



Strategy

2024 – 2029

Children first, always, everywhere

Welcome

The UCL Great Ormond Street Institute of Child Health is, with its clinical partner Great Ormond Street Hospital, Europe's leading centre for child health research and education. Our mission is to maximise and advocate for the holistic health of all children, young people and the adults they will become, through world-class research, education and public engagement.

It gives me enormous pleasure to present the UCL Great Ormond Street Institute of Child Health (UCL GOS ICH) academic strategy 2024-2029.

Since our last strategy we have seen continued progress in all areas, despite the challenges of the global pandemic. Together with our hospital partner Great Ormond Street Hospital (GOSH), we provide one of the highest concentrations of child health research worldwide and continue amongst the top five institutes with regard to outputs. The opening of the Zayed Centre for Research into Rare Disease in Children (ZCR) in collaboration with GOSH and GOSH Charity, has been a major step forward in delivering our strategy, along with the NIHR GOSH Biomedical Research Centre (BRC), the only paediatric BRC in the UK, renewed in 2022 incorporating links with Liverpool, Birmingham and Sheffield through the paediatric excellence initiative.

In addition to major advances in rare disease, our research is of relevance to the health of all children, with areas of expertise in obesity, epilepsy, infection and mental health as well as health inequalities and delivery of national trials. Two NIHR Policy Research Units, Children and Families, and Healthy Weight, led by UCL GOS ICH have been recommissioned (of a total 20 in the UK, five of which are based at UCL).

We have also cemented external partnerships globally, particularly through the International Precision Child Health Partnership (IPCHiP) with Boston Children's Hospital, SickKids Toronto and the Murdoch Children's Research Institute, Melbourne. With the development of a specific education strategy we have seen growth in existing and new programmes. We are proud to have achieved the Athena SWAN Gold award and regard equality, diversity and inclusion as integral to our values.

As we look to the future, we aim to be amongst the top three for child health research and educational institutions worldwide by 2029. We look to align strategies internally and with key partners, and highlight where we wish to be over the next five years in both education and research. We will maintain our departmental structure, incorporating previous strategic initiatives of data science, stem cell biology and global health as cross cutting themes and creating a Centre for Paediatric Mental Health, as well as a new strategic focus on cancer, and have set out our ambitions and priorities for the coming period.



**Professor
J Helen Cross OBE**

Director,
UCL Great Ormond Street
Institute of Child Health
and The Prince of Wales's
Chair of Childhood Epilepsy

UCL Great Ormond Street Institute of Child Health Strategy 2024-2029



Our vision:
Children first, always, everywhere

Our mission:

To maximise and advocate for the holistic health of all children, young people and the adults they will become, through world-class research, education and public engagement.

Our values:

- Excellence in research and education
- Accelerating translation
- Equality, Diversity and Inclusion
- Local, national and international partnership
- Developing leaders of the future
- Sustainability and climate change

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Child Health within the UCL Faculty of Population Health Sciences

The UCL Great Ormond Street Institute of Child Health continues to be a global leader in translating research based discovery to real world benefits for the most vulnerable in our populations and educating the next generation of child health leaders. While delivering world class research and education, UCL GOS ICH achievements in inclusion and diversity has been exemplary with staff and students united by a desire to deliver tangible benefits to everyone in our wider community.

I have set out the [Faculty of Population Health Sciences' 2022-27 strategy](#), mirroring UCL's Grand Challenges in the areas of climate change, inequalities and mental health and wellbeing, with additional focus on health security, cancers, trials, data science and rare diseases. UCL GOS ICH, our largest Institute, has made, and will continue to make, significant contributions to addressing these UCL grand challenges and the goals of our faculty strategy. I am delighted to see multiple alignments between the UCL GOS ICH academic strategy 2024-2029 and our Faculty's and UCL's strategy documents. I am equally pleased to see good alignment with the strategic direction of the Great Ormond Street Hospital and the GOSH Charity.

The Faculty Strategy 2022-27 highlights the cross-cutting importance of taking a "life course" approach to population health. Childhood is a critical element of this approach; many of the factors that adversely impact on adult physical and mental health, such as inequalities and the impact of deprivation, can be best mitigated in early life. Research at UCL GOS ICH continues to improve the lives of children by identifying new diagnostics and therapeutics in specific disease areas, but also identifies the social determinants of health and mobilises research to advocate for better opportunities for all, influencing policy makers and wider society.



Professor Ibrahim Abubakar
Dean,
Faculty of Population Health
Sciences and Pro-Provost
(Health), UCL

UCL GOS ICH shows how we are greater than the sum of our parts and that partnership working brings great dividends. We are privileged at UCL in working closely with our external partners as well as drawing on our diverse inter-disciplinary community. In particular, the NIHR GOSH Biomedical Research Centre (BRC) is a key partnership, the only paediatric BRC in the UK, working with colleagues in Liverpool, Birmingham and Sheffield through the paediatric excellence initiative. International collaborations with other leading centres for child health research in Boston, Toronto and Melbourne illustrates another dimension of UCL GOS ICH's leadership. Such collaborations are both strategically important and also exemplars of our true national and international reach.

In addition to being a key player in national collaborations and Centres of Excellence, our academic teams at the UCL GOS ICH are addressing some of the most pressing global health challenges including newborn mortality, infant and child malnutrition, disability, epilepsy and other chronic conditions, vision, water sanitation, infection, early marriage, mental health and racism. Much of this work is being conducted in partnership with teams based in lower and middle income countries and involve substantial effort in local capacity building to nurture the next generation of global child health researchers. This international work will become even more important over the next five years and beyond as we address climate that will impact health outcomes for our children, and the adults they will become.

I wish my colleagues every success in implementing such a wide-ranging and ambitious programme and I have the confidence that they will continue to deliver key elements of UCL's strategic objectives to benefit children and young people across the world.

Our ambitions

1. Excellence in research and education

a. Increase our research portfolio. To achieve this ambition we will:

- increase applications to funding bodies that attract Full Economic Costing (FEC) and Quality Research (QR), increasing research funding by 20% by 2029;
- maximise our USPs in areas including developmental neurosciences, genomics, cancer, rare diseases, infection, life course and population health science;
- promote and foster research applications in priority areas of children's mental health, global health, data science and stem cell biology;
- ensure all research advocates for equalities in care.

b. Expand our education portfolio. To achieve this ambition we will:

- create new education courses, by promoting and facilitating engagement with other departments within the Faculty of Population Health Sciences and wider UCL;
- refine and consolidate our current education portfolio of courses, aligning with our research strengths;
- increase the number of students by 20% by 2029;
- ensure education activities are part of the portfolio of all academic, research and teaching staff.

2. Accelerating translation

Work in partnership with the NIHR GOSH BRC, Policy Research Units, UCL, UCLP, AHSC and hospital partners as well as charities to translate our research findings into clinical relevance. To achieve this ambition, we will:

- continue to promote and facilitate the development of novel therapies through innovation and enterprise;
- increase clinical trials (in parallel with the NIHR GOSH BRC and the Institute of Clinical Trials and Methodology (ICTM));
- advocate for policy change through evidence-based research.

3. Equality, Diversity and Inclusion

Continue to ensure EDI principles are embedded in all departmental processes and those of the Institute to build on an inclusive, supportive and fair working environment. To achieve this ambition, we will be:

- renewing our Athena SWAN Gold Award submission;
- supporting an inclusive culture and community at the Institute;

- working to protect and improve work/life balance;
- ensuring recruitment and career progression opportunities are clear and equitable at all levels.

4. Local, national and international partnership

Continue and grow local, national and international partnerships.

To achieve this ambition, we will:

- promote partnerships within and across UCL Faculties, hospital partners and charities;
- work nationally with partners to develop, deliver and communicate on child health research priorities;
- work internationally with partners to deliver on rare disease research and strive to be in the top three child health research institutes in the world.

5. Developing leaders of the future

Develop our academic, teaching and professional services staff in becoming leaders of the future. To achieve this ambition, we will:

- promote and increase fellowship applications and success at all levels;
- enhance joint working with Great Ormond Street Hospital and training bodies to develop academic training in child health;
- encourage all staff to develop through management and leadership training.

6. Sustainability and climate change

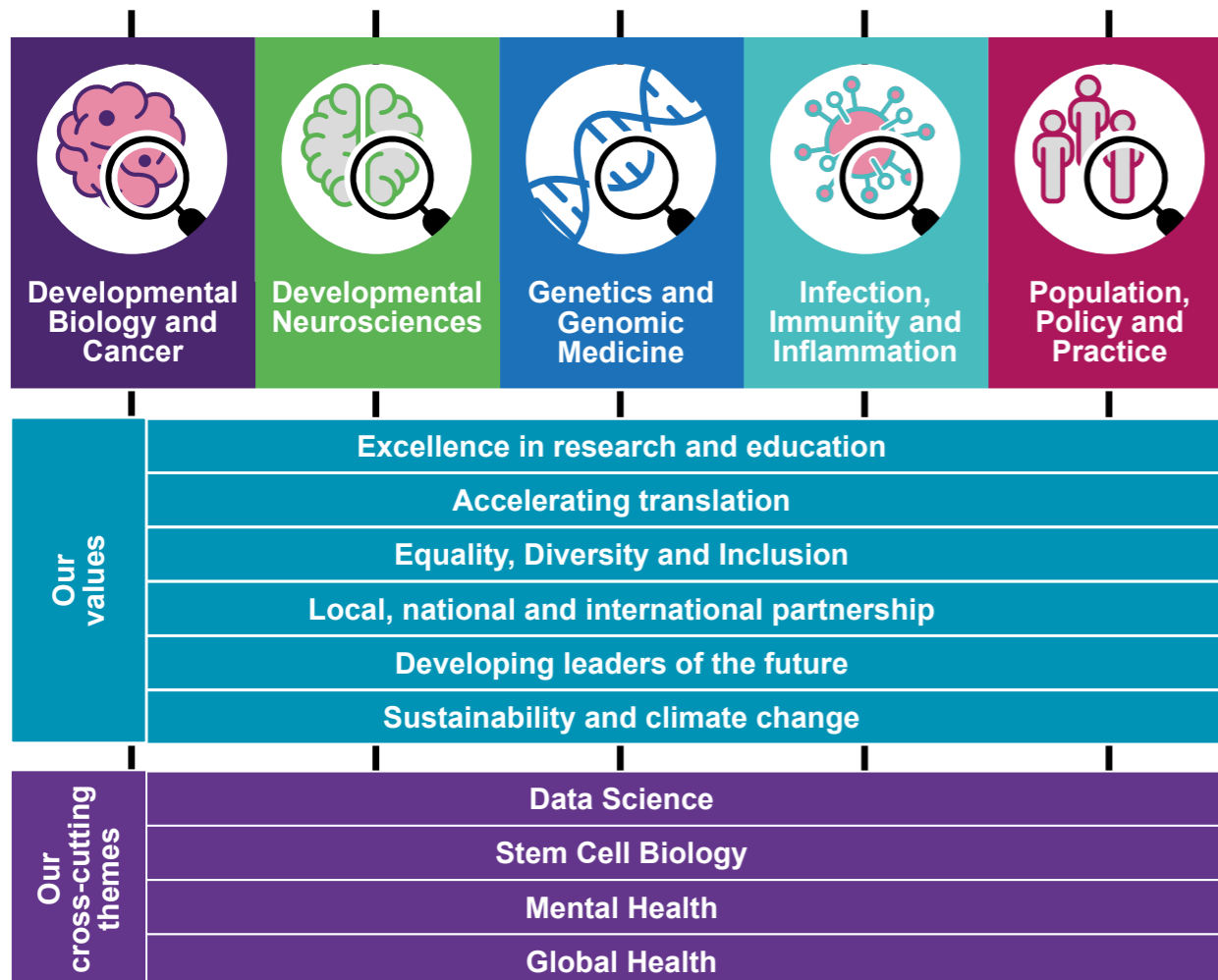
Lead on the UCL sustainability strategy, encouraging and empowering our staff to do their part in co-creating a world where everyone and especially children and young people can live together within the capacity of the planet. To achieve this ambition, we will:

- work towards climate change initiatives that will impact health outcomes our children and the adults they become;
- enable research into the impact of climate change on the health of children and young people;
- ensure that all that we do incorporates climate change awareness including our labs and offices applying for the UCL LEAF and Green Impact programmes;
- ensure all our purchasing, disposal and recycling of equipment is undertaken with sustainability in mind.

Our cross-cutting themes' priorities

In the next five years, our cross-cutting themes will focus on the following priorities:

UCL GOS Institute of Child Health



Data Science

The aim of the Data Science cross-cutting theme is to improve the quality, quantity and pace of data science-related research at UCL GOS ICH, to support our mission: to 'maximise and advocate for the holistic health of all children, young people and the adults they will become, through world-class research, education and public engagement'. Our vision is to develop a research environment to support child health data science at scale. UCL and GOSH hold world unique data resources and infrastructure supporting research from 'omics to public health. This includes the Digital Research Environment, allowing anonymised patient records from GOSH to be used for research, and Education and Child Health Insights from Linked Data (ECHILD), linking health, education and social care records for over 20 million pupils in England born since 1984. We firmly believe that Data science is team science. Therefore, as well as continuing to support large data resources and infrastructure for child health research, the data Science initiative focuses on capacity building and career development for the excellent data scientists across UCL GOS ICH and GOSH.

Priorities for the next five years:

1. Work towards establishing further mid-career permanent positions (lecturers and associate professors) in data science at UCL GOS ICH.
2. Support dedicated and sustained UCL institutional investment in data holdings (including UCL wide holdings of national primary and secondary care data resources such as CPRD and Hospital Episode Statistics), and continued developments and accreditation of the UCL data safe haven to hold and process sensitive data, including NHS data.
3. Develop clear pathways for UCL institutional support for setting up of national data resources (ECHILD, KEHC).



Global Health

Our academic teams are addressing some of the most pressing global health challenges of our era – including newborn mortality, infant and child malnutrition, disability, epilepsy, vision, water sanitation, infection, early marriage, mental health and racism. Much of this work is being conducted in partnership with teams across many of the countries in the Africa region and South Asia, and involve substantial effort in local capacity building to nurture the next generation of global child health researchers. For example, researchers from UCL GOS ICH have worked with teams in South Africa, Malawi, Zimbabwe and Bangladesh to develop an award-winning digital learning health system that improves postnatal newborn care and survival (Neotree). In several African settings, trials have shown that different forms of nutritional supplementation can prevent and treat severe undernutrition. Trials in China and the UK have shown that an intervention to improve the mental health of breastfeeding mothers improves the growth of their infant. Our research in Nepal has also shown the many penalties for women and their offspring associated with girls being married as children. Complementing our overseas activities, we have a strong track record of rapid innovation and translation of evidence and knowledge to the UK setting – for example in community engagement and empowerment.

Priorities for the next five years:

1. Increase our research contribution to the goal of promoting equality and equity in child health.
2. Support the career development of Early Career Researchers at UCL GOS ICH prioritising global child health.
3. Increase the mentorship we provide to collaborating child health researchers in low- and middle-income countries.

Mental Health

Since the COVID-19 pandemic led to serious disruption to the everyday lives of children and adolescents, there has been increased attention to the impact on child mental health in the general population. This focus has largely eclipsed the equally important needs of children with physical health problems, such as epilepsy, genetic disorders, and serious life-threatening conditions that present primarily to paediatric specialists. Emotional and behavioural problems often accompany such conditions, but are rarely assessed as a matter of routine, or treated, in part because of a lack of awareness, in part because of a paucity of expertise. Among the academic mental health specialists at the Institute, there is a wealth of expertise in both the appropriate assessment and management of paediatric mental health problems associated with physical health disorders, as witnessed by the many grants and academic papers produced by those experts. Currently, no leading paediatric hospital in the world has a specialist centre whose focus is to support children with chronic and severe conditions necessitating ongoing medical care and supervision. Our objective is to create a Centre of Paediatric Mental Health at UCL GOS ICH, bringing together interdisciplinary expertise from colleagues at UCL GOS ICH and the wider UCL community, to take an international lead on innovative interventions to improve the lives of children with chronic paediatric conditions.

Priorities for the next five years:

1. Increase the visibility of, and to raise awareness about, mental health expertise at UCL GOS ICH/GOSH within UCL, the UK and internationally by creating a Centre for Paediatric Mental Health.
2. Attract funding for paediatric mental health research.
3. Improve interventions for paediatric mental health disorders through research, education and advocacy.



Our Research and Teaching Departments' priorities

UCL GOS ICH has five Research and Teaching Departments. Over the next five years, they will focus on the following priorities:

Stem Cell Biology

We are developing broad expertise in human stem cell biology, focused on using induced pluripotent stem cell (iPSC) systems to understand physiology, model rare childhood-onset disease and develop novel effective treatments for future clinical translation. Model systems are developed from both patient-derived and CRISPR-generated iPSC, which are differentiated into the appropriate cell cultures or 3D cellular systems for studying physiological and disease states. We now have capabilities in iPSC modelling for neurological, neuromuscular, eye, liver, immune and respiratory diseases and hope to expand further to other organ systems. We are also applying state-of-the-art technologies such as single cell and spatial transcriptomics and advanced electrophysiology approaches to identify disease-specific cellular phenotypes. We are establishing proof-of-concept for using these models to test new therapies including DNA and RNA-based genetic therapies, novel small molecule therapies and high throughput drug screening. Our goals will be to network and expand our knowledge widely throughout UCL, and more broadly within the UK and internationally through research collaborations and initiatives. Our monthly seminars and workshops will also provide an important source of education for researchers in the field.

Priorities for the next five years:

1. Integrate the UCL GOS ICH Stem Cell Initiative across UCL, the London Stem Cell Network and other UK universities. We aim to develop a national stem cell network that will connect researchers nationally, for example through an MRC rare disease node, technology hub or equivalent.
2. Advance research, create new collaborations and develop research synergy.
3. Advance knowledge and encourage Early Career Researchers into stem cell research through educational initiatives.



Developmental Biology and Cancer Research and Teaching Department

The overarching goal of the Developmental Biology and Cancer Research and Teaching Department (DBC) is to study the mechanisms underlying embryonic development and tissue homeostasis; advance our understanding of the causes of paediatric cancer and congenital anomalies and; to develop treatments for these conditions.

Key achievements over the last five years:

Over the past five years DBC researchers have engaged in discovery science enhancing our understanding of nervous system development, renal defects, cardiovascular morphogenesis, craniofacial malformations, pituitary development, stem cell-derived organ models and mechanisms underlying solid and haematological tumours.

This work has provided the basis for translational research aiming to provide new treatments for children through genetic therapies for inherited disorders of hearing, metabolism and kidney function, cell-based and drug therapies for childhood cancer, nutritional interventions to prevent birth defects and stem cell and regenerative strategies to repair disorders of organs such as gut and eye. DBC researchers have led pioneering clinical trials in rare brain cancers and leukaemia, and interventions to improve surgical outcomes for conjoined twins and babies with spina bifida.

DBC researchers participate in numerous national and international partnerships and play key roles as UCL Leads in national networks including the UK Rare Disease Research Platform (REOLUT Node), Human Developmental Biology Initiative, Human Developmental Biology Resource, INCAR (Innovative CAR Therapy Platform), and National Mouse Genetics Network (Congenital Anomalies Cluster).

Alongside academics and clinicians, DBC has hosted holders of fellowships from several funders including Wellcome Trust, UKRI, MRC, British Heart Foundation, Kidney Research UK and Alzheimer's Research UK.



Priorities for the next five years:

1. To increase our understanding of congenital anomalies and paediatric cancer through exploiting opportunities for multi-disciplinary research and partnerships within and between departments, across the wider UCL, with GOSH partners, nationally and worldwide.

Metrics:

- Initiate and develop three+ cross-department and/or cross-faculty strategic initiatives in research and education over the next five years;
- Take a leading role in national strategies in rare diseases and cancer (and aligning with the GOSH Charity cancer research strategy);
- Initiate new strategic links with other centres of excellence in Europe with expertise in congenital anomalies and paediatric cancer.

2. To support the development of the next generation of researchers and educators in developmental, stem cell and cancer biology underlying congenital anomalies and paediatric cancer.

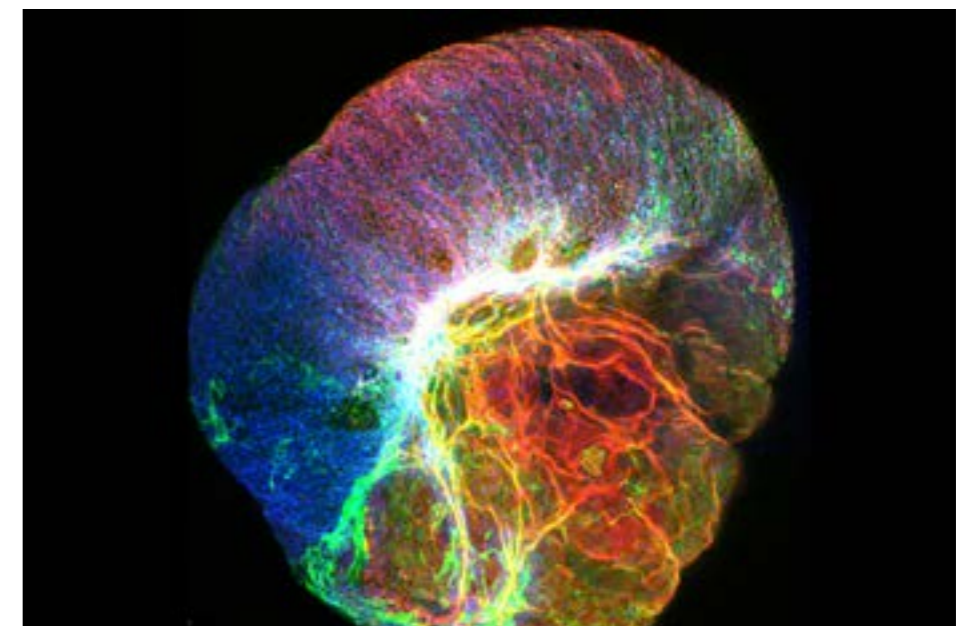
Metrics:

- Through mentoring, peer support and pro-active outreach we aim to increase the number of successful funding applications led by ECRs (fellowships and other grants) and to provide opportunities to gaining teaching and supervisory experience, including contribution to development of new courses/modules.

3. To develop improved therapeutic and preventive strategies for treatment of children with paediatric cancer and developmental anomalies through pre-clinical and clinical research, and to capitalise on commercial opportunities to advance these translational programmes.

Metrics:

- Increased support for translational research through grant funding, venture capital, and partnering with industry;
- Development of clinical trials and guidelines to treat children with congenital anomalies and paediatric cancer;
- Through building on links with UCL-B and Great Ormond Street Hospital (including the GOSH Children's Cancer Centre) increasing the number of patents filed to protect IP.



Credit: Giada Benedetti
NIHR GOSH BRC Research
Image Competition

Developmental Neurosciences Research and Teaching Department

The primary focus of the Developmental Neuroscience Research and Teaching Department (DN) is to minimise the impact of disorders affecting the developing central, peripheral nervous system and muscles, by studying the mechanisms of injury and repair, improving diagnosis and prognosis, evaluating therapeutic strategies and optimising functional outcome.

Key achievements over the last five years:

DN Researchers have contributed two impact studies to the [UCL REF 2021 submission](#):

- Developing the first treatments for life-limiting neuromuscular diseases that affect over two million children - Duchenne Muscular Dystrophy and Spinal Muscular Atrophy – resulting in FDA approved therapies (eteplirsen and golodirsen for DMD, Spinraza for SMA).
- Transforming the lives of children with severe epilepsy – state of the art imaging and a new care pathway devised by clinical scientists at UCL are transforming the lives of hundreds of children with severe epilepsy. The team has built international clinical networks and developed machine learning tools that help ensure children suitable for curative surgery can benefit.

Neurogenetic research in Developmental Neurosciences has attracted over £7M of research funding for translational genetic research. This has resulted in the establishment of rapid exome analysis in infants with epilepsy (international GeneSTEPS study) for implementation into clinical practice, as well as a new UCL spinout – Bloomsbury Genetic Therapies – for the development of novel gene therapies for rare neurometabolic and neurodegenerative diseases. The genetic therapy accelerator has been established across UCL GOS ICH and the Institute of Neurology aiming to consolidate and widen outreach of translational research.



Priorities for the next five years:

1. Development of new research initiatives to fundamentally increase our understanding of aetiology and disease course of disorders of the nervous system and muscles with the aim of developing novel diagnostics and ultimately effective/ curative treatments.

Metrics:

- Achievement of major advances in stem cell based disease modelling for neurological disorders with the development of model systems for testing gene therapy, CRISPR editing, RNA therapies and small molecule drug development;
- Development of advanced AI-aided imaging and diagnostic decision support with evaluation in clinical practice, and objective imaging biomarkers for treatment trials, in close collaboration with the GOSH Digital Research Environment and Department of Radiology, and wider UCL.

2. Ensure an impact on neurological health outcomes through research innovation and commercialization.

Metrics:

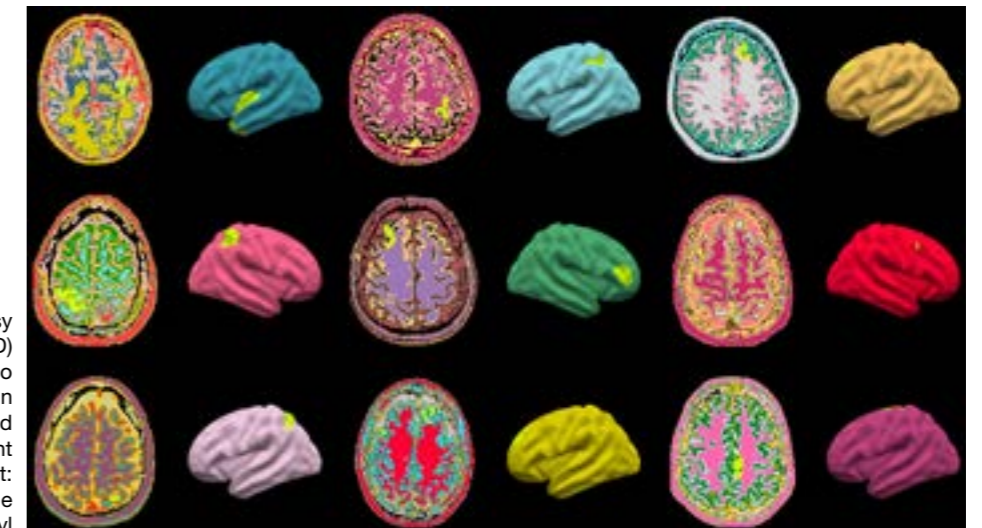
- Major expansion of clinical trials in novel therapies, small molecules and gene therapies, with a focus on commercial enterprise;
- Providing a greater voice to patients and families in our work through PPI Initiatives and utilisation of generically relevant disease/patient support groups;
- The creation of a new academic unit of functional neurosurgery to consolidate collaboration with state-of-the-art neurotechnology for the treatment of severe neurological syndromes;
- Development and translation of at least two further neuromuscular therapies.

3. To expand our education portfolio, by increasing access through online course development and new short courses.

Metrics:

- Develop two+ new short educational courses on novel translational technologies and one hybrid accredited diploma course.

The Multicentre Epilepsy Lesion Detection (MELD) project creates AI tools to detect subtle MRI brain abnormalities in children and adults with drug-resistant epilepsy. Image credit: Mathilde Ripart, Dr Sophie Adler, Dr Konrad Wagstyl



Genetics and Genomic Medicine Research and Teaching Department

The Genetics and Genomic Medicine Research and Teaching Department (GGM) comprises a multi-disciplinary group of researchers whose aim is to better understand, diagnose and treat a wide range of rare (and more common) genetic conditions.

Key achievements over the last five years:

In the past five years, GGM researchers have used unique patient cohorts and models to discover many new genetic causes and mechanisms of human disease. UCL Genomics and the UCL Centre for Translational Mass Spectroscopy have supported with state-of-the-art technologies for discovery research and diagnostics and contributed significantly to national SARS-CoV2/COVID-19 sequencing and the development of novel biomarker tests (COG-UK, Operation Moonshot). Our translational research team based in the North Thames Genomic Laboratory Hub led developments in non-invasive prenatal diagnosis and rapid genome sequencing resulting in implementation of globally unique NHS services to diagnose genetic conditions prenatally and in early life.

GGM investigators lead many National and International research networks and trials, including pioneering novel viral and non-viral genetic therapies (e.g. UKRI MRC National Metabolomics/Lipid and Platform of Nucleic Acid Therapy Nodes, LifeArc Respiratory National Translational Centre, NATA Delivery Challenge Consortium, CF Trust Strategic Research Centre, Livingstone Skin Research Centre), resulting in patents, spin out companies (Bloomsbury Therapeutics, Axovia, Guilford Street Laboratories) as well as significant research partnerships internationally and consultancies with industry. Patients and advocacy are key in our work, they lead in some of our workstreams to ensure equitable and sustainable research with better access for all to medicines and trials. Our extensive educational programme includes our MSc in Personalised Medicine and Novel Therapies and support for 20-30 clinical and non-clinical PhDs students at any time.

Priorities for the next five years:

1. To gain a deeper understanding of disease mechanisms and diagnosis by utilising powerful rare disease DNA and tissue cohorts, disease models (human stem cell, whole animal and organoids), and advances in genome sequencing, single cell and spatial transcriptomics and translational mass-spectrometry.

Metrics:

- Increase our leading role in national/international initiatives in genetics and genomic medicine research by leveraging emerging technologies, supported by UCL Genomics, for analysis of gene variants, transcriptomes and



molecular interactions, within the spatial organisation of cells and tissues and at single cell level;

- Expansion of model systems for genetics and genomic medicine research, utilising rapid human, mouse, yeast, zebrafish mutants, neuroscience screens, high-content and drug repurposing screens, and new functional biotools (e.g. biosensors);
- Grow our diagnostic capabilities and personalised disease treatments with new clinically reliable molecular genetic tests, novel biomarkers and drug targets, supported by developments in computational infrastructure, AI-machine learning and large data panels for translational mass spectrometry and rare disease-omics.

2. To advance the preclinical and early clinical development of innovative genetic therapies, including non-viral and viral gene therapy, genome editing, and expansion of RNA/nucleic acid therapies.

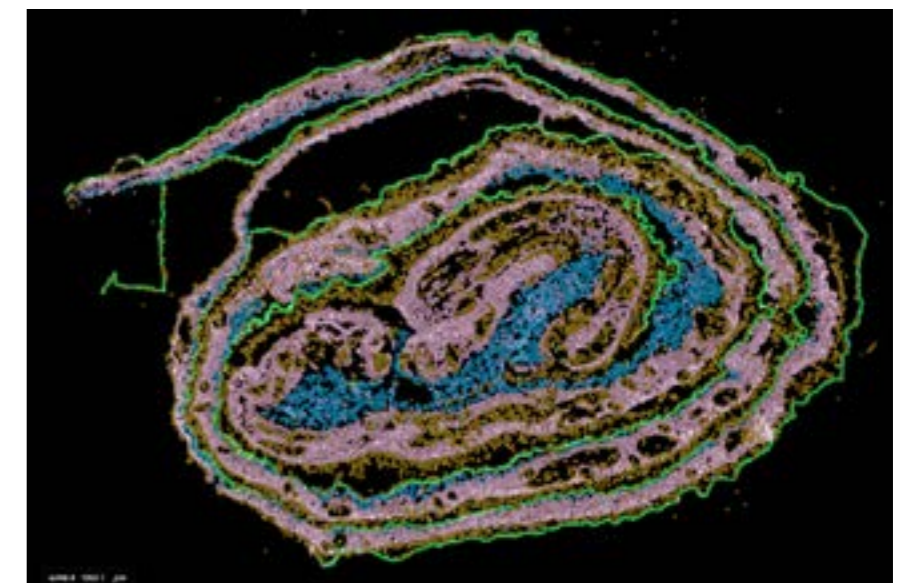
Metrics:

- Target a large range of rare diseases to generate proof-of-concept evidence that mitigates risks in the translational pipeline, attracting new in-kind matched and stand-alone industry funds;
- Increase external multidisciplinary collaborations and partnerships with national and international organisations, that lead to academic-led clinical trials of genetic therapy;
- Increase regulatory body engagement to streamline transition of therapies to clinical application.

3. To provide a thriving research environment with research and educational opportunities supporting the professional development of our early career researchers.

Metrics:

- Foster support for ECRs through mentorship, transferable skills development including data science and commercial enterprise, increased links to NHS clinical cohorts and translational research opportunities, facilitating ECR fellowships and co-investigator led grant applications;
- Extend our inter-disciplinary expertise to other ICH/UCL departments in collaborative initiatives that contribute to mentoring, project supervision, personal tutoring, thesis committee support;
- Expand our teaching contribution through support and development of postgraduate courses.



Credit: Theodoros Xenakis
Spatial transcriptomics

Infection, Immunity and Inflammation Research and Teaching Department

The Infection, Immunity and Inflammation Research and Teaching Department (III) aims to deliver world class interdisciplinary research for children with infectious, immunological and inflammatory disease, children with life threatening respiratory disease, and critically ill children on intensive care. Our research also aims to understand basic immunological mechanisms by studying the immunological development and function in children.

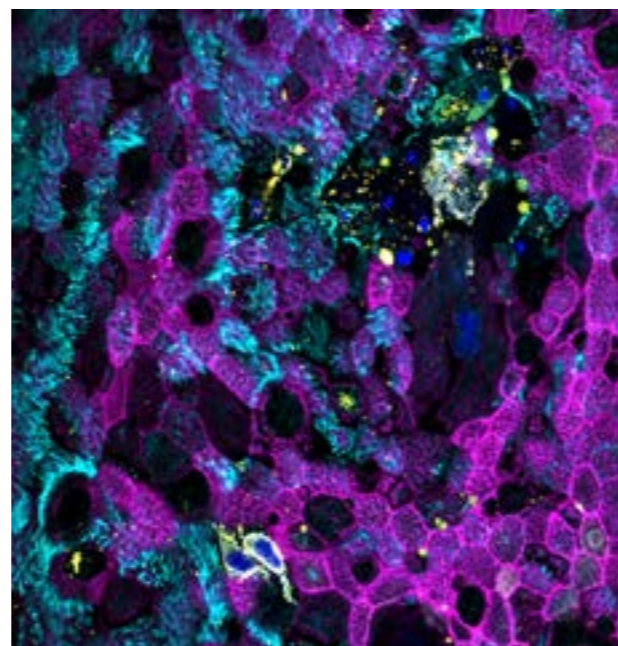
Key achievements over the last five years:

The III Department is one of the largest within UCL GOS ICH and aims to deliver world class interdisciplinary research for children with infectious, immunological and inflammatory diseases. Our research truly has an impact on the lives and health of children and families with these conditions.

Our infection section integrates basic science, epidemiology and clinical trials across vaccine research, virology and microbiology with findings relevant globally. Work from our investigators was critical during the COVID-19 pandemic providing insights into immunity and leading to over 20 publications in high impact journal including NEJM and Nature. A new cause of a viral hepatitis outbreak was also identified using cutting edge pathogen genomic tools developed here and reported in *Nature*.

Building on research improving understanding of disease mechanisms and biomarkers, our inflammation section is expanding knowledge and developing new treatments for patients with immunological and inflammatory diseases. In the past few years, the team has led clinical trials in Kawasaki Disease and Polyarteritis Nodosa using novel methodologies specific to rare diseases which has now been adopted by other NIHR trials. The GOSH/UCL Autoinflammation Centre of Excellence has discovered 15 new genes in the past seven years and developed functional assays now widely available in the NHS. The UCL Centre for Adolescent Rheumatology/JDM study has proposed new treatments in childhood onset myositis and is leading a new clinical trial proposal for Juvenile Dermatomyositis (JDM).

The molecular and cellular immunology section has consistently delivered pioneering translational research developing therapies for rare genetic diseases using gene and cell therapies which has



Credit: Dr Maximillian Woodall

directly benefitted patients. In the past five years we have opened 8 first in man innovative clinical trials to treat children with inherited immune disorders and haematological malignancies. We also host Europe's only thymic transplantation programme and related research has led to the identification of genes implicated in novel immune deficiencies such as PAX-1.

We also have a very strong track record in teaching and education with investigators leading on 6 MSc courses and numerous post-graduate and short courses.

Priorities for the next five years:

1. To combat pathogen resistance through screening and mechanistic studies to develop diagnostics and novel/re-purposed therapeutics.

Metrics:

- An increase in and streamlining of laboratory capacity (including enabling CL3 pathogen research) to support clinical trials and surveillance through infrastructure grant funding, service provision, and partnering with industry;
- Development of clinical trials, guidelines and surveillance strategies for important pathogens and support of testing and development of vaccines through new strategic links within UCL infection and with national and international partners;
- An increase in Infection-related training courses building on links with Great Ormond Street Hospital.

2. To understand disease mechanisms (genomics and multiomics) and develop novel/targeted therapies for inflammatory disorders across all ages.

Metrics:

- Define disease mechanisms (through genomics and multiomics) in common and rare inflammatory disorders and identify novel targeted therapies for these conditions;
- Develop and/or lead clinical trials of novel therapies including trials for first in human genetic therapies for rare inflammatory disorders;
- Enhance funding support for these studies through both industrial partnerships and research grants.

3. To develop new and improved gene and cell therapies for genetic disorders, haematological malignancies and transplantation and explore the mechanisms that underlie malignant transformation and transplant rejection.

Metrics:

- Secure high levels of funding for translational research on cell and gene therapies for genetic disorders, haematological malignancies and transplantation through grants and industry collaboration;
- Deliver both academic and industry-sponsored novel first in man clinical studies to treat children with these disorders;
- Develop the next generation of PIs in this field by increasing the number of successful fellowship applications for early career researchers;
- Lead a national strategy for improving the translational pathway for new gene therapies in ultra-rare diseases and ensure equitable access to clinical trials and licensed products.



Population, Policy and Practice Research and Teaching Department

The overarching goal of the Population, Policy and Practice Department (PPP) is to conduct excellent, impactful and responsive research on child and adolescent health and wellbeing and deliver world-class teaching and training in a vibrant, inclusive and supportive environment, with an emphasis on accelerating translation of research findings into practice and policy.

Key achievements over the last five years:

Over the last five years, PPP undertook key national and international research in relation to the COVID-19 pandemic for children, young people and families, including on Long COVID-19, mental health, homelessness, vaccination, and health services use. PPP has driven change in policy, public health and clinical practice by providing evidence in areas as diverse as digital marketing of unhealthy foods, childhood vaccinations, treating chronic/complex conditions including cystic fibrosis, HIV, and inflammatory eye disease, measuring child growth, and low-intensity psychological interventions in paediatric hospitals. Funding successes include renewal of our two NIHR Policy Research Units on Children and Families and on Healthy Weight, an NIHR Global Research Professorship, two UKRI Future Leader Fellowships, four NIHR senior fellowships and a Wellcome Trust Investigator Award.

PPP extended its data science expertise, including establishment of the ECHILD (Education and Child Health Insights from Linked Data) database and strengthening links with the NIHR BRC at GOSH through its new Data Science theme. PPP's academic impact (within UCL's UoA2 in REF2021) was rated as internationally and nationally leading. PPP academics received new/renewed NIHR Senior Investigator Awards and Academy of Medical Sciences Fellowships, and staff continued to provide expertise to various Government departments/agencies, in the UK and elsewhere, the WHO and research funders. We also host the newly appointed Chair of Palliative Care, ensuring we make a difference to all those with chronic disease.



Priorities for the next five years:

1. To generate and apply new knowledge on determinants of child and adolescent health and wellbeing to advance prevention, diagnosis and treatment of childhood disorders and disabilities, and to narrow health inequalities across the inter-generational life course

Metrics

- Translation of our population discovery science findings into evidence-based practice and policies as evidenced by, for example, citations in national and international clinical guidelines and policy briefs from national and international public bodies;
- Increased funding and partnerships for interdisciplinary child health research to address the complex pathways leading to health inequalities;
- Children and young people visible as key populations in the FPHS priority disease areas of obesity, mental health, infections and rare diseases as a result of our research, networking and advocacy.

2. To innovate, implement and share quantitative, qualitative and mixed methods for child population health and to create and share data resources, built on equitable engagement activities and open science practices

Metrics

- Design and delivery of research-informed teaching and training at UCL and externally, sharing innovations in methods; new partnerships to strengthen research capacity; retention of excellent mid-career researchers through support and mentoring to secure senior fellowship funding;
- Unique data resources for child population health research maintained and shared externally, such as national longitudinal linked health, education and social care administrative datasets;
- Involvement of underserved communities in our research, as drivers, directors and co-producers of research.

3. To improve implementation of effective health, education, and social interventions for child and adolescent health through research, education and advocacy

Metrics

- New local, national and global partnerships with key stakeholders and research users in child and adolescent health that maximise the impact of our research;
- Building implementation science research capacity through scientific exchange and training;
- Development of a system to track our research, education and advocacy impact.

UCL Child Health Open Research: <https://f1000research.com/UCLChildHealth>



REF2021

The Research Excellence Framework (REF) is carried out approximately every six to seven years to assess the quality of research across 157 UK universities and to share how this research benefits society both in the UK and globally. Research England manages the REF on behalf of all four UK higher education funding bodies.

The results are significant for benchmarking research excellence across UK institutions and are used by the four UK higher education funding bodies to inform the allocation of around £2billion of public investment in research every year. This Quality Related (QR) funding enables us to invest in the best people and facilities, to provide an environment in which early career researchers can thrive and work with our partners to address the biggest challenges facing humanity.



UCL highlights:

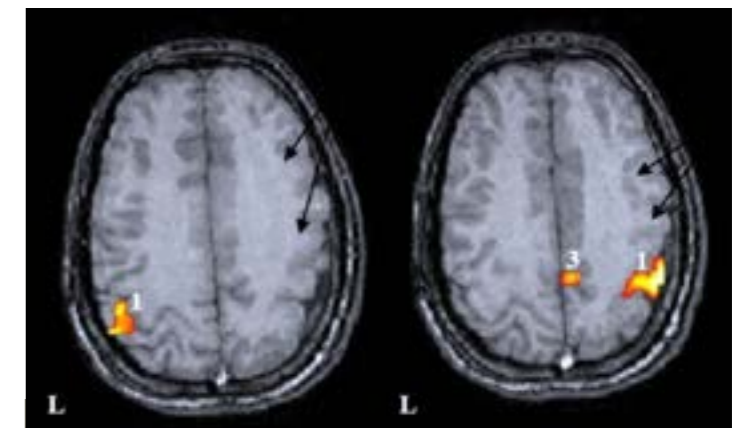
- UCL came top for research power in the main panels of 'medicine, health and life sciences' and 'social sciences'.
- UCL overall is No.2 in the UK for research power.
- 93% of UCL's research was graded 'world leading' and 'internationally excellent'.
- The Faculty of Population Health Sciences contributed a total of 17 innovative pieces of research which show insights our experts have developed to some of the greatest challenges facing the world and evidence of the benefit to humanity

Institute impact:

- For the 2021 submission, Institute staff were split across three units of assessments, UoA 1 (Clinical Medicine) with 100 people, UOA 2 (Public Health, Health Services and Primary Care) with 30 people, and 1 person submitted in UOA5 (Biosciences). There were 285 outputs included from UCL GOS ICH.
- UCL GOS ICH UOA1 scored 41.9% 4* for outputs, UOA2 scored 42.7% 4* for outputs.
- UOA1 UCL GOS ICH staff contributed 26.5% of the outputs (total outputs 860) and 27.9% of the FTE (total FTE 344.18)
- UOA2 UCL GOS ICH staff contributed 10.8% of the outputs (total outputs 520) and 12.4% of the FTE (total FTE).
- Both UOA1 and UOA2 scored 100% 4* for their environment statements.

Impact Case Studies from Institute researchers

[Transforming the lives of children with severe epilepsy](#)



[Children with neuromuscular diseases given new lease of life](#)



[Helping people with rare immune disorders lead long, healthy lives](#)



[Enabling safer, better and quicker pre-natal testing](#)



Education

Our mission is to maximise and advocate for the holistic health of all children, young people and the adults they will become, through world-class research, education and public engagement. UCL GOS ICH already has a reputation for its high calibre research and we aim to match that with the provision of world class education in child health.

We will build on the successful established educational offerings of UCL GOS ICH to ensure that the next generation of clinicians and scientists are equipped with the skills and knowledge necessary to inform policy and deliver services to influence and improve child health in a context of the challenge of changing patterns of children's health.

Building on the research strengths of the five Research and Education Departments, we will explore the potential for expanding our educational provision and increasing student numbers by offering general and specialist paediatric and child health related programmes and short courses which align with our research strengths.

We will provide an inclusive environment, which promotes equality of respect and opportunity for all members of our diverse community. Every student, regardless of their background, race, gender or religious beliefs will be treated with respect and encouraged to achieve their own personal milestones fully supported within an education encompassing Institute.

Education includes not only face-to-face classroom teaching but also PhD supervision and online and written provision of training materials. Where the contribution of Institute staff to education at UCL GOS ICH is measured, we include support activities such as MSc/BSc project supervision, personal tutoring for each student, peer-assessment of teaching colleagues, marking of assessments and similar peripheral roles.



Where we are now

We provide the following highly rated educational programmes:

Undergraduate

- Integrated BSc in Paediatrics and Child Health for UCL MBBS students in their third year.
- Leadership for and contributions to teaching on the MBBS, including the Child and Family Health with dermatology (Year 5) .
- Leadership for modules on the BSc Population Health.

Postgraduate taught

- MSc in Paediatrics and Child Health with pathways in:
 - Advanced Paediatrics
 - Community Child Health
 - Global Child Health
 - Molecular and Genomic Paediatrics
 - Intensive Care
- MSc Child and Adolescent Mental Health
- MSc Cell and Gene Therapy
- MSc Personalised Medicine and Novel Therapies
- MSc Infancy and Early Childhood Development
- MSc Paediatric Neuropsychology (Applied and Clinical)
- MSc Physiotherapy programme in four speciality areas with clinical and non-clinical pathways in
 - Cardiorespiratory
 - Paediatrics
 - Neurophysiotherapy
 - Musculoskeletal

Scholarships

The **Michelle Zalkin scholarship** is available for students with a proven interest in child protection applying for the MSc Paediatrics and Child Health. The award offers £10,000 towards fees.

The **Peter and Loek Wheeler Child Health Scholarship** provides one student from a low or middle income country with funding to cover tuition fees for the MSc Paediatrics and Child Health or the MSc Paediatrics and Child Health with Clinical Practice.

In addition, up to 10 bursaries of £3,000 each are available to MSc/MRes students and are awarded competitively.

Short and taster courses

- UCL GOS ICH hosts the UCL Centre for Applied Statistic Courses (CASC), as the largest provider of short (non-examined) statistics based courses within UCL and established primary statistical educator of non-statisticians in the UK.
- The physiotherapy group at UCL GOS ICH have an established programme of taster and non-examined participation in scheduled MSc module classes and an online Fundamentals of Paediatric Physiotherapy course consisting of 3 modules.
- Short courses are allied to other MSc modules. For example, as part of the MSc Paediatrics and Child Health immunisation module, a two day course 'Fundamentals of Immunisation' is run annually in collaboration with UK Health Security Agency. This attracts about 100 delegates mainly from Primary Care.

Postgraduate research

- MRes Child Health;
- MPhil/PhD programme offers postgraduate research degrees in a wide range of health-related fields that reflect the diverse expertise of the five Research and Teaching Departments. The UCL GOS ICH, supported by funds from its Charitable Investment Organisation (CIO) and the BRC, runs a nationally competitive PhD programme. Six to ten internally peer reviewed PhD projects are available per annum, awarded following national advert, application and interview. In addition, students have funded studentships from charities, including Wellcome Trust, research councils, NIHR and national governments. Some are self-funded – including members of staff (who are also able to access the Study Assistance Scheme).



Educational strengths

We have built a solid foundation on which to extend our educational activity.

- Our research is world leading and our educational offerings are already highly rated by students in their feedback;
- Our students benefit from our strong clinical links with Great Ormond Street Hospital, University College London Hospital (UCLH) and other major teaching hospitals;
- We have a body of highly committed staff already fully engaged who can act as ambassadors for our educational activities nationally and internationally;
- We have been able to use charitable funds to support and augment our educational activity, through scholarships.

Priorities for the next five years:

1. Deliver outstanding research informed education to address UK and global child health needs:

- Our educational offerings will be enhanced and developed informed by market research among current and potential students as well as employers to ensure we are catering to the changing needs of provision of healthcare, the population and students.

2. Foster an environment which values outstanding and innovative teaching through:

- Education forums established to offer focussed sessions on focused sessions on specific education topics for those already engaged in or wishing to become involved in education. Recent topics include: Climate Crisis and Sustainability in Healthcare and; Health Science Education;
- Department education leads (DELs) in each UCL GOS ICH research and teaching departments who are a key link between the departments and the senior education team and a source of information for department staff on both teaching opportunities for staff and support for short course development;
- UCL Arena Centre which offers professional development opportunities and support for all staff who teach or support students' learning, aimed at improving the quality of teaching across UCL and sharing and recognising best practice.;
- Involvement in educational activities, an important contribution to the life of the university that as such is recognised in promotion.

3. Strengthening links with GOSH and wider UCL:

- To ensure the successful continuing development of shared Education programmes such as MSc courses and short courses, we are drawing up contracts with Great Ormond Street Learning Academy.

4. Provide students with a transformative education experience:

- UCL GOS ICH is committed to working in partnership with its students to place them at the heart of decision making and help to improve their experience. Our Staff Student Consultative Committee (SSCC) plays a key role in enabling this and providing forums for student representatives and staff to work together;
- Working with professional bodies;
- Organising employability workshops;
- Providing career advice through UCL careers;
- Building our portfolio of scholarships to support students.

5. Support a thriving, inclusive, and global student community:

- Organising social events;
- Ensuring Equality Diversity and Inclusion is embedded and celebrated;
- Actively work on de-colonising of the curriculum.

6. Embed climate change and sustainability into the curriculum:

- Implementing UCL sustainability initiatives;
- Embedding research in our teaching activities.

Patient and Public Involvement and Engagement (PPIE)

We raise awareness and keep people informed about research at the Institute through our engagement programme that encourages conversations between patients, the public, staff and our researchers, working closely together with our NIHR GOSH BRC colleagues.

We run a diverse programme of events and activities including [work experience programmes](#) as well as annual activities to mark [Rare Disease Day](#), celebrate [International Clinical Trials Day](#) and taking part in research engagement [events](#) in the local community.

GOSH has a well-established Young Persons' Advisory Group (YPAG) for research who provide feedback and input to researchers to help them carry out research which is relevant to children and young people. GOSH YPAG is part of a national network of groups called [Generation R](#).

Find out more: [NIHR GOSH BRC | Involving patients and the public in our research](#)



GOSH YPAG representatives in the hospital

Working closely with our NHS partners; a powerful partnership with GOSH

Great Ormond Street Hospital (GOSH) has a legacy of leading cutting-edge, world first research with our closest research partner the UCL Great Ormond Street Institute of Child Health. We are proud to form the largest concentration of children's health research in Europe and our powerful partnership is only getting stronger.

We know that patients seen at hospitals that carry out research have better outcomes – which is why research and innovation partnerships like ours are crucial to GOSH's core purpose: to advance care for children and young people with rare or complex diseases.

Research carried out with the UCL GOS ICH is woven through our whole hospital, whether through the NIHR GOSH Biomedical Research Centre and Clinical Research Facility, or embedded in clinical research or discovery science from shared buildings like the Zayed Centre for Research.

These are enduring links.

This is why we are delighted to see that a recent analysis of our research partnership shows the strength of our published research and the power of collaboration. Around half of all GOSH papers are collaborations with UCL GOS ICH, working with other NHS trusts, national leading research organisation and global scientific collaborations.

As an Intelligent Research Hospital, we are focused on embedding research into everything we do and harnessing the power of our data. We know that our partnership with UCL GOS ICH is instrumental to this aim and welcome the new UCL GOS ICH Strategy, with a focus on areas of shared strength, like genomics and cancer, underpinned by robust core values and development of the research leaders of tomorrow.



Dr Matthew Shaw

CEO, Great Ormond Street Hospital for Children NHS Foundation Trust



Dr Kiki Syrad

Director of Research and Innovation, Great Ormond Street Hospital for Children NHS Foundation Trust

Child health partnership with Great Ormond Street Hospital Charity

A bibliographic analysis of research at UCL GOS ICH and GOSH for the period 2018-2022 by *Clarivate* (using web of science data¹) shows:

- A powerful, influential partnership that is of continually increasing impact and has high influence;
 - Collectively cited ~3x more than the global average
 - Consistently in the world top 10% of most cited research
- Strengths in paediatrics, neurology, neuroscience, genetics & heredity;
- Emerging areas in cancer and psychiatry;
- Commercial relevance – 85 patents citing GOSH/UCL GOS ICH papers;
- Overall, consistently have a higher share of highly cited papers compared to national and international comparators;

Our researchers also work with other Health Care Trusts including University College Hospital, the Whittington and Moorfields Eye Hospital.

¹ Research analysis was co-funded by UCL GOS ICH, GOSH, GOSH Charity and NIHR GOSH BRC. Performed by Clarivate using web science data. Citation is a good indicator of impacts and influence and how we benchmark with our comparators, accepting that there are limitations to the analysis. Data set consists of 10,558 publications over the period 2018-2022.

Thanks to research, we have seen major improvements in the lives of children being treated for rare or complex disease – but there remains an urgent need to invest more into child health research. As the UK’s largest charity dedicated to funding paediatric research, our ambition is to transform the lives of seriously ill children through research-led care. But we cannot achieve this alone.

Our partnership with GOSH and UCL Great Ormond Street Institute of Child Health offers an unrivalled opportunity to tackle some of the challenges faced by seriously ill children and their families and to make a global impact on child health. We have always aligned our strategies with these partners and are delighted to see such synergy between the ambitions and priorities of UCL GOS ICH and those of the Charity. We now plan an even greater focus on working together so that our combined efforts achieve greater impact than if we operated in silos.

As Europe’s largest centre for child-health research, UCL GOS ICH is home to our outstanding team who conduct world-leading research into childhood diseases. We understand that these researchers require the infrastructure to support their work, including state-of-the-art buildings such as the Zayed Centre for Rare Disease in Children, technology, clinical and non-clinical teams, and adequate funding, including support for new ideas. Thanks to the generous contributions of our supporters, and guided by our [2023-2028 research strategy](#), we are able to invest in these areas to work in partnership with UCL GOS ICH and supporting research to make significant advances in the diagnosis, treatment and care of seriously ill children.

Together we can do so much, and we are looking forward to working closely with the Institute to support the delivery of their strategy and maximise our investment together towards a common goal of transforming the lives of seriously ill children.



Dr Louise Parkes
Chief Executive, Great Ormond Street Hospital Charity



Zayed Centre for Research into Rare Disease in Children

Opened in 2019 this Centre is a partnership between University College London, Great Ormond Street Hospital NHS Foundation Trust and Great Ormond Street Hospital Charity and is a hub of research excellence enabling hundreds of scientists and clinicians to more accurately diagnose, treat and cure young people with rare diseases.

Rare diseases globally

Rare diseases consist of around 6,000 conditions, including several types of childhood cancer, cystic fibrosis and muscular dystrophy. Rare diseases represent a considerable health burden globally:

- 1 in 17 people are affected at some point in their lives;
- 75% of rare diseases affect children;
- of those, nearly one-third who are diagnosed will die before their fifth birthday.

Rare diseases are currently under-researched and many are undiagnosed and therefore untreated.

The centre utilises recent advances in science and technology to offer new hope to thousands of children and their families diagnosed with rare diseases. Bringing knowledge, technology and patients together in one place will accelerate the bench to bedside process of developing new treatments and cures for children with rare diseases both nationally and globally.

Working side by side in a purpose-built centre, with state-of-the-art equipment and facilities, will accelerate the discovery of new treatments and cures with:

- more trials and testing;
- a better understanding and reading of genetic codes;
- increased diagnostic capabilities;
- a greater number of new gene and cell therapies developed to clinical standards;
- the use of stem cells to regenerate organs and tissues.



NIHR GOSH Biomedical Research Centre (BRC)

Now in its 4th iteration (2022-2027) the NIHR GOSH Biomedical Research Centre (BRC) is based on a five year NIHR award to a partnership between the GOSH NHS Foundation Trust and the Great Ormond Street Institute of Child Health as part of UCL. UCL also houses two further BRCs, UCL Hospital BRC and Moorfields BRC. The core task of the BRCs is develop translational medicine from bench to bedside.

The GOSH BRC is the only BRC in the UK entirely focused on childhood diseases. In contrast to the 19 other BRCs the GOSH BRC has a primary focus on methodological innovation, not on diseases or disease groups, reflecting our view that innovation in translational medicine is largely driven by emerging methodologies. Our five central themes funded by the GOSH BRC award are: (i) Gene, Stem, and Cellular Therapies; (ii) Genomic Medicine; (iii) Accelerating Novel Therapies; (iv) Tissue Engineering and Regenerative Medicine; (v) Applied Child Health Informatics. These themes promote and develop new methodologies such as CRISPR-based and lentivirus-based or Adeno-associated-virus-based gene and cell therapies, emerging technologies for genomic analysis, RNA-based and small-molecule- or biologics-based therapies, bioengineering approaches to organ reconstruction and -replacement and advanced health informatics to tackle the mostly rare and frequently genetic diseases afflicting children. This ambitious approach is based on continuous development of the experimental platforms, supported by ongoing NIHR funding. This has led to important advances including 11 new drugs developed or co-developed by GOSH BRC PIs obtaining FDA/EMA approvals over the previous five year BRC award; the development of pioneering new treatments for haematological oncology; and the implementation of new genomic diagnostic services in the NHS. Data science has emerged as a powerful driver of innovation over the last decade, and the GOSH Trust was the first UK trust to be awarded HIMSS level-7 data maturity status. Data science now permeates and drives approaches across all fields from genomics to lifetime perspectives of therapeutic interventions – topics tackled within our Child Health Informatics theme.

This thematic focus is underpinned by a BRC Central Developmental Hub which supports our successful Career Development Academy (CDA), and our commercial, public and patient engagement, communications, and EDI activities. The CDA supports translational researchers from the undergraduate stage through to postgraduate fellowships and has a strong track record of shaping the career development future translational scientists. Over the current BRC term, the CDA will fund more than 20 clinical and non-clinical PhDs, 25 wider healthcare professional internships, 20 post-doctoral catalyst and intermediate fellowships, and 15 undergraduate internships. The GOSH BRC is works closely with the NIHR-funded GOSH Clinical Research Facility (CRF) to deliver experimental drugs through innovative trials, which are partly investigator- and partly industry-driven. These trials ensure cutting-edge medical innovation is accessible to our patients with currently 81 phase 1 and phase two clinical trials under way.

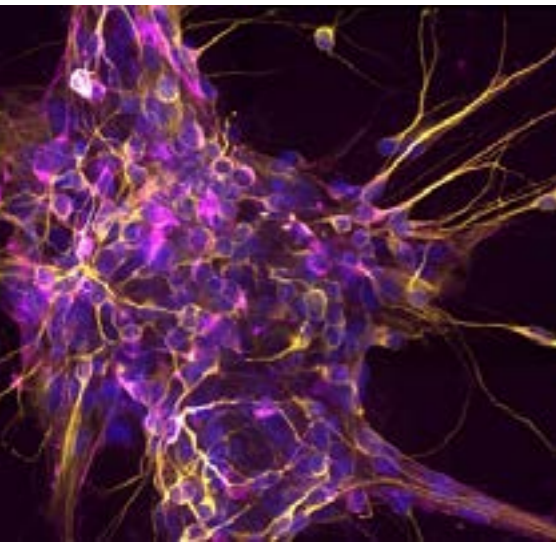
As the experimental medicine platform for both UCL GOS ICH and GOSH, the BRC is highly aligned with the strategies of its host institutions while remaining focused on its core mission of translational innovation.



Professor Thomas Voit, Director NIHR GOSH Biomedical Research Centre

Core Scientific Facilities and Research Centres

The UCL Great Ormond Street Institute of Child Health offers laboratory services to researchers based both inside and outside the organisation. We have invested in state of the art equipment to facilitate research of the highest quality. Our researchers are experts in their field and can advise on the most suitable investigation for your research.



Confocal Microscopy

The UCL GOS ICH imaging facility offers state of the art light microscopy equipment, and expert guidance in image acquisition and analysis. Training and advice on all aspects of microscopy are provided to enable researchers to acquire high quality images and reliable data. The facility has a range of confocal, spinning disk, and multiphoton systems for 3D imaging, a live cell imaging system, and systems for brightfield and fluorescence. In 2024 we will add a cleared tissue light sheet system for large sample 3D imaging. Courses are run periodically to teach staff image processing software, as part of the facilities philosophy of providing the broadest possible support for all imaging needs.

For more information:

<https://www.ucl.ac.uk/child-health/core-scientific-facilities-centres/confocal-microscopy>



Flow Cytometry Core Facility

The UCL GOS ICH flow cytometry facility provides state of the art instrumentation and expertise in single cell analysis and sorting. The facility offers hands-on training, experiment design, advice, and helps researchers generate reliable and consistent high-quality multidimensional data. The facility has five cell analysers and offers cell sorting on three highspeed cell sorters. The facility's eight flow cytometers and two analysis computers can accommodate users' cell analysis and cell sorting based on up to 30 colours simultaneously. Cell analysers can analyse cell suspensions from different devices including high throughput analysis from 96 or 384 well plates. Cell sorters can purify any cell populations of interest (up to six simultaneously) for further study and perform single cell cloning into multi-well plates.

For more information:

<https://www.ucl.ac.uk/child-health/core-scientific-facilities-centres/flow-cytometry-core-facility>

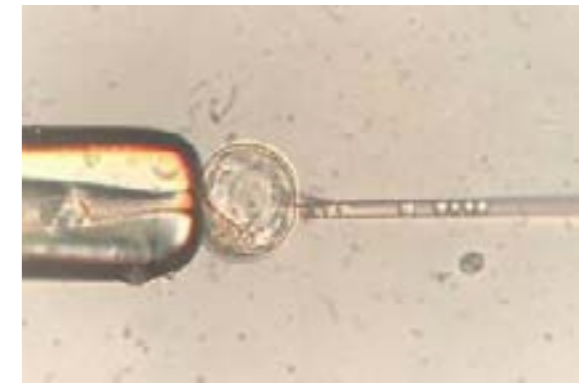
UCL Transgenic Service

The UCL Transgenic Service provides state of the art techniques and expertise in the creation, manipulation and delivery of genetically modified embryonic stem cell lines and mouse models.

The aim of the service is to help the scientific community with bespoke tools to better understand the molecular mechanism of mammalian genetics and development. The service focuses on applying a 3Rs approach to all of the projects undertaken. The UCL Transgenic Service facility provides services such as cryopreservation, rederivation/cryorecovery, ES cell injection and targeting, TALEN microinjection, CRISPR/Cas9 microinjection and ES cell targeting, ES derivation and morula aggregation. The services provided may be tailored to suit each customer's needs.

For more information:

www.ucl.ac.uk/child-health/core-scientific-facilities-centres/ucl-transgenic-service



Genomics Collaborative Research Facilities

Biological Mass Spectrometry Centre



The Translational Mass Spectrometry Research Group (TMSRG) is a bespoke research group that brings together cutting-edge technology with precision and stratified medicine for the benefit of patients. We use omic technology to help understand disease mechanisms and identify drug targets, but we also have the ability to translate any biomarker into a test to NHS or Industry to accredited standards. We specialise in using AI/Machine Learning with multiplexed bespoke panels of biomarkers for the diagnosis and monitoring of treatment of patients with neurodegenerative and complex diseases. Our group works closely with our BRC partners at GOSH, UCL Queen Square Institute of Neurology and UCL Institute of Cardiovascular Science to elucidate disease mechanisms involved in rare and neurodegenerative conditions. It is a unique, state of the art environment where clinical and non-clinical researchers work side-by-side using state-of-the-art omic technology in translational research.

For more information:

<https://www.ucl.ac.uk/child-health/research/genetics-and-genomic-medicine-programme/biological-mass-spectrometry-centre>

UCL Genomics



UCL Genomics is a collaborative research and education facility that provides expertise in the latest genomics technologies including state-of-the art DNA/RNA and single cell sequencing, and spatial transcriptomics as well as microarray profiling (genotyping, methylation and pharmacogenomics). The facility offers automated high-throughput DNA/RNA extractions and is equipped with a full suite of QC instrumentation. The facility supports a wide range of genomic projects at UCL as well as supporting our national and global collaborators. Highly experienced application specialists offer dedicated support at every stage from experimental design to data analysis. We welcome collaborations from all disciplines and can assist with grant applications. UCL Genomics is 100% cost recovery, providing consistently excellent value when compared to commercial and core academic alternatives.

For more information:

<https://www.ucl.ac.uk/child-health/research/genetics-and-genomic-medicine-programme/ucl-genomics>



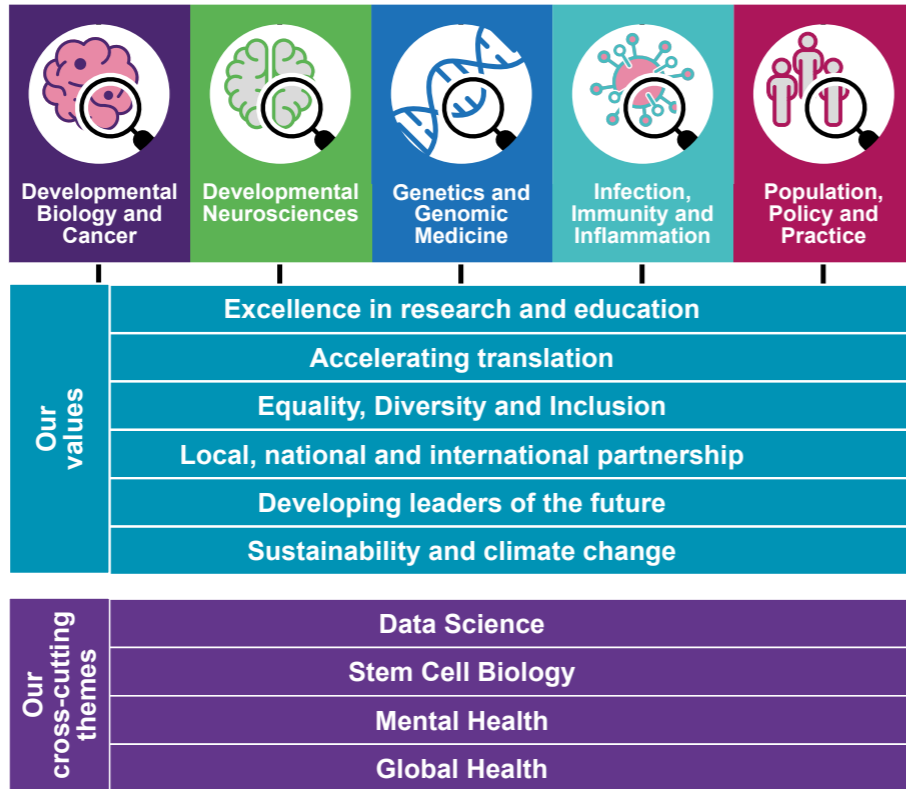
Institute Overview

Our vision:

Children first, always, everywhere

Our mission:

To maximise and advocate for the holistic health of all children, young people and the adults they will become, through world-class research, education and public engagement.



Education

On RESEARCH programmes

196 PhD and MD(Res) students

On TAUGHT programmes

346 MSc/PG Diploma/PG Certificate & iBSc students

Our taught education programmes

- MSc Cell and Gene Therapy
- MSc Personalised Medicine and Novel Therapies
- MSc Paediatrics and Child Health
- MSc Infancy & Early Childhood Development
- MSc Child and Adolescent Mental Health
- MRes Child Health
- MSc Advanced Physiotherapy
- MSc Physiotherapy Studies*
- MSc Applied Paediatric Neuropsychology
- PG Dip Clinical Paediatric Neuropsychology
- iBSc Medical Sciences with Paediatrics and Child Health

* children and adult specialist pathways available



ZAYED CENTRE FOR RESEARCH INTO RARE DISEASE IN CHILDREN

This Centre is a partnership between University College London (UCL), Great Ormond Street Hospital NHS Foundation Trust (GOSH) and Great Ormond Street Children's Charity (GOSH Charity) and

is a hub of research excellence enabling hundreds of scientists and clinicians to more accurately diagnose, treat and cure young people with rare diseases.

www.ucl.ac.uk/population-health-sciences/research/zayed-centre-for-research

Our impact: Peer reviewed publications¹: 906



Current active research projects²: 874 (£293.5M)



Our annual turnover 2022-23: £71.5M³



¹Period: July 2022-July 2023. ²Active Sponsored Research with end date after 31/7/2023. ³As at year end 31st July 2023.

Our staff numbers

13

Fellows of the Academy of Medical Sciences

155

Principal Investigators

86

Professors

Total staff: **570**

13

NIHR Senior Investigators

6

NIHR Research Professors

5

Academic Departments

Our Strategic Partners

Great Ormond Street Hospital NHS Foundation Trust (GOSH)

<https://www.gosh.nhs.uk>

Great Ormond Street Hospital Charity (GOSH Charity)

<https://www.gosh.org>

UCL Partners (UCLP)

<https://uclpartners.com>



www.ucl.ac.uk/child-health/about-us/equality-diversity-and-inclusion



www.ucl.ac.uk/child-health/research/ref-2021

www.ucl.ac.uk/child-health

<https://twitter.com/UCLchildhealth>

ich.comms@ucl.ac.uk

www.youtube.com/c/UCLChildHealth


Our dedicated Research Centres

- 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
- ARUK Centre for Adolescent Rheumatology
- Reuben's Centre for Paediatric Virology and Metagenomics
- NIHR Healthy Weight Policy Research Unit
- NIHR Children and Families' Policy Research Unit
- Louis Dundas Centre for Children's Palliative Care
- Centre for Paediatric Mental Health


Contact details:

UCL Great Ormond Street
Institute of Child Health
Faculty of Population Health Sciences
30 Guilford Street
London WC1N 1EH

 www.ucl.ac.uk/child-health

 [@UCLchildhealth](https://twitter.com/UCLchildhealth)

 www.youtube.com/UCLchildhealth

 (alumni): www.linkedin.com/groups/4448859

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