility more accurately than ever before and will deliver personalised medicine. The CRRDC will offer gene and cell therapies for a wide variety of inherited and acquired conditions including primary immunodeficiencies, metabolic diseases, and specific cancers. The CRRDC will support research groups in the rapidly growing area of regenerative medicine and will develop autologous cell based organ structures that can be transplanted into children with congenital malformations.

The cross-cutting theme for rare diseases will harness the excellence in these fields within the programmes:

- **To improve the lives of children with rare diseases through basic and translational research.**

- **To create greater visibility and emphasis on rare diseases** by enhancing research activity, facilitating joint working between ICH and GOSH and engaging with external partners.

- **To offer tangible benefits for children with rare diseases either at GOSH or nationally and internationally** using the latest technologies and life course research.
Strategic Partners

UCL Partners (UCLP)

UCL Partners is arguably the strongest and most developed Academic Health Science Centre in England. GOSH is a leading partner and UCLP has recently been re-accredited as an AHSC, with Child Health as one of its six leading programmes. The goal of the AHSC is to harness our academic strengths, partnerships and industrial links to the fullest extent to benefit the health and wealth of society.
Key facts about ICH

Research

In REF 2014, ICH was part of a UCL return to the Clinical Medicine sub-panel of Main Panel A. In this sub-panel, 80% of UCL research was assessed as either world-leading (43%) or internationally excellent (37%). In assessment of research power, UCL’s performance was top in the UK. The strength and range of Child Health research at UCL was specifically commended.

Research grant income is around £32m per annum.

Bibliometric assessment of journal articles has placed ICH/GOSH in the top five children’s academic medical centres in the world. It was joint third for citation impact, fourth for most highly cited papers and fifth on the number of publications.**

Thirteen Professors are Fellows of the Academy of Medical Sciences.

Ten Professors are NIHR Senior Investigators.

Education

ICH leads the Child Health component of the UCL undergraduate medical degree for approximately 370 students each year, a vital component of training tomorrow’s doctors.

ICH has nearly 400 postgraduate students, half of whom are undertaking our taught programmes (Masters, PG Diploma and PG Certificate) and half of whom are research students (MRes, PhD).

** Paper numbers, percentage of highly-cited papers and 5-year average citation impact (Evidence, Thomson Reuters Business) comparing papers published between 2008 and 2012 from GOSH/ICH with its International Comparators.

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