

Academic Strategy 2019-2024



Improving the health and wellbeing of children, and the adults they will become, through world-class research, education and public engagement

Welcome

The UCL Great Ormond Street Institute of Child Health (GOS ICH) is, with its clinical partner Great Ormond Street Hospital (GOSH), Europe's leading centre for child health research and education. Our mission is to improve the health and wellbeing of children and the adults they will become, through world-class research, education and public engagement.

It is an enormous privilege for us to deliver our ambitious mission, and in 2014, we launched a new academic strategy to achieve this, focussed on five programmatic areas (now Research and Teaching Departments). One of our key areas of strength is that we cover the full spectrum of research that is relevant to child health from genetic and molecular based science through clinical research to population and applied health research. Our academic strategy has enabled us to articulate more clearly both internally and externally our areas of strength and focus, and to accomplish our mission more effectively in a complex and evolving environment.

Over the last five years, we have demonstrated, validated by the reports of our International External Advisory Board, a growth and improvement in performance against exacting international metrics. During that time we have changed our name to reflect our very close partnership with Great Ormond Street Hospital NHS Foundation Trust (GOSH). In turn the partnership with the Hospital and the Hospital's Charity (GOSHCC) has strengthened and deepened, and integral to our activities have been the Great Ormond Street Hospital Charities' research strategy and the very strong engagement with the Great Ormond Street Hospital Biomedical Research Centre. The opening of the Zayed Centre for Research

into Rare Disease in Children in 2019 is a joint initiative with these partners and provides a world-class building and facilities to enable us to diagnose and treat children with rare diseases much more effectively.

Our refreshed Academic Strategy has been developed with detailed consultation internally and with external partners and has had very helpful input from our international External Advisory Board. As we enter this next five year period we feel confident that we will deliver even greater benefits in the future to the health and wellbeing of children and families whom we have the great privilege to serve. This focus remains at the heart of all our endeavours.



Professor Rosalind L Smyth

Director

UCL Great Ormond Street Institute of Child Health

GOS ICH within the Faculty of Population Health Sciences

The Faculty of Population Health Sciences 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 the Institute chose to be one of the founding Institutes within the Faculty, and it is clear that GOS 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.

The Faculty has built upon and harnessed 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. With the move to the interrogation of big data, and data science as a key strand in the new strategy, I am confident that cross-Faculty and cross-UCL collaboration will continue to 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 helps translate

the underpinning science and discoveries about the mechanisms of disease into treatments that will result in individual and population health gain.

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



Professor Graham Hart
Dean
UCL Faculty of Population Health Sciences

Equality, Diversity and Inclusion

We are proud to hold an Athena SWAN Silver award, renewed in 2017. In July 2018 we launched a new initiative, expanding the work we started under Athena SWAN, to address equality, diversity and inclusion issues more broadly. The new GOS ICH Equality, Diversity and Inclusion (EDI) Initiative, aims to be 'A VOICE FOR ALL'. We recognise that access and experience are not always equal for everyone and we want to redress the balance for all under-represented groups, alongside addressing barriers that affect the progression of the careers of women.

The EDI initiative consists of a Steering Committee and a number of Equality, Diversity and Inclusion Focus groups. The Focus Groups are open to all staff and students based at GOS ICH, and include Awareness, Community, Public Engagement, Wellbeing, Mums and Dads (MADs), Carers, Student, Researcher and Mentoring groups. They address issues that have been prioritised by staff and students at GOS ICH and represent – or 'voice' – the needs of different sections of the GOS ICH community (Read more on our website: www.ucl.ac.uk/child-health/about-us/equality-diversity-and-inclusion)

Strategy refresh

The consultation and discussions that were held to develop this refreshed strategy emphasised to us the success of the five academic departments and the continuing importance of our four key principles:

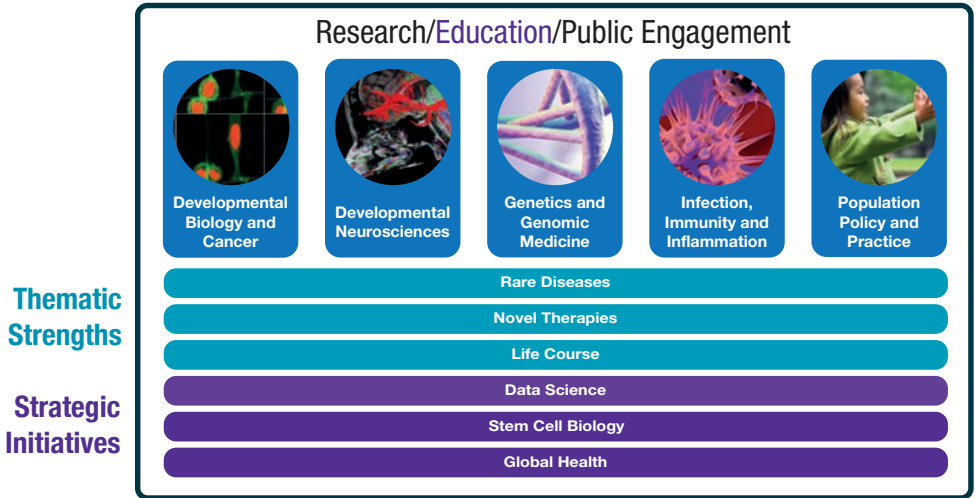
- interdisciplinarity
- accelerating translation
- national and international partnership and leadership
- developing our academic leaders

We were told that there had been a general upswing in activity and that the strategy had delivered greater inter-disciplinarity and collaboration across the Institute. We continue to recognise and celebrate thematic strengths in rare diseases, novel therapies and the life course. We are developing strategic initiatives, which address scientific areas where we have strengths and want to build those across

the activities of our five departments. These strategic initiatives are relevant externally and of importance to UK and international science.

Currently, our strategic initiatives are 'Data Science', 'Stem Cell Biology' and 'Global Health'. We will welcome the opportunity to add to these over the next five years. Education is at the heart of what we do and this refreshed strategy integrates a new Education Strategy, which will deliver world-leading child health education. Over the next five years we want to continue to grow our partnership with GOSH. We will seek to articulate a shared strategic vision in areas where we work together closely, such as in Education and Data Science and these will be developed more fully into joint strategies. Finally, our international partnerships have contributed strength over the last five years and we will broaden and deepen those including with other world-leading child health academic institutes.

UCL GOS Institute of Child Health working with GOSH



**Thematic
Strengths**

**Strategic
Initiatives**

Strategic initiatives

Data science

The vision of the data science strategy is to develop a research environment to support child health data science at scale. With its clinical and research base and unique data resources at UCL and GOSH (including the introduction of GOS ICH electronic patient records and an associated research platform), from omics to public health, GOS ICH should become world-leading in data science for children's health. Our vision is for project ideas to be supported from inception through to funding and data applications, project implementation, impact and data re-use, and for training in data science to be firmly embedded into the educational programme and offerings of the institute.

Our strategy aims to build skills, systems and collaborations for high quality research and to mitigate the risks inherent in using patient and population data:

- **Data systems, platforms and governance:** ensuring the needs of GOS ICH researchers are met by feeding into new and ongoing initiatives at GOSH, UCL and nationally; and supporting data applications and secure storage of large datasets
- **Education and training:** developing a new programme of short courses in data science aligned to the developing GOS ICH education strategy and PhD studentships in data science
- **Public engagement:** developing a GOS ICH data science webpage, training for parents, children and young people in data science topics
- **Supporting data science collaborations and team science:** holding seminars, coding clubs and networking events to encourage new cross-departmental working and grant income in data science

Stem cell biology

We are implementing a stem cell research strategy that builds on existing GOS ICH strengths to harness stem cell technologies for therapeutic uses. Therapeutic uses are broadly considered, ranging from cell-based therapies, mechanistic understanding of disease, target identification and validation, to applications for demonstrating efficacy of new therapeutics, including small molecules, biologics and gene therapies. Following a review and consultation process, four priority stem cell research activities were identified for further development:

- fundamental stem cell and developmental biology
- tissue engineering
- disease models and mechanisms
- therapeutic discovery

Much of the expertise in these areas is already present within the Institute, and we will enhance these areas by encouraging internal cooperation and strategic collaboration with the wider UCL community. A key aspect of the strategy will be building a dynamic stem cell research community within the Institute through a variety of mechanisms. These will include a graduate-level course in stem cell science, open to all first year PhD students; a postdoc-led seminar series that will host speakers from outside GOS ICH; and a translational forum of regular meetings with biotech and pharma groups using stem cell technologies in different stages of the drug development process.

Global health

Global health concerns the health of populations in the global context. Research activities aim to improve health, and achieve equity in health, for all people worldwide, paying particular attention to problems that transcend national borders or have a global impact. The majority of the world's children live in low- and middle-income countries, and have historically lacked access to the full benefits of research capacities that have developed in high-income countries.

GOS ICH is developing a new global health strategic initiative, aiming to identify fresh opportunities to target this critical dimension of child health. There are exciting opportunities for both clinical expertise in GOSH, and basic, clinical and population health science expertise in GOS ICH, to be shared more widely. Our strategy will build on ongoing collaborations with UCL's Institute for Global Health and a wide range of overseas research partners. Our vision is to maximise the benefits of the research capabilities at GOS ICH for the health and well-being of the world's children.



Research and Teaching Departments' strategic aims

Developmental Biology and Cancer

■ **Science underpinning Developmental Biology, Stem Cells and Cancer**

To study the key processes underlying normal embryonic development, tissue homeostasis and paediatric cancer and to advance the clinical management of children with congenital disorders and cancer.

■ **Birth Defects Research**

To increase our understanding of clinically important birth defects by combining genetics, innovative imaging, biochemical and developmental biology approaches and to develop novel treatments and strategies for prevention of these conditions.

■ **Childhood Cancer Research**

To understand the basis of paediatric cancer and improve the diagnosis and treatment through the combination of cutting-edge molecular and cellular approaches with preclinical and clinical research.

■ **Stem Cells and Regenerative Medicine**

To study stem cell biology and to develop novel, cell-based disease modelling, tissue engineering and regenerative approaches to improve the treatment of children with acquired or developmental anomalies.

Developmental Neurosciences

■ To use *in vivo* and *in vitro* model systems to fully understand cell and molecular mechanisms underlying disease processes in neurological diseases, and provide novel, personalised therapeutic interventions.

■ To develop rapid translational pipelines to diagnose and individualise therapies for children with rare neurological disease.

■ To progress biomarker development for monitoring of response to interventions.

Genetics and Genomic Medicine

- **Molecular Basis of Rare Diseases**
To employ genomic and functional analysis to understand the mechanisms of rare diseases and to use this insight to develop new therapeutic and early diagnostic strategies.
- **Cilia Disorders**
To conduct basic cilia research for translation into patient benefit by modelling cilia assembly, transport and signalling mechanisms, understanding fundamental genetic principles and designing novel ciliotherapeutics.
- **Inherited Disorders of Metabolism**
To develop the full translational pathway for inherited diseases arising from disorders of metabolism and specific organelles, by improving diagnosis, describing disease phenotype, discovering disease mechanism, and developing and monitoring new therapies.
- **Genome Biology and Personalised Medicine**
To understand the workings of the genome to better personalise disease treatment in children. We combine computational, population and omics analysis of rare variation and its genetic background to better assess differences in the molecular consequences of rare disease.



Infection, Immunity and Inflammation

- **Molecular basis of immunological and inflammatory disease**
To apply high-throughput genetics and systematic deep phenotyping of patients.
- **Basic immunological mechanisms**
To experiment with 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.
- **World-leading translational research**
To undertake innovative clinical trials in children with immunological and inflammatory diseases using novel biologic agents, and cell and gene therapies.
- **Respiratory disease and intensive care**
To improve the understanding of early determinants of lung disease in children, including critical care physiology, and development novel therapeutic and management strategies.



Population, Policy and Practice

- To improve understanding of aetiology through identification of the biological, economic, social and behavioural factors that act prenatally or during childhood to shape health and disease across the life-course and understanding the pathways through which they act.
- To develop and innovate scientific methods for child health including in data science, health informatics, biostatistics, and mixed methods research.
- To identify both the population-level burden of specific disorders/conditions and the person-level impact on affected children and their families of living with illness or disability, including inter-relationships between child and parental health.
- To reduce health inequalities and improve population health by identifying the policy, health, education, and social interventions that can promote health and prevent, or diagnose and treat, childhood disorders and disabilities
- To integrate research into practice and policy through translation into evidence-based clinical, social care, and public health practices, and public and social policies and working to ensure their implementation.

Education

Our mission is to provide an outstanding and inspirational student experience by promoting education as a key component of all existing activity, encompassing research, clinical and scientific endeavours.

Over the next five years we aim to develop a world-class education platform for health professionals and lay public and become as renowned internationally for education in child health as we now are for research. This will be achieved through research-led teaching using evidence-based education aligned with our partner GOSH. We will ensure that our students are offered an outstanding educational experience to equip them for a range of careers in child health related disciplines. To shape children's health in the future we will build on our existing educational portfolio:

Leadership and teaching on UCL

Undergraduate programmes:

- MBBS
- Intercolated BSc Paediatrics and Child Health
- BSc Population Health

Postgraduate taught programmes (Masters, PG Certificate and PG Diploma):

- Paediatrics and Child Health with specialised pathways in Advanced Paediatrics, Community Child Health, Global Child Health, Molecular and Genomic Paediatrics, Intensive Care and Clinical Practice
- Child and Adolescent Mental Health
- Cell and Gene Therapy
- Infancy and Early Childhood Development
- Applied and Clinical Paediatric Neuropsychology

- Physiotherapy Studies -pathways in Cardiorespiratory, Paediatrics and Neurophysiotherapy
- Personalised Medicine and Novel Therapies

MRes and MPhil/PhD degrees reflecting the diversity of research across GOS ICH.

The Short Courses and Events team provide an extensive programme of training. We will further develop the model of offering taught course modules to non-degree students. These avenues will provide one route to identify and encourage sustained engagement of the best child health students of the future.

www.ucl.ac.uk/child-health/events





Partnership with Great Ormond Street Hospital NHS Foundation Trust

Together GOSH and GOS 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 and excellent scientific infrastructure underpinned by our NIHR Funded GOSH/UCL Biomedical Research Centre (BRC) and clinical research facility, with our partners we 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.

2019 sees the opening of the Zayed Centre for Research into Rare Disease in Children, the UK's first purpose-built centre dedicated to paediatric research into rare diseases. The Centre is a partnership between Great Ormond Street Hospital (GOSH), University College London (UCL) and the Great Ormond Street Hospital Children's Charity (GOSHCC). It will bring hundreds of clinicians and researchers together under one roof to drive forward new treatments and cures for children with rare diseases.

Patient and public engagement

We raise awareness and keep people informed about research at GOS ICH through our engagement programme that encourages conversations between patients, the public, staff and our researchers. We run a diverse programme of events and activities including an annual NIHR BRC Family Fun Day as well as activities to mark Rare Disease Day and International Clinical Trials Day.

GOSH has a Young Person's Advisory Group (YPAG) who provide feedback to researchers to help them carry out research which is relevant to children and young people. YPAG is part of a national network of groups called Generation R.



Why do you think it's important for young people to have their say on research?

because the little things in research trials are a big deal to make their experience better - e.g. having a nicer happy nurse. e.g. games to play whilst waiting around. e.g. food after a fasting blood test.

It is only logical for US to shape the world WE will live in.

I think it's important for young people to have their say on research because young people have different views and needs and YPAG can help attend to those needs.

quotes from GOSH YPAG members

International partnerships

Over the last five years we have developed a number of key collaborative partnerships with outstanding child health academic science centres across the world. The focus of these partnerships has been to achieve a collaboration that is broader and deeper than individual collaborative programme/projects, and provides considerable added value to the existing collaboration between individual researchers. The nature of the partnerships varies, but these have now been established Hospital for SickKids Toronto and the University of Toronto, Boston Children's Hospital (Harvard) and the Murdoch Children's Research Institute, Melbourne, Australia. In addition, we work closely with outstanding academic Institutions in middle and low income countries where there is a clear focus on Child Health. These include Chongqing Children's Hospital in China, Chulalongkorn University, Bangkok, and The African Health Research Institute, KwaZulu-Natal, South Africa.



- ① Hospital for SickKids Toronto and the University of Toronto
- ② Boston Children's Hospital (Harvard)
- ③ The African Health Research Institute, KwaZulu-Natal, South Africa
- ④ Chulalongkorn University, Bangkok
- ⑤ Chongqing Children's Hospital in China
- ⑥ Murdoch Children's Research Institute, Melbourne, Australia

GOS ICH in numbers (2017-2018)

Our Impact



PEER-REVIEWED PUBLICATIONS

1207

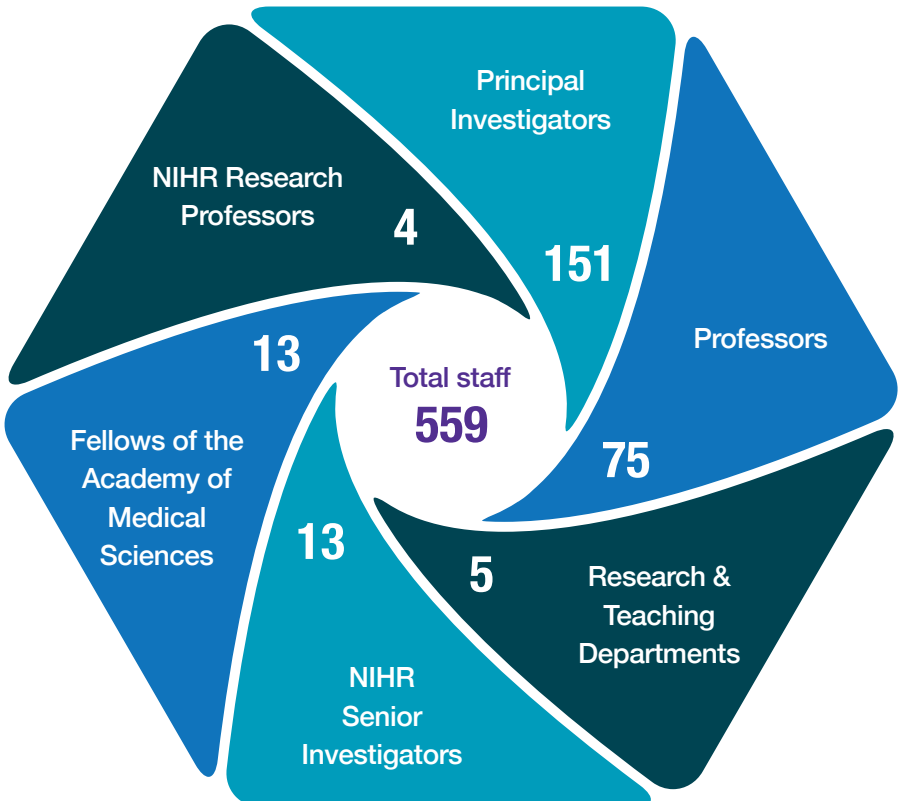


**Current active
research projects***
587 projects £200m



**Annual turnover
2017-18**
£64m**

*Active Sponsored Research with end date after 31/7/2018 **as at year end 31st July 2018



Education

PhD and MD(Res)
students

227



On RESEARCH
programmes

On TAUGHT
programmes

261



MSc/PG Diploma/
PG Certificate
and iBSc students

Independent short
courses
(1-5 days duration)

101



Education statistics 2017-18 academic year

Our dedicated Research Centres include:



Zayed Centre for Research into
Rare Disease in Children



NIHR GOSH Biomedical
Research Centre



Newlife Birth Defects Research
Centre



Human Developmental
Biology Resource



Dubowitz Neuromuscular Centre



Centre for Inborn Errors of
Metabolism



Arthritis Research UK Centre for
Adolescent Rheumatology



Reuben's Centre for Paediatric
Virology and Metagenomics



Obesity Policy Research Unit



Children's Policy Research Unit



Louis Dundas Centre for
Children's Palliative Care



UCL Great Ormond Street Institute of Child Health
30 Guilford Street, London WC1N 1EH

www.ucl.ac.uk/child-health

 @UCLchildhealth