Centre for Research into Rare Disease in Children
Ciaran became the first child in the world to undergo a ground-breaking stem cell trachea transplant in 2010.
Introduction

The Centre for Research into Rare Disease in Children, due to open in 2018, will be a world-leading centre of excellence that will tackle some of the most challenging scientific questions, enabling our scientists and clinicians to more accurately diagnose, treat and cure children and young people with rare diseases.

Seventy-five per cent of rare diseases affect children and nearly one-third who are diagnosed will die before their fifth birthday. However, rare diseases are currently under-researched and many are undiagnosed and therefore untreated.

Rare diseases consist of around 6,000 conditions, such as childhood cancers, cystic fibrosis and muscular dystrophy. Taken together, rare diseases represent a considerable health burden globally as one in 17 people will be affected by a rare disease at some point in their lives. To this end, the Department of Health set out a UK Strategy for Rare Diseases in November 2013, aiming to ensure people living with a rare disease have the best care and treatment across the whole patient journey by 2020.

Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (UCL ICH) are uniquely placed to translate groundbreaking research into new treatments. Together they form the largest centre in Europe devoted to paediatric research and see one of the largest patient cohorts for rare disease in the world.

Our vision is to create a dedicated state-of-the-art research and medical building that will bring together our world-class scientific and clinical expertise and unique patient population, with centralised technologies and equipment. This will facilitate and promote the translation of rare disease research into tangible treatments and devices, consolidating the role of GOSH and UCL ICH as one of the world-leading centres for the development and manufacture of novel therapies for children with rare diseases.

The inclusion of a dedicated outpatients facility will not only provide an outstanding environment for patient care but will also promote an ethos of patient-focused research, facilitating the bench-to-bedside model.

Collaboration will be integral to the centre’s vision. The relatively small number of patients affected by each rare disease places great importance on national and international partnerships with other hospitals and academic and research institutions. Meanwhile, the research completed in the centre could unlock better understanding and treatments for more common and adult diseases on a worldwide scale. We will need to learn from the findings of others and distribute the knowledge gained so that the world can share our insights.

We want the centre to be a global resource. It’s not just about finding new cures and treatments for patients with rare diseases at GOSH; it’s about finding new cures and treatments that can be applied to children with rare diseases across the world.
Great Ormond Street Hospital (GOSH) opened its doors at 49 Great Ormond Street on Valentine’s Day 1852 with 10 beds. It was founded by Dr Charles West, who was driven to set up a dedicated children’s hospital in response to the high level of infant mortality in London. It was the first hospital in the English-speaking world dedicated solely to the treatment of children, recognising that children had specific medical needs separate from adults, that required a tailored approach.

For over 160 years, GOSH has been at the forefront of paediatric care, pioneering new and better ways to treat childhood disease and care for sick children. Dr West published the handbook, How to Nurse Sick Children, five years before Florence Nightingale’s Notes on Nursing and in 1878 opened the School of Nursing, which soon established itself as a national centre for paediatric nursing.

Dr Charles West encouraged clinical research in paediatrics and the hospital’s medical school opened in 1888, becoming widely known as an international centre of excellence in research and teaching for medical and surgical staff.

This attracted some of the best clinicians in the country, who identified and developed treatments for new and complex diseases. Thanks to the sustained and rigorous efforts of our staff, GOSH has built on this legacy until the present day, and continue to push forward in understanding the nature and causes of disease, pioneering new treatments and discovering better ways of working.

Some of our breakthroughs and firsts include:

- appointing the UK’s first consultant paediatric surgeon, Denis Browne, in 1928
- opening the first heart and lung unit in 1947
- opening the first leukaemia research unit in 1961
- pioneering the first heart and lung bypass machine for children in 1962 to help repair heart problems
- performing the first successful bone marrow transplant in Britain in 1979
- pioneering the non-surgical replacement of pulmonary heart valves in 2001
- launching the world’s first gene therapy trials for children born without functioning immune systems in 2001
- undertaking the world’s first stem cell supported tracheal transplant in a child in 2010
- becoming Europe’s first children’s hospital to offer a portable haemodialysis service in 2010
Great Ormond Street Hospital today

Great Ormond Street Hospital (GOSH) is in the top five paediatric hospitals in the world. Children are referred to us from other hospitals throughout the UK and the rest of the world and we have an international team of experts working across over 50 different clinical specialties, the widest range of any children’s hospital in the UK. We specialise in the care of children with rare diseases and host the largest number (19) of nationally commissioned services in the UK.

This puts the new Centre for Research into Rare Disease in Children in a strong position to be a leader in the field of rare disease research, because we are likely to see more children with rare conditions and a wider diversity than any other hospital in the world.

Each year, GOSH has more than 240,000 patient visits and sees children ranging in age from newborn babies to 18-year-olds. The number of patients who visit us is set to increase by up to 20 per cent over the next five years.

GOSH places patient experience at the heart of our work and looks beyond a child’s medical condition to care for all his or her needs, as well as those of their family. It is anticipated that the new dedicated outpatients facility will improve the patient experience by better co-ordinating multiple appointments for children who are treated by a number of different specialists within the new centre. This joined up approach will minimise financial and emotional strain on families.

Education and training is a central pillar for the hospital and GOSH remains at the forefront of paediatric training in the UK, in particular for children’s nurses.

We are likely to see more children with rare conditions and a wider diversity than any other hospital in the world

We are a world and European leader in the following areas:

- world-leading centre for transplants and gene therapy
- pioneered non-surgical replacement of pulmonary heart valves
- one of the largest centres for children’s heart transplants in the world
- only dedicated paediatric tracheal service in Europe
- the leading centre in Europe for the management of conjoined twins
- one of the largest epilepsy surgery centres in Europe
- one of the largest centres in Europe for children with cancer aged one to 11
Great Ormond Street Hospital and the UCL Institute of Child Health – a powerful partnership

Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health (UCL ICH) is a unique partnership of academic scientists and clinicians. Together we form the largest concentration of paediatric research and clinical expertise in Europe, the only Biomedical Research Centre (BRC) specialising in child health in the UK and have one of the largest patient cohorts of children with rare diseases in the world.

Together our partnership has already delivered significant breakthroughs and achievements in the advancement of rare diseases in children, such as GOSgene. A leading-edge genetic facility, GOSgene uses DNA technology to pinpoint the faulty genes responsible for a child’s condition. So far 34 gene defects have been identified, and in over 50 per cent of these cases, the discovery of the faulty gene has not been previously reported in medical literature.

In 2014, GOSH and UCL ICH developed a new academic strategy to focus on five academic programmes with one cutting-edge theme: rare diseases. This will help to ensure that academic research and education interface seamlessly with clinical research and education. The BRC also established a new strategy for patient and public involvement and engagement in research.

Working side by side in a new purpose-built Centre for Research into Rare Disease in Children with state-of-the-art equipment and facilities will help to accelerate the discovery of new treatments and cures for rare diseases even further through:

- increased clinical trials capacity
- reading and understanding genetic codes more quickly
- increased diagnostic capabilities
- more new gene and cell therapies developed to clinical standards
- use of stem cells to regenerate organs and tissues

This unique partnership of academic scientists and clinicians forms the largest concentration of paediatric research and clinical expertise in Europe.

About the UCL Institute of Child Health

The UCL Institute of Child Health (UCL ICH) has been the hospital’s official research partner since it was first founded in 1945 in four cubicles of an old ward in Great Ormond Street Hospital (GOSH).

In 1966, UCL ICH moved from GOSH to Guilford Street – where it is still based today – and was opened by Her Majesty the Queen. The mission of UCL ICH is to improve the health and well-being of children through world-class research, education and public engagement.

Today, UCL ICH is a key part of University College London’s School of Life and Medical Sciences (UCL).

The School of Life and Medical Sciences is the UK’s strongest medical research grouping with 70 – 80 per cent of research graded as internationally excellent or world-leading*. It attracted £234 million of grant income in 2013/14 and accounts for over 60% of UCL’s research and academic staff.

UCL was ranked fifth in the recent QS World University rankings for 2014/15 and has produced 29 Nobel prize winners, including the 2014 winner for Physiology or Medicine John O’Keefe.

*(Research Power Score is graded by the Research Assessment Exercise (RAE), undertaken every five years on behalf of the four UK higher education funding bodies to evaluate the quality of research undertaken by British higher education institutions, by a subject specialist peer review panel. The next RAE assessment is due to be published in December 2014).
Global reach

Our vision is that through the work undertaken at Great Ormond Street Hospital, more sick children across the world can get better and have a higher quality of life than is possible today.

This chimes with our vision for the new Centre for Research into Rare Disease in Children in which we aim to create a global resource to find new cures and treatments for children with rare diseases, which can be promoted and applied to children with rare diseases across the world.

We attract a significant international patient cohort as children are referred to us by other hospitals across the world that do not have the expertise or specialist care required to care for them.

Our reputation as an international centre of excellence in research and teaching for medical and nursing staff has attracted experts from across the world. These experts have in turn contributed to our heritage of ground-breaking discoveries and training and thus helped to perpetuate our world-leading reputation and enabled us to recruit and educate on an international platform.

We aim to consistently deliver clinical outcomes that place us among the top five children’s hospitals in the world, but we recognise that it will take time for all our specialties to achieve this goal.

We have developed a programme for identifying key outcomes for each of the specialties, and are developing mechanisms to publish our outcomes on our website in real time. To facilitate this process we have established relationships with the other leading children’s hospitals in the world and made agreements about data sharing and benchmarking.

Working with our academic partner, the UCL Institute of Child Health, we are one of the largest centres for research into childhood illness in the world. We therefore collaborate with an extensive network of international academic and research institutions in the pursuit of discovering new cures and breakthroughs for children with rare diseases.

We attract a significant international patient cohort as children are referred to us by other hospitals across the world that do not have the expertise or specialist care required to care for them.

International patient cohort

Over the last three years from 2011–12 to 2013–14 we had over 90,000 patient visits (inpatient admissions or outpatient appointments) to our International and Private Patients division and saw 33,000 patients from 109 countries.

International benchmarking for improvement

We work with international partners to ensure that we are competing on a global scale as a world-class institute compared with our peers. It allows us to establish common measures so that we can learn from each other to improve.

Our international benchmarking has ensured we are part of important initiatives such as the International Consortium for Health Outcomes Measurement (ICHOM), which is systematically developing outcome measures by condition and has established an international collaboration between 16 paediatric centres of excellence to agree common outcome measures.

In partnership with Texas Children’s Hospital, GOSH recommended cleft lip and palate as the first paediatric condition for ICHOM to work on.

We also wish to ensure that we record and report effectively those outcomes reported by patients across the world to measure health outcomes from a global patient’s perspective.
International expertise and training

**International staff expertise**

In 2013–14, 21 per cent of our staff nationalities were non-UK and represented the following continents: Europe (non-UK), Africa, Asia, Oceania, Gulf State and North and South America.

We actively recruit overseas and have a specialist commercial partner to target high-quality nurses particularly from Ireland and Portugal. The initiative was initially focused on staff for Intensive Care Unit areas, but has proved so successful that staff have been recruited to specialisms in medicine, and to our International and Private Patients division.

Within the new Centre for Research into Rare Disease in Children, we will have high-calibre, expert children’s nurses to meet the complex needs of the children and young people we treat with rare diseases.

Great Ormond Street Hospital (GOSH) develops clinical and non-clinical education programmes to support the development of children’s services on an international level.

- Working with London South Bank University (LSBU), we are a significant trainer of children’s healthcare specialists and we train more children’s nurses than any other hospital.
- GOSH plays a leading role in training paediatric doctors, including many of the children’s cardiac surgeons currently practising in the United States of America.
- We have engaged with health ministries in the Gulf in partnership with the Dubai Health Authority and the Kuwaiti Ministry of Health, to enhance paediatric services in the government hospitals.

**Examples of our international training programmes**

Enhancing services in Kuwait for children with cancer and haematological disorders

In 2010 we developed a three-year partnership programme with the Kuwaiti Government to provide clinical advice on paediatric patient management, education, training and service consultancy on oncology.

Combined with site visits to Great Ormond Street Hospital (GOSH), the programme has meant that local skills are developed and children are treated safely in their own environment rather than having to travel overseas.

**Assessment and management of the sick child in Dubai**

We have worked with the Dubai Health Authority to deliver a programme of foundation level competencies for nurses.

A core group of experts from GOSH trained 90 nurses in Dubai’s Government hospitals to develop skills in a number of areas such as the psychosocial needs of children and young people admitted to hospital, assessment and management of children and young people’s pain, drug calculations and the safe administration of medication.

**Dubai visiting programme**

The hospital works with the Dubai Health Authority and the Ministry of Health in the United Arab Emirates (UAE), where specialists, consultants, dietitians, clinical nurse specialists and professionals allied to medicine from the hospital visit the UAE to see children in the comfort of their own environment, rather than have families travel to London.

Through regular visits, our consultants develop direct relationships with the doctors looking after the children in their own country.

Through regular visits, consultants develop direct relationships with the doctors looking after the children in their own country and communication between treating doctors is maintained during the entire course of the child’s treatment.
International research collaborations

Working with our academic partner, the UCL Institute of Child Health (UCL ICH), we are one of the largest centres for research into childhood illness in the world.

During 2013, Thomson Reuters (Evidence) were commissioned to undertake a bibliometric analysis of UCL ICH and Great Ormond Street Hospital (GOSH) publications spanning 2008–12.

This analysis showed that, compared to the top paediatric research organisations in the world, UCL ICH and GOSH together rank third on citation impact, fourth on the percentage of papers that were highly cited and fifth on the numbers of original research publications. In addition, the citation impact of our publications was twice the world average.

Collaboration will be an integral component to the success of the vision for the Centre for Research into Rare Disease in Children

The results demonstrate the quality and global importance of the research taking place at UCL ICH and GOSH.

Collaboration will be an integral component to the success of the vision for the Centre for Research into Rare Disease in Children.

The relatively small number of patients affected by each rare disease places great importance on national and international partnerships with other hospitals and academic and research institutions.

The research completed in the centre could unlock invaluable treatments for more common and adult diseases on a worldwide scale.

Lung Function in Growth and Aging – a united worldwide approach

Children’s lung function differs based on race, ethnicity and other factors. The aim of the project is to understand what is considered ‘normal’ in different ethnic populations. This will improve the diagnosis of children with respiratory diseases by providing a more accurate set of lung function charts based on race and ethnicity.

It involves the European Respiratory Society, the American Thoracic Society and Academy of Chest Physicians, the Asian Pacific Society of Respirology and the joint Australian and New Zealand Society of Respiratory Scientists and Thoracic Society.

The lung function charts have been endorsed by all partners, and are now being implemented into commercially available lung function equipment to encourage widespread clinical uptake and use by professional bodies such as the Health Survey of England and the UK Cystic Fibrosis Registry.

“It has been known for about 150 years that there are ethnic differences in lung function, but never before has there been a multi-ethnic study of this size.”

Professor Janet Stocks, who chaired the Global Lung Function Initiative and was the Professor of Respiratory Physiology at the UCL Institute of Child Health (until December 2013) said: “It has been known for about 150 years that there are ethnic differences in lung function, but never before has there been a multi-ethnic study of this size among primary school children to establish the true impact of these differences and how we might minimise them by taking more appropriate measurements of body physique.

“The new lung growth charts that we create will enable clinicians to assess children’s lungs more accurately, irrespective of ethnicity, and improve the diagnosis and treatment of conditions like sickle cell disease, asthma and cystic fibrosis.”

UCL ICH and GOSH researchers collaborated within 111 countries on research papers between 2008 and 2012. The top partner countries were the USA (19 per cent), Italy and Germany (10 per cent each), Netherlands (9 per cent), France (8 per cent) and Australia and Canada (6 per cent each).
World-first gene therapy trial for patient born without immune system

A team led by Bobby Gaspar, Professor of Paediatrics and Immunology at the UCL Institute of Child Health and Honorary Consultant in Paediatric Immunology at Great Ormond Street Hospital have devised a revolutionary new treatment called gene therapy for children born with severe combined immunodeficiency (SCID).

SCID is a condition which causes children to be born without a functioning immune system because of a defect in one of their genes. The disease affects about one in 250,000 children in the UK. This condition is sometimes referred to as ‘bubble baby’ disease because patients need to live in a sterile environment.

Treatment for SCID usually involves patients receiving a bone marrow transplant in order to boost the numbers of their white blood cells but without a compatible bone marrow donor this can be unsuccessful. If a donor can’t be found, some patients can now be given gene therapy where a faulty gene is replaced by a working gene, leading to their immune systems functioning at normal levels.

“Meet Nina

At five weeks old, Nina was diagnosed with SCID. She had multiple infections and was placed in isolation to stop her coming into contact with any more diseases. Nina needed a bone marrow transplant to treat her condition, but doctors were unable to find a match.

Professor Gaspar discussed gene therapy with Nina’s parents. The pioneering treatment would involve re-engineering Nina’s bone marrow to add a vital missing gene, which would reboot her defence systems and allow her to develop a fully functioning immune system.

Nina received the radical therapy in 2013. Six months after her treatment, she has shown very encouraging signs of recovering her immune system, and doctors have been able to stop most of her protective medication.

“The doctors and nurses at GOSH are amazing,” says Nina’s mum, Aga. “It has given us the hope that one day Nina will be able to lead a good quality of life.”
Why the focus on paediatric rare disease?

Rare diseases represent a considerable health burden, a fact that is attracting increasing concern both nationally and internationally. This is because, when taken together, these rare conditions are relatively common. More needs to be done to help those whose lives are affected by rare diseases, including much greater emphasis on medical research.

Most rare diseases are caused by a genetic defect, which means that children are born with the condition and will not get better by themselves. Rare diseases in children include childhood cancers, cystic fibrosis and muscular dystrophy. There are over 6,000 conditions in total. Individually, each disease affects less than one in 2,000 people. But as a group, they will affect one in 17 of us at some point in our lives.

Seventy-five per cent of rare diseases affect children, and nearly one-third will die before their fifth birthday.

The symptoms of rare diseases can often be very serious, making patients very sick or causing disabilities that impact on how long they will live and their quality of life.

Rare diseases are complex and not well understood in comparison to other illnesses. This means that those with rare diseases often experience a delay in getting diagnosed and have limited options for treatment.

However, recent advances in science and technology offer new hope. Genomics (the science of genetic mapping and DNA sequencing) is helping scientists to identify the genetic basis of rare diseases. New treatments such as stem cell therapies allow us to offer patients the chance of a longer and fuller life.

Bringing knowledge, technology and patients together in the Centre for Research into Rare Disease in Children will speed up the bench-to-bedside process of developing new treatments.

Most importantly, the centre will bring us closer to breakthroughs and cures for rare diseases with each passing day.

Sources
1 Orphanet
2 Official Journal of European Commission

In the UK alone that equates to approximately 3.5 million people.

Only a quarter of rare diseases have had their molecular basis defined, meaning many risk being undiagnosed and therefore untreated.

1 in 17 people will suffer from a rare disease at some point in their lives.

THERE ARE AT LEAST

6,000

RARE DISEASES

Many rare diseases (approximately 80%) are of genetic origin.

Seventy-five per cent of rare diseases affect children.

30% of rare disease patients die before their fifth birthday.
How the new centre will support our work

Working together, Great Ormond Street Hospital (GOSH) and the UCL Institute of Child Health are the largest centre for paediatric research in Europe and one of the largest worldwide. This is one of the only centres in the world with the specialist expertise and diverse patient population needed to discover cures for rare diseases.

Discovering cures for rare diseases in children is particularly challenging, as each condition affects comparatively small numbers of patients, so it’s difficult for researchers to obtain enough patients to study each disease thoroughly. We urgently need better facilities and more space to allow us to help more patients, develop new treatments and continue to share our discoveries with others.

The new Centre for Research into Rare Disease in Children will support scientists, clinicians, engineers and other experts to pool their knowledge and access the patients they need in order to:

• understand the genetic basis of rare diseases more rapidly
• develop gene and cell therapies to treat genetic conditions
• use stem cells to regenerate organs or tissues
• manufacture new medical devices

The new outpatients facility within the centre will enable us to treat up to 20 per cent more patients which will help us to cope with ever-increasing demand.

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Viewpoints

Two of our leaders who carry out clinical work and research explain how the centre will support their work.

“The centre will be the first of its kind to bring clinicians, patients and scientists together, to translate pioneering research techniques into hope for children worldwide who have rare diseases.”

“I have led a number of successful trials that saw gene therapy transform the outcomes of children with severe combined immunodeficiency – a rare immune disorder. My hope is that this new centre will allow us to go even further and develop gene therapy as well as cell and stem cell therapies as a standard treatment for many more rare conditions.”

Professor Bobby Gaspar, Paediatric Consultant Immunologist at GOSH and Director-Designate for the Centre for Research into Rare Disease in Children.

“The really exciting thing about the new building is the possibility of bringing everyone into the same working environment. As a clinician, I need to explore my options for treating a patient with experts who can help me develop them. For example, specialist computer modelling can help test and refine new devices before we use them in patients.”

“Many different professionals coming together under one roof allows us to turn innovative thinking into practical solutions. It also gives us a manufacturing capacity to develop those solutions at scale so we can help more and more patients.”

Professor Andrew Taylor, Divisional Director of the Cardio-respiratory Service at GOSH and Professor of Cardiovascular Imaging at the UCL Institute of Cardiovascular Sciences.

Professor Taylor has developed a multi-disciplinary approach to treating his patients’ conditions. Scientists, engineers and even mathematicians work with the cardiologists to pool their knowledge and develop devices that are unique to a patient’s anatomy.
World-leading researchers and clinicians

The researchers who will be based in the Centre for Research into Rare Disease in Children are international leaders in their field, leading pioneering programmes of research.

Many are clinically trained and will be based across the hospital campus, including the new centre, providing important insights into the clinical problems facing children with rare diseases.

The interface between basic and clinical scientists is core to the philosophy of the centre and will inspire a truly translational and patient-focused approach to research.

The research undertaken at the centre will be across seven main areas, covering a range of rare diseases and with a collaborative approach to research.

The themes and the principal investigators that will lead those areas are outlined here.
Locating the Centre for Research into Rare Disease in Children

The Great Ormond Street Hospital campus is located in central London within the Bloomsbury Conservation Area. It is bordered by Coram’s Fields, historic buildings and other medical and academic centres and close to excellent transport links.

The existing building

The existing building at 20 Guilford Street is an office block built in the 1960s. Previously used as a computer centre by the University of London, it became surplus to requirements and has been disused for some time.

The site was purchased by Great Ormond Street Hospital Children’s Charity in 2010 with the intention of establishing the Centre for Research into Rare Disease in Children.

Site location

The site for the proposed new building is located on Guilford Street, directly opposite Coram’s Fields, a Grade II-listed public space.

The new building will be adjacent to the existing hospital and UCL Institute of Child Health, extending our campus.

The site sits within three sub-areas of the Bloomsbury Conservation Area and there are a number of listed buildings in the immediate vicinity.

Coram’s Fields: a heritage of caring for children

Coram’s Fields are situated on the site of the former Foundling Hospital, established in 1739. The new centre would continue a long history of the use of this area for activities to support children’s welfare.

The Foundling Hospital was founded by Captain Thomas Coram and provided a home and education for orphaned children. The institution was described as “the most imposing single monument erected by 18th century benevolence”, and caught the public imagination to become London’s most popular charity during the 18th century. It was relocated outside of London in the 1920s.

Campaigning and fundraising by local residents and a donation from the Harmsworth family of newspaper proprietors led to the creation of the current park, which opened in 1936. Today, Coram’s Fields provides much-needed space and facilities for our city’s children and young people.
The centre creates a fantastic opportunity to create a dynamic integrated space which enables scientists and doctors to work side by side to discover and deliver new and better treatments for children with rare diseases. Our award-winning architects, Stanton Williams, have been working closely with our staff and experts in construction, medicine and research. They aim to create a high-quality building with outstanding research and clinical facilities to promote interaction between the various disciplines, support exceptional patient care and deliver ground-breaking research.

The building will contain:

- a range of research laboratories and other hi-tech facilities such as a flow cytometry suite facility (for counting and analysis of cells)
- a Good Manufacturing Practice facility to manufacture specialist products for novel therapies
- a much-needed new outpatients clinic with facilities suitable for medical staff, patients and their families
- research write-up areas and meeting rooms to allow our scientists and medical teams to progress their work

We also want the building to:

- relate sensitively to the site and its context within a conservation area
- have a human, friendly scale with appropriate use of high-quality materials
- symbolise excellence and provide a world-class patient experience
- engage patients and the public in the important work taking place inside
The design of the building needs to fit sensitively into the architecture of the local area whilst maximising the amount of space for the ground-breaking research and clinical facilities.

In response to guidance received during the planning application, the proposed building responds to the formal layout that once characterised Coram’s Fields by reinstating the former symmetrical entrance to Guilford Place.

Coram’s Fields is framed by five and six storey buildings, mostly institutional and civic in function, including:

- the UCL Institute of Child Health to the south
- the International Hall on Lansdowne Terrace (UCL student hall), the Brunswick Centre and the UCL School of Pharmacy to the west
- Goodenough College and the residential blocks on Mecklenburgh Square to the east

The proposed height and massing (shape) of the new building will form part of this urban setting along Guilford Street. The building will be six storeys high along Guilford Street, with two set-back levels to reduce its visual impact.

At the rear, the building reduces to four storeys along Millman Street and two storeys at the south-west corner of Millman Mews to minimise the impact on the residential buildings to the south of the site.
Building exterior: design features

The building is being designed to ensure that it is sensitive to the site and local context. It will be clad in high-quality materials, which is in keeping with the surrounding conservation area.

From Guilford Street, the identity of the building will be given a ‘civic’ public expression. Views into the building and the principal laboratory spaces will be created from the pavement.

By using brickwork and smaller windows, the appearance of the building along Millman Street and Millman Mews will be more residential in nature than the front of the building.
Inside the building: key features

A key feature of the building is transparency, to bring the work that takes place in the Centre for Research into Rare Disease in Children to the outside world.

Main entrance

Views from Guilford Street into the double-height laboratory below are enhanced by large areas of glazing and a set-back ground floor façade, revealing the activities within the building.

The building entrance is conceived as a ‘bridge’ over the laboratory space, connecting Guilford Street with the foyer areas.

Interior

The bridge takes visitors into a light-filled atrium, which is the central heart of the building. The interior is designed to maximise daylight access and views to the exterior.

Outpatients

The outpatient space is expected to provide 21 consultation rooms, eight clinical investigation rooms and other facilities set over two floors.

The ground floor waiting room is set back from Guilford Street and this quiet, private space includes a small café and external terrace.

A two-storey atrium will flood both outpatient floors with natural light and the generously proportioned upstairs waiting area offers views across Coram’s Fields.
Laboratories and manufacturing facilities

The building will house two large laboratories on the lower ground floor, one of which will be visible from Guilford Street.

Other high-tech facilities and workspace will include tissue culture laboratories, a cardiology suite, a flow cytometry suite facility (for counting and analysis of cells), freezers and liquid nitrogen stores.

The Good Manufacturing Practice (GMP) facility

A Good Manufacturing Practice (GMP) facility on the fourth floor will include laboratories to manufacture specialist products for novel therapies.

It will also provide specially equipped and licenced facilities so we can safely manufacture cell and gene therapies.

This will allow us to:

• further develop our regenerative medicine programme for children with absent or damaged tissues (such as windpipes, ears or noses)
• provide gene therapies that result in a permanent correction for children with genetic diseases who have limited or no other options for treatment
• develop these treatments at Great Ormond Street Hospital along with partner organisations to ensure effective treatments for children wherever they are

Sustainability and the environment

The building has been designed to be as sustainable and efficient as possible.

Climate change is a significant threat to child health and Great Ormond Street Hospital is serious about creating sustainable buildings.

The building will achieve a BREEAM ‘excellent’ rating, placing it within the top 10 per cent of new-build non-domestic properties in the UK.

We propose the following sustainability and biodiversity measures:

• a high-performance building ‘envelope’, which preserves heat and excludes damp
• low energy and motion-responsive lighting
• water conservation systems and rain water recycling
• responsibly sourced, sustainable and recycled construction materials
• combined heat and power (CHP), generating electricity while also using the waste heat from hot water and space heating purposes
• solar panels at roof level below the proposed parapet to supplement electrical energy requirements
• the Design team will be working with an ecologist to ensure that the building enhances the ecological value of the site, including local plant, insect and animal life
• a green roof is also proposed to enhance ecological value and biodiversity
Art strategy

In the Centre for Research into Rare Disease in Children, we aim to integrate artworks into the structure of the building and complement the building, way-finding and interior design concepts.

We will ensure that artworks engage the public in the identity of the centre, use art to explore biomedical themes and promote wider knowledge and understanding of research into rare diseases and inspire interaction and provide opportunities for play.

The arts programme at Great Ormond Street Hospital (GOSH) is lead by a team called GO Create! They are tasked with helping to create a healing environment and a positive hospital experience for patients, visitors and staff through the creation of relaxing, engaging and child-friendly surroundings.

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Art has long played a positive role in the quality of care for patients in the health service, but its association first started at The Foundling Hospital, which was formerly built on the grounds for the new Centre for Research into Rare Disease in Children. William Hogarth, who was a Founding Hospital Governor, decorated the walls of the hospital with works of art donated by contemporary British artists. In doing so, he also provided the first contemporary gallery of British art.

Key aims of the art strategy for the Centre for Research into Rare Disease in Children:

- Commission 2D, 3D, digital and interactive artworks that appeal to all users of the centre, particularly children and families
- Run an ongoing programme of participatory projects with children and young people and their families
- "Interactive fish pool" commissioned by GO Create! for the main hospital reception
- "The Nature Trail" by Jason Bruges Studio was commissioned by GO Create! in 2012 as an interactive artwork to help create a calming journey to the operating theatre
- Willow (far left) and Summer (left) taking part in a participatory workshop for the Big Draw that was held during GOSH Family Arts Week 2014
- Photograph by James Medcraft
- "The Nature Trail" by Jason Bruges Studio was commissioned by GO Create! in 2012 as an interactive artwork to help create a calming journey to the operating theatre
Consultation process and feedback

Maintaining good relationships with the local community is essential to the success of the new Centre for Research into Rare Disease in Children.

In June and July 2014, Great Ormond Street Hospital (GOSH) offered all stakeholders in the centre an opportunity to view our building plans at an early stage and influence them as they developed, prior to submitting our application to the local authority for planning permission.

We then held a public exhibition in September 2014 to provide information on the feedback we received during our consultation and an update on our design process.

The process of engaging stakeholders was held in accordance with the Localism Act, which came into force in April 2012 and placed great emphasis on the requirement for involvement and consultation of local communities, and alongside engagement with the planning authority.

Consultation with planning officials prior to consulting with the public and making a formal submission allowed the design team to anticipate the type of concerns that members of the public may have and make adjustments to the design where appropriate. One such resulting key change was to reduce the height and massing by removing one story above ground and creating a second basement level.

The feedback we received from the public consultations indicated that the majority of stakeholders were supportive of the proposals. Many respondents expressed support for the proposed use of the building and associated benefits to child health, enthusiasm for its design features and recognition of its potential to improve and revitalise the area.

However, there were naturally some concerns expressed by local people about a major construction project and the increased movement of vehicles and people accessing the new building. Some respondents also raised concerns about the height of the building and potential impacts to the levels of daylight and sunlight received to their properties.

Maintaining good relationships with the local community is essential to the success of the new Centre for Research into Rare Disease in Children

Scheme changes resulting from the public consultation stage include:

- Identifying the best option for servicing the building, following residents’ concerns to minimise traffic in residential streets and following advice from planning officials to consider the viability of on-site servicing options
- Acknowledging residents’ feedback on the importance of revitalising the site and accommodating plants and trees, with plans to improve the streetscape and establish green roofs and landscaping
- Removing a large portion of glazing from early designs and reducing the width of some windows to provide a greater sense of privacy within the building and for neighbours

An ongoing engagement plan is in place for the following key stakeholders:

- Local residents through the Redevelopment Residents’ Liaison Group
- Patients and families through the Foundation Trust Member’s communications programme and through consultation of the arts and engagement strategy
- Staff and internal stakeholders through regular staff briefings and newsletters
- Charity donors and supporters through a series of regular newsletters and magazines

“It sounds like a brilliant idea to incorporate research and treatment into a unified area and will benefit many children, their families and the staff involved.” Local resident

“I believe taking pressure off the existing outpatients service at GOSH will improve the service offered by the hospital tremendously.” Staff member

“Looks very impressive. Lots of good ideas. I like the fact that the labs are ‘on show’. Good for public engagement.” Visitor to a public exhibition
“My opinion is that it will be a beautiful building for a very good cause, pleased that it won’t be too tall. Will improve the whole area; need to keep dust and noise to a minimum during demolition and building.” Local resident

“For me it’s important children know it’s primarily a building for and about them. I hope the architects always consider the sights, sounds, textures and experience of the building from this perspective.”

Clinical staff member

“You have clearly gone to a lot of trouble to ensure that the new building is sympathetic to the Bloomsbury Conservation area and in tune with its neighbours. I particularly welcome your plans to enlarge the public realm along the Guilford Street frontage.”

Representative of the Marchmont Association (a Bloomsbury community group)