Cell and gene therapy in Cambridge, London and Oxford:

An invitation to collaborate

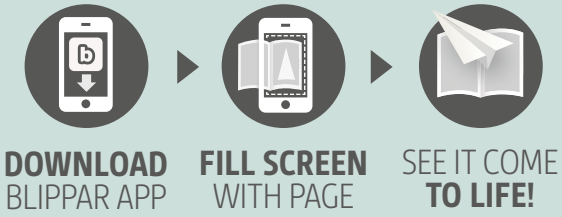
GREAT
BRITAIN & NORTHERN IRELAND

LONDON
& PARTNERS

MedCity

Contents

Download the Blippar app
with your Android, Apple or Windows phone, to access exclusive video content



Section 1

Section 2

Section 3

Foreword

The discovery of the double helix structure of DNA was a great moment in UK science and heralded the dawn of a new era in medicine. The transformation in how we understand and therefore treat disease since then has been staggering. Cell and gene therapies now offer a whole new model and approach for medical science.

Both the discovery of DNA and the more recent Nobel-winning development of induced pluripotent stem cells by Shinya Yamanaka in Kyoto and John Gurdon in Cambridge highlight something that for me is essential for scientific progress – collaboration and team-work. Great discoveries are not made in isolation; they are the product of teams with a variety of expertise, experience and perspectives. Increasingly those teams are international and cross-sector, bringing together the best people from industry and academia around the world.

Japan and the UK are natural partners in this field; we share similar demographic and health challenges, and a determination to move cell and gene therapy science out of the lab and to the patient as quickly as possible. The south east region of England is home to some of the best and most exciting research centres and companies in the world, which are working together to constantly push forward the boundaries of what we know and what we can do.

We are excited at the prospect of what more we can achieve together. MedCity is your guide to the region and is here to help you explore the options.

Paul Nurse
Director
Francis Crick Institute

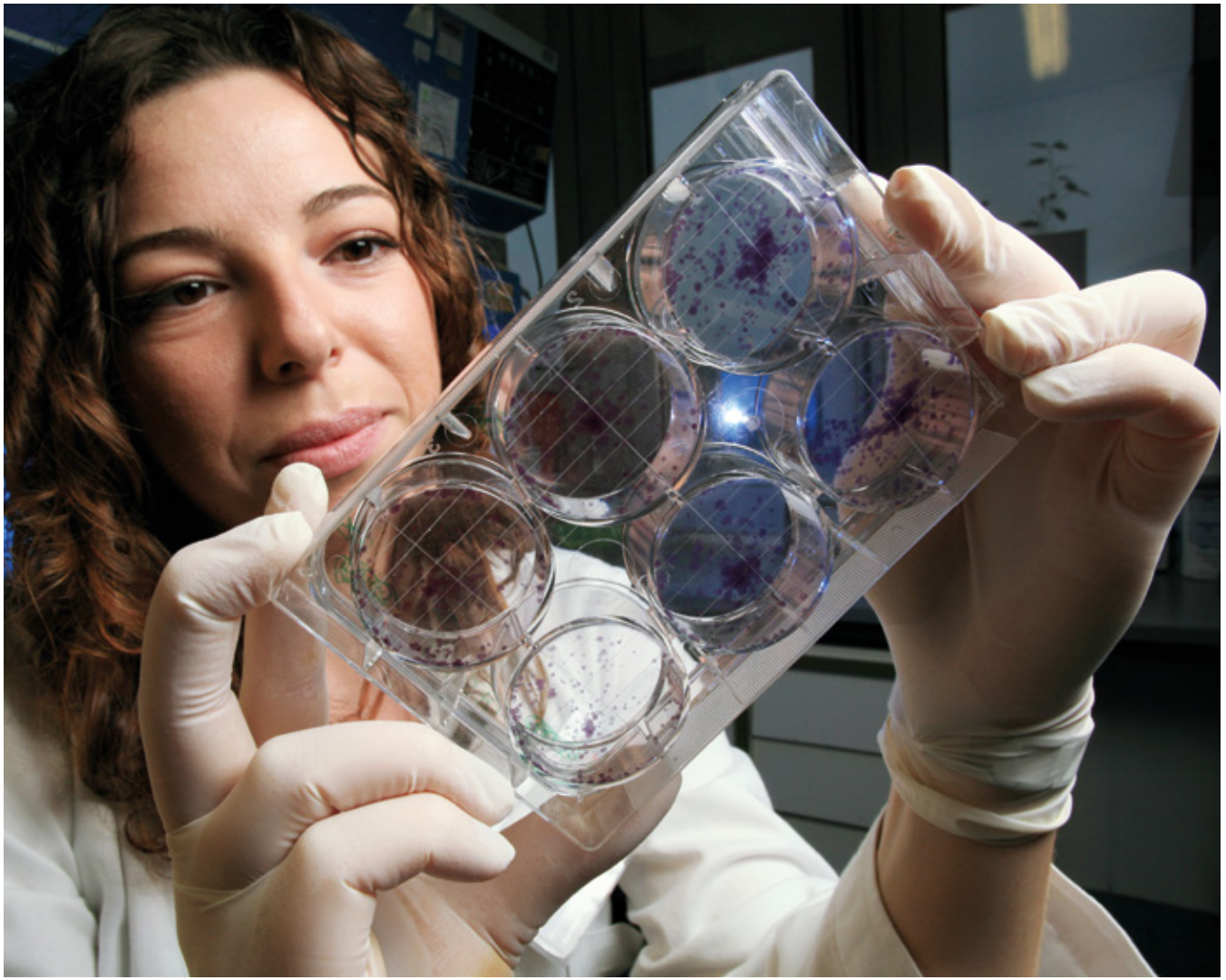
View of the Francis Crick Institute from St Pancras International © Justin Piperger Photography/Wadsworth3

The opportunity to collaborate with the golden triangle: Shared expertise, transformative outcomes

The golden triangle is the world-leading life sciences cluster of Cambridge, London, Oxford and the greater south east region of England. It comprises multiple award-winning research institutions, thousands of talented scientists, deep experience in clinical trials, and a thriving global business and science community. Key to the region's success has been its collaborative mindset – a drive to embrace and exchange ideas with scientists and professionals from around the world.

Cellular and gene therapies are at the heart of the UK government's long-term strategy for life sciences, and for the translation of great ideas into great partnerships, strong businesses, and, ultimately, ground-breaking therapies.

As such, the golden triangle eagerly invites and welcomes collaborations with colleagues from Japan in these fields. Japan's world-leading scientific pedigree, its award-winning achievements in cell and gene therapy, coupled with its long-term perspective and strong government support, make it a natural ally. So too do the nations' shared ambition to improve their populations' health, and grow globally competitive life sciences sectors.



Firm evidence already exists of the two countries' combined excellence in cell therapy, and of the fruits of shared ideas: in 2012, Professor Sir John Gurdon from the University of Cambridge and Kyoto University's Professor Shinya Yamanaka received a joint Nobel Prize for findings which together revolutionised our understanding of how cells and organisms develop. Professor Gurdon's discovery in 1962 that cell specialisation is reversible, and Professor Yamanaka's successful reprogramming, forty years later, of mature cells to become pluripotent stem cells, opened up entirely new research fields and myriad new opportunities to develop better diagnostic and therapeutic treatments.

As cell and gene therapy research and development flourishes in both countries, helped by strong government support and regulatory advances, the golden triangle's doors are wide open to Japan's researchers, businesses and investors. By working together, sharing expertise, developing new techniques and accessing de-risked investment, many more revolutionary findings and therapies will emerge, to the benefit of society as a whole.

"World-class research and publications, great clinical centres, talent, strong government support, industry-academic collaborations, regulatory expertise – all this has made the golden triangle a major melting pot for cell and gene therapy," Professor Chris Mason, Chair of Regenerative Medicine Bioprocessing at University College London.

UK Stem Cell Tool Kit: online tool helping those working with human stem cells in a clinical setting to more easily navigate regulatory requirements around research, development, manufacturing and approval.

sc-toolkit.ac.uk

Cell and gene therapy

Cell and gene therapy, and the technologies associated with and evolving from them, today underpin a large and growing portion of biomedical research.

Cancer immunotherapy, one of the hottest discovery efforts today, encompasses both cell and gene therapy. Chimeric antigen receptor (CAR) T-cell therapy is one approach that genetically reprogrammes patients' T-cells, ex vivo, to hunt down and kill cancer cells. These modified T-cells are then infused back into the patient, where they multiply.

Gene therapy – the transfer of genetic materials and the uptake of the gene into the appropriate cells of the body – has come of age to provide marketed treatments for people with rare, inherited genetic diseases.

Techniques such as RNA interference are enjoying a second coming, as targeting and delivery improves, while new gene editing tools such as those based on bacterial CRISPR-associated protein-9 nuclease (Cas 9) may eventually help address a wide range of medical needs. Indeed, "it's hard to imagine a biotech or pharma company which is not using gene and/or cell therapeutic techniques" in their quest for novel therapeutics, notes Eliot Forster, Executive Chair of MedCity and CEO, Immunocore. "Gene editing, harnessing the pluri-potential activities of cells and stem cell research are all central to a new era of drugs in which modified cells are the engines for therapy. We face a growing population of patients with neuro-degeneration, autoimmune and frailty disorders. I believe cell-based therapies will ultimately be able to solve much of this."

"Japan is a world-leader in cell therapy; the golden triangle represents a great place to partner, because of our manufacturing ability and the access we provide to patients for trials in the west."

Eliot Forster, Executive Chair of MedCity and CEO, Immunocore

Use Blippar to unlock a video

The ecosystem

There are many compelling reasons to choose the golden triangle of Cambridge, London, Oxford and the greater south east region of England, for cell and gene therapy partnerships. The region offers an unparalleled ecosystem that includes world-leading science, deep clinical and translational expertise and infrastructure, widespread funding opportunities, a thriving biopharmaceutical business community, and committed government support for life sciences. The Cell Therapy Catapult, based at Guy's Hospital London, was set up specifically to help translate the best cell therapy and regenerative medicine research into impactful new medicines and methodologies.

Research excellence

As Europe's leader in cell and gene therapy research and development, the golden triangle already draws scientists, clinicians, entrepreneurs, investors and other professionals from across the globe. The region is home to four of the world's top ten universities (Imperial College London, University of Cambridge, University of Oxford and University College London) and world-renowned research institutes including the Wellcome Trust, the Wellcome Trust Sanger Institute and the Institute of Cancer Research.

Eliot Forster emphasises that "the UK has a legislative advantage relative to the US when it comes to the manipulation of human cells. There are fewer restrictions and thus a broader ability within the academic and medical community to continue to do cell-based research."

Use Blippar to unlock a video

A clinical trial hotspot

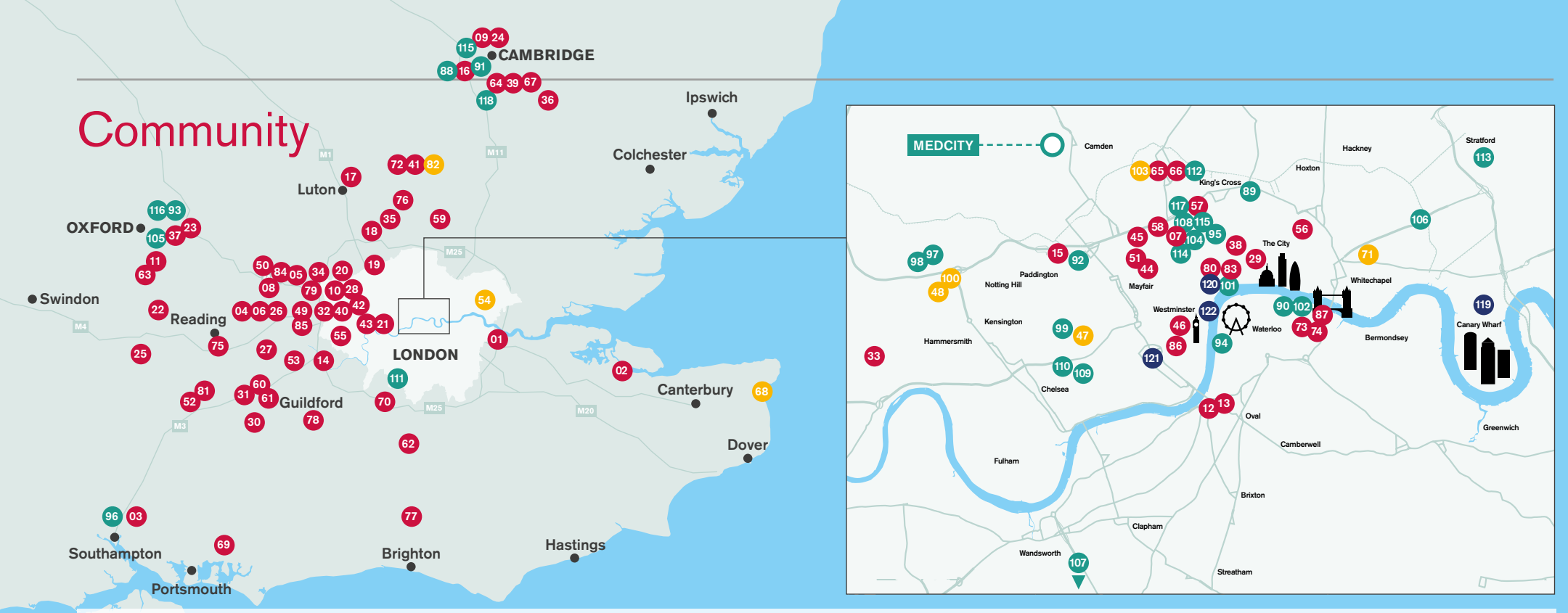
Within its rich ecosystem of universities, hospitals, medical charities, biotechs and pharma, the golden triangle also offers access to a unique clinical trial population via the National Health Service. The NHS has over eight million patients registered in London alone, a highly diverse pool of individuals, and an established point of contact for those seeking to recruit and run trials, in the form of the National Institute for Health Research.

NHS clinicians and nurses, alongside the region's research institutions, have significant experience running trials; as importantly, they – and their patients – are enthusiastic participants. "Nearly every centre we asked to join a trial said 'yes', they wanted to be involved," says Gregg Sando, CEO of London-based cell therapy firm Cell Medica. "There was great interest in the science, and in wanting to be on the leading edge."

"The importance of the golden triangle is that we cover all the elements, from the fundamental understanding of disease, to having access to over nine million patients. We are also the hub for the 100,000 Genome Project, which will have patient outcome data correlated with genetic data in a way that has not been done before."

Eliot Forster, Executive Chair of MedCity and CEO, Immunocore

Community



Commercial/R&D/Manufacturing

- **01–05** Abbott
- **06** Abbvie
- **07** Abcodia
- **08** Allergan
- **09–11** Amgen
- **12** Association of the British Healthcare Industries
- **13** Association of the British Pharmaceutical Industry
- **14** Astellas
- **15–17** AstraZeneca
- **18–23** Baxter
- **24–25** Bayer
- **26** Biogen Idec
- **27** Boehringer Ingelheim
- **28** Bristol-Myers Squibb
- **29–31** BTG
- **32** Celgene
- **33** Chugai Pharma
- **34** Daiichi Sankyo
- **35** Eisai
- **36–37** Genzyme
- **38–40** Gilead Sciences
- **41–43** GlaxoSmithKline
- **44** GW Pharmaceuticals
- **45** Immodulon Therapeutics
- **46** ImmuPharma
- **47** Imperial College Bio Incubator
- **48** Imperial College ThinkSpace
- **49** Ipsen
- **50** Janssen
- **51** Johnson & Johnson Innovation Centre
- **52–53** Lilly
- **54** London East Science Park
- **55** Merck Serono
- **56** Mitsubishi Tanabe Pharma
- **57** MRC Technology
- **58–59** MSD
- **60–61** Novartis
- **62** Novo Nordisk
- **63** OBN
- **64** One Nucleus
- **65–70** Pfizer
- **71** Queen Mary Bio Enterprises
- **72–75** Quintiles
- **76–77** Roche
- **78** Sanofi
- **79** Servier
- **80** Shionogi Limited
- **81** Shire
- **82** Stevenage Biocatalyst
- **83–84** Takeda
- **85** UCB
- **86** UK BioIndustry Association
- **87** Verona Pharma

Academic Health Science Environment

- **88** Cambridge University Health Partners
- **89** Cancer Research UK
- **90** Cell Therapy Catapult
- **91** Genomic Medicine Centre East of England
- **92** Genomic Medicine Centre Imperial College Health Partners
- **93** Genomic Medicine Centre Oxford
- **94** Genomic Medicine Centre South London
- **95** Genomic Medicine Centre UCLPartners
- **96** Genomic Medicine Centre Wessex
- **97** Imanova
- **98** Imperial College Academic Health Science Centre
- **99** Imperial College London
- **100** Imperial West
- **101** King's College London
- **102** King's Health Partners
- **103** London BioScience Innovation Centre
- **104** London School of Hygiene and Tropical Medicine
- **105** Oxford Academic Health Science Centre
- **106** Queen Mary University of London
- **107** St. George's, University of London
- **108** The Francis Crick Institute
- **109–111** The Institute of Cancer Research
- **112** The Royal Veterinary College
- **113** UCL East
- **114** UCLPartners
- **115** University College London
- **115** University of Cambridge
- **116** University of Oxford
- **117** Wellcome Trust
- **118** Wellcome Trust Sanger Institute

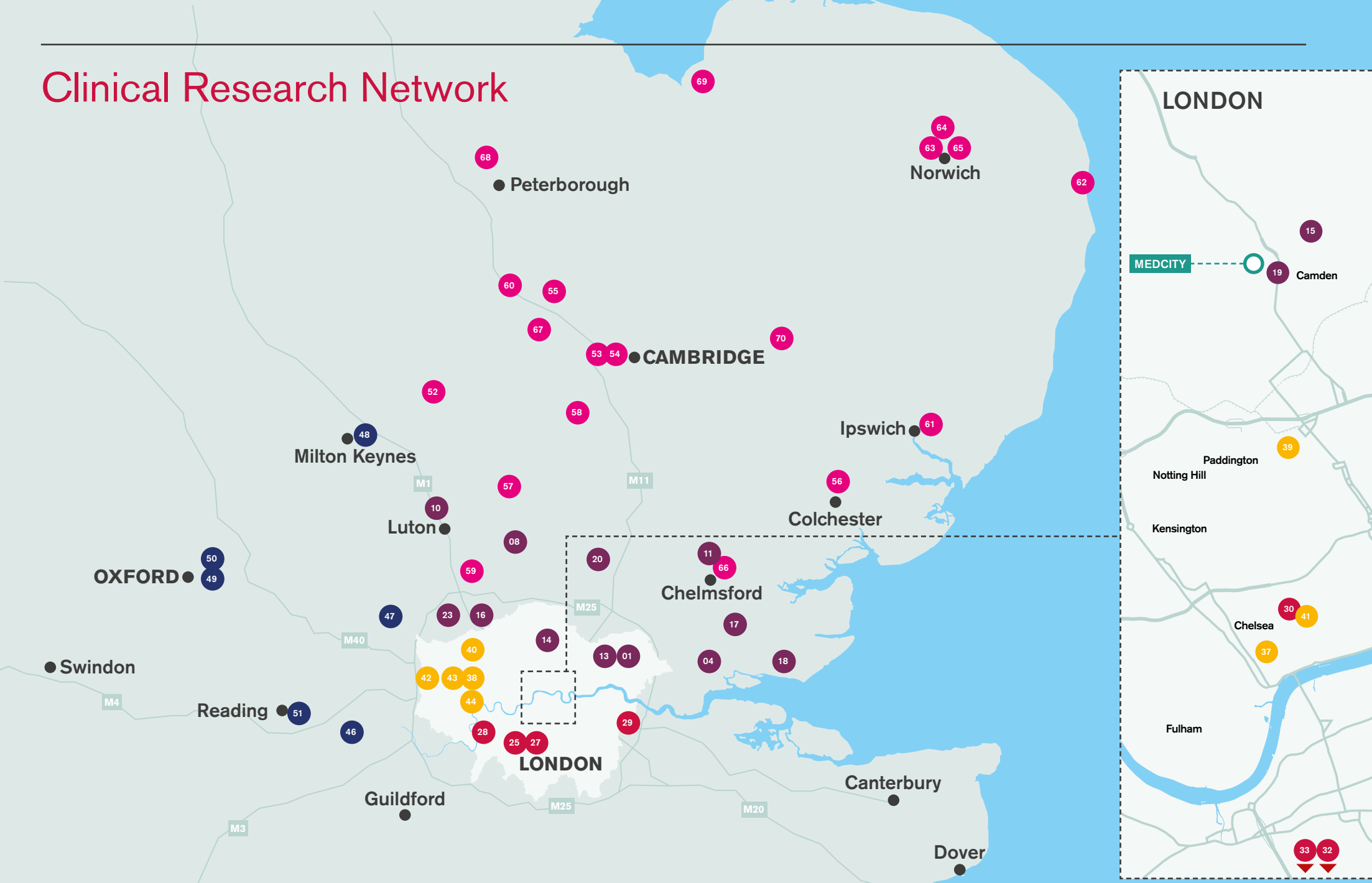
Agency

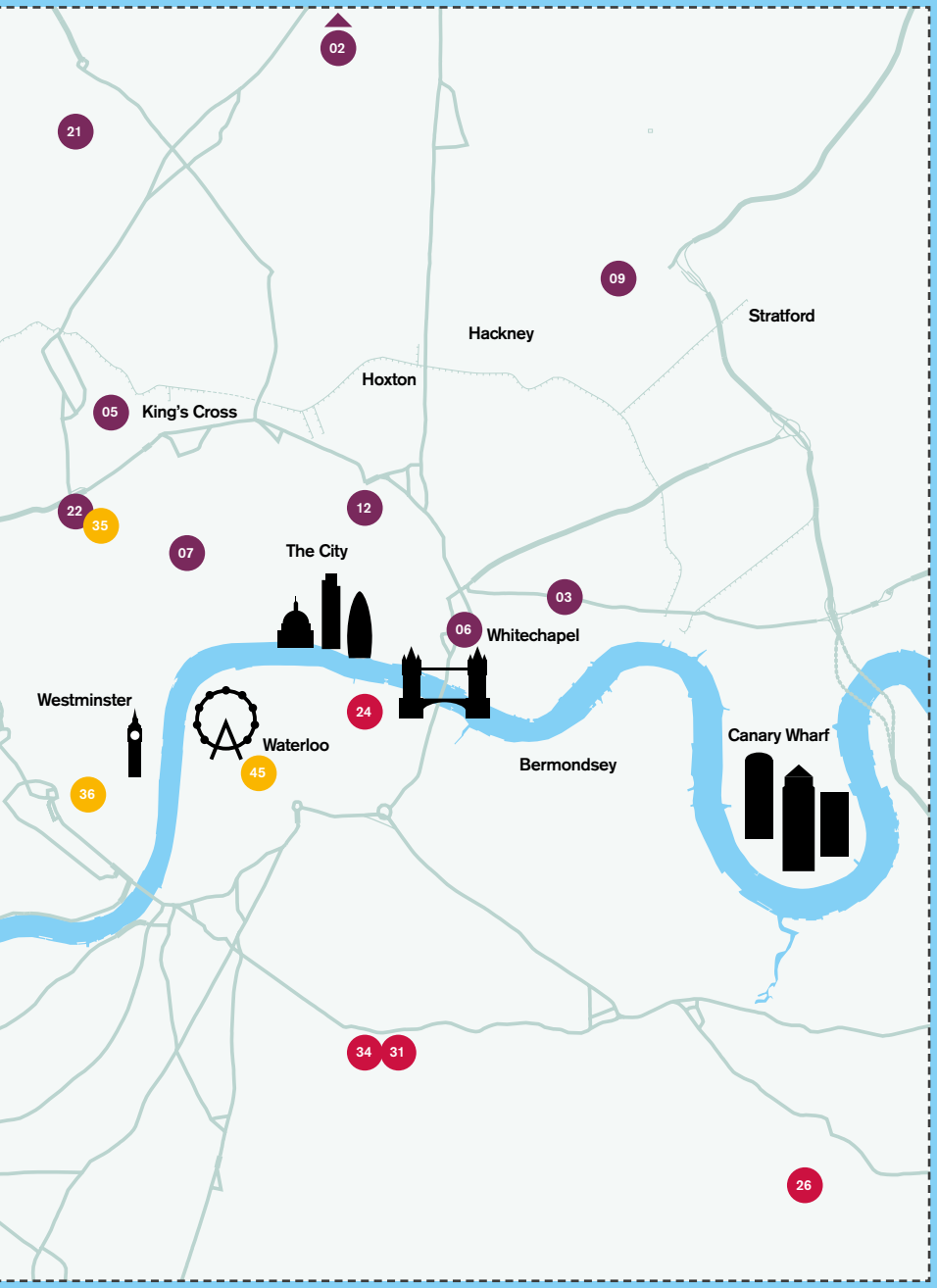
- **119** European Medicines Agency
- **120** Medical Research Council
- **121** Medicines and Healthcare Products Regulatory Agency
- **122** National Institute for Health Research

Incubators/ Science Parks

- **47** Imperial College Bio Incubator
- **48** Imperial College ThinkSpace
- **54** London East Science Park
- **68** Discovery Park Kent
- **71** Queen Mary Bio Enterprises
- **82** Stevenage Biocatalyst
- **100** Imperial West
- **103** London BioScience Innovation Centre

Clinical Research Network





LONDON

NORTH THAMES CLINICAL RESEARCH NETWORK

01 Barking, Havering and Redbridge University Hospitals NHS Trust
02 Barnet, Enfield and Haringey Mental Health NHS Trust
03 Barts Health NHS Trust
04 Basildon & Thurrock University Hospitals NHS Foundation Trust
05 Camden and Islington NHS Foundation Trust
06 East London NHS Foundation Trust
07 Great Ormond Street Hosital for Children NHS Foundation Trust
08 Hertfordshire Community NHS Trust
09 Homerton University Hospital NHS Foundation Trust
10 Luton and Dunstable Hospital NHS Foundation Trust
11 Mid Essex Hospital Services NHS Trust
12 Moorfields Eye Hospital NHS Foundation Trust
13 North East London Foundation Trust
14 North Middlesex University Hospital NHS Trust
15 Royal Free London NHS Foundation Trust
16 Royal National Orthopaedic Hospital NHS Trust
17 South Essex University Partnership NHS Foundation Trust
18 Southend University Hospital NHS Foundation Trust
19 Tavistock and Portman NHS Foundation Trust
20 Princess Alexandra Hospitals NHS Trust
21 The Whittington Hospital NHS Trust
22 University College London Hospitals NHS Foundation Trust
23 West Hertfordshire Hospitals NHS Trust

SOUTH LONDON CLINICAL RESEARCH NETWORK

24 Guy's and St Thomas' Foundation NHS Trust
25 Epsom and St Helier University Hospitals NHS Trust
26 Lewisham and Greenwich NHS Trust
27 Croydon Health Services NHS Trust
28 Kingston Hospital NHS Foundation Trust
29 Oxleas NHS Foundation Trust
30 The Royal Marsden NHS Foundation Trust
31 South London and Maudsley NHS Foundation Trust
32 South West London and St George's Mental Health NHS Trust
33 St George's University Hospitals NHS Foundation Trust
34 Kings College Hospital NHS Foundation Trust

NORTH WEST LONDON CLINICAL RESERCH NETWORK

35 Central And North West London NHS Foundation Trust
36 Central London Community Healthcare NHS Trust
37 Chelsea And Westminster Hospital NHS Foundation Trust
38 Ealing Hospital NHS Trust
39 Imperial College Healthcare NHS Trust
40 London North West Healthcare Trust
41 Royal Brompton & Harefield NHS Foundation Trust
42 The Hillingdon Hospitals NHS Foundation Trust
43 West London Mental Health NHS Trust
44 West Middlesex University Hospital NHS Trust
45 West London Ambulance Trust

SOUTH EAST

THAMES VALLEY AND SOUTH MIDLANDS

46 Berkshire Healthcare NHS Foundation Trust
47 Buckinghamshire Healthcare NHS Trust
48 Milton Keynes University Hospital NHS Foundation Trust
49 Oxford Health NHS Foundation Trust
50 Oxford University Hospitals NHS Trust
51 Royal Berkshire NHS Foundation Trust

EASTERN

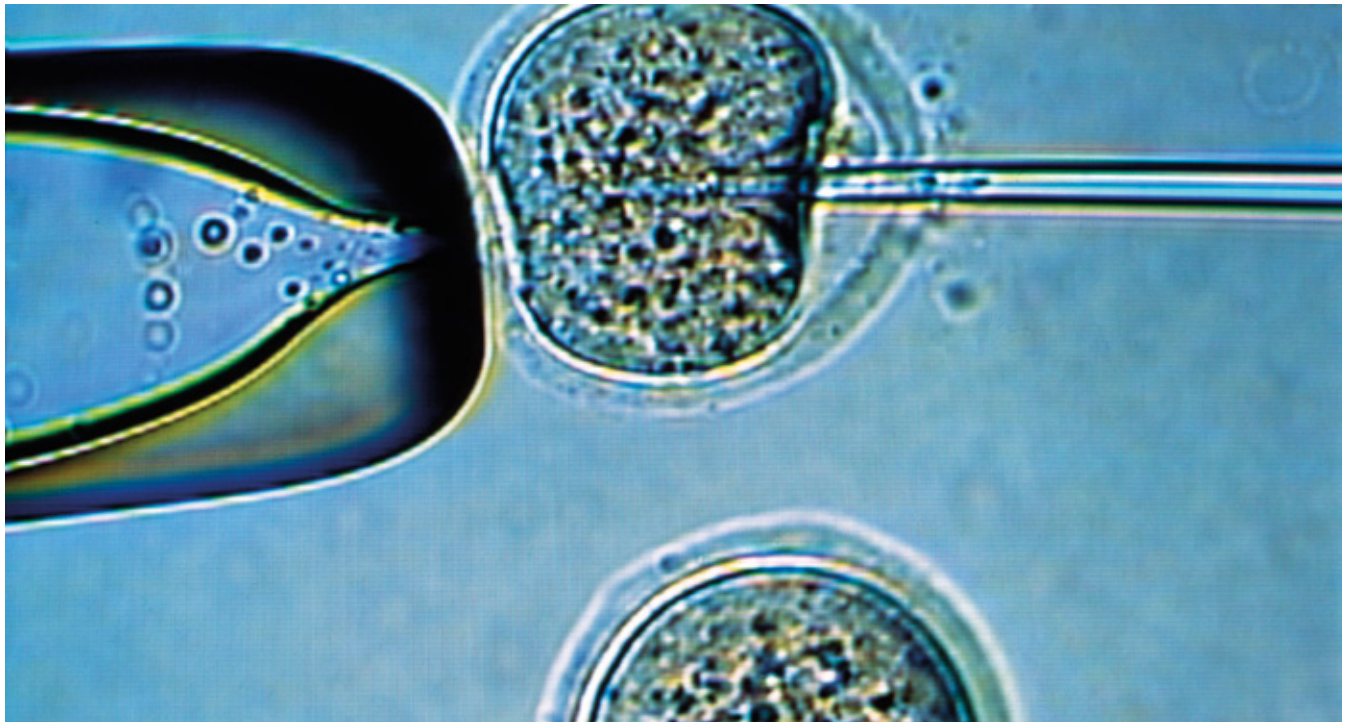
52 Bedford Hospital NHS Trust
53 Cambridge University Hospitals NHS Foundation Trust
54 Cambridgeshire and Peterborough NHS Foundation Trust
55 Cambridgeshire Community Services NHS Trust
56 Colchester Hospital University NHS Foundation Trust
57 East and North Hertfordshire NHS Trust
58 East of England Ambulance Service NHS Trust
59 Hertfordshire Partnership University NHS Foundation Trust
60 Hinchingbrooke Health Care NHS Trust
61 Ipswich Hospital NHS Trust
62 James Paget University Hospitals NHS Foundation Trust
63 Norfolk and Norwich University Hospitals NHS Foundation Trust
64 Norfolk and Suffolk NHS Foundation Trust
65 Norfolk Community Health and Care NHS Trust
66 North Essex Partnership University NHS Foundation Trust
67 Papworth Hospital NHS Foundation Trust
68 Peterborough and Stamford Hospitals NHS Foundation Trust
69 The Queen Elizabeth Hospital, King's Lynn, NHS Foundation Trust
70 West Suffolk NHS Foundation Trust

Discover more about the golden triangle's ecosystem at **medcitymap.com**

Translational excellence

The UK has long recognised the need to translate research into marketable therapies, improved health outcomes, successful businesses and a growing economy. The golden triangle has established highly sophisticated technology transfer organisations, and supported investment in world-class production and manufacturing infrastructure. King's College London's Cell Therapy Unit is Europe's largest GMP lab facility for cellular and gene therapies used in trials and academia. In 2017 the Cell Therapy Catapult will open a major £55 million manufacturing centre on the Stevenage BioScience Catalyst campus, north of London; this will be used to make late phase clinical trial and commercial supply of cell and gene therapies.

London is also home to the UK drugs regulator, the Medicines and Healthcare products Regulatory Agency (MHRA), plus the world-class healthcare assessment agency, the National Institute of Health and Clinical Excellence (NICE).



The National Institute for Health and Care Excellence (NICE)

The National Institute for Health and Care Excellence (NICE) technology appraisal programme reviews clinical and economic evidence to set recommendations on the use of new and existing medicines for the NHS in England. The evaluation and decision making frameworks for the programme compare the clinical and cost effectiveness of a diverse range of medicines for different disease areas. These methods can also be applied to regenerative medicines and cell therapies. The UK Regenerative Medicine Expert Group, which NICE was actively involved in, identified areas where evaluating regenerative medicine and cell therapies was particularly complex and recommended that NICE investigate these complexities and propose potential solutions. This study is in progress and includes consideration of a hypothetical CAR-T cell product for treating acute lymphoblastic leukaemia, with characteristics based on early clinical data for related treatments supplemented with hypothetical evidence. It explores multiple scenarios, varying parameters such as estimates of effectiveness, maturity of evidence, discounting rates applied, price and payment models and managed access arrangements.

Diverse biopharma talent

The region's strong scientific and clinical base, coupled with London's status as a global financial centre, has helped generate Europe's leading cluster of cell and gene therapy start-ups. These include T-cell-focused Adaptimmune, Oxford-based Immunocore, which recently secured the biggest ever private fundraising by a European biotech, Cell Medica in London, and newly-founded Autolus, which is working on engineered chimeric antigen receptors (CAR) T-cell products. Meanwhile, established players such as genetherapy-focused Oxford BioMedica, listed on the London Stock Exchange, and AIM-listed ReNeuron PLC, continue to lead in their respective fields.

The region also draws in large multinational pharmaceutical and biotech firms hungry for talent, new science, and partnerships. Pfizer set up its gene therapy unit in London in late 2014; about the same time that Japan's Tokyo Electron Limited chose the Stevenage Bioscience Catalyst to launch its open innovation stem cell technology centre, to develop smart cell processing technologies. AstraZeneca recently chose Cambridge for its new global R&D hub, joining other multinational R&D players in the area, including Takeda. US-headquartered Gilead Sciences in February 2015 announced it too was expanding its R&D in Cambridge, as well as opening a new UK commercial headquarters in London.

Fantastic funding opportunities

These strategic investors, plus the City of London's growing venture capital and institutional investment communities, and world-class research institutions, provide research partners and entrepreneurs with an expanding suite of funding opportunities.

"The UK gets a really big tick for funding options," enthuses Cell Medica's CEO, Gregg Sando. Besides the Medical Research Council and Innovate UK, a public body which supports science and technology innovations, he singles out the Wellcome Trust as "a tremendous force" for helping to translate academic ideas into commercial products in the biomedical sector.

Further downstream, almost $2 billion was raised in life sciences IPOs and follow-on offerings on the London Stock Exchange in 2014.

Committed, focused government support

The UK benefits from sustained long-term commitment to life sciences. In 2014 it became the first country to appoint a dedicated Minister for Life Sciences: industry veteran George Freeman. His work includes fast-tracking the uptake of new drugs and technologies with a particular focus on genomics and regenerative medicine.

Government-funded organisations such as Innovate UK are dedicated to building successful businesses, while newer more focused initiatives such as the Cell Therapy Catapult have also emerged, specifically to encourage and de-risk gene therapy and regenerative medicine.

The UK's suite of support for business and innovation includes Patent Box, which reduces corporation tax on profits resulting from patented inventions, and R&D tax credits, tax relief for small and mid-sized enterprises that engage in R&D, making the UK an extremely competitive place to invest and invent.

Bringing these strengths together is MedCity, launched by Mayor of London Boris Johnson in April 2014. MedCity is a unique portal for the international life sciences industry to access and collaborate with the region's expertise.

"A number of Japanese companies are locating here and actively collaborating here. My mission as Minister in both Department for Business and Health is to make sure that we have the infrastructure for these Japanese companies"

George Freeman, Parliamentary Under Secretary of State for Life Sciences

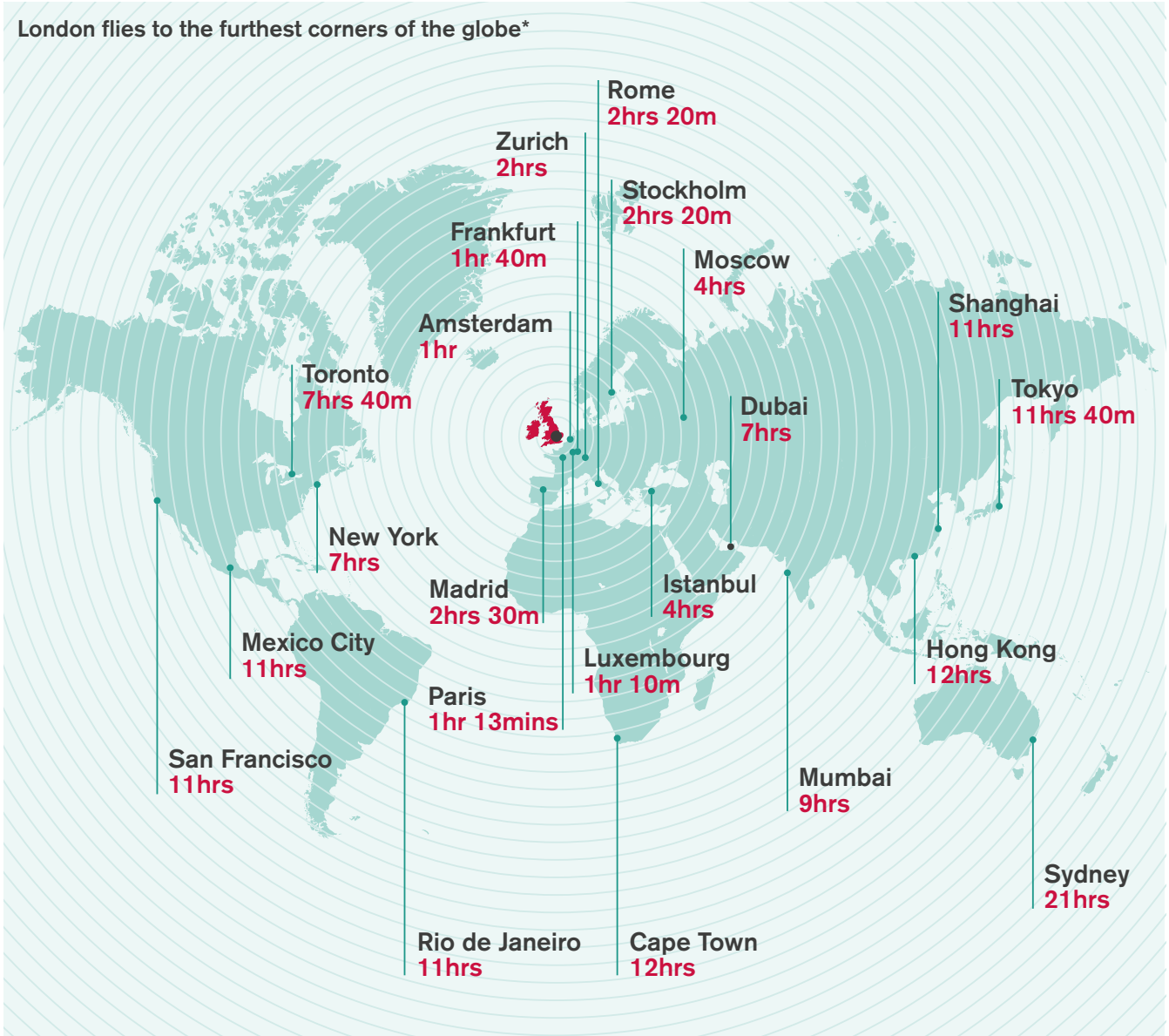
Use Blippar to unlock a video

Rich networks, deep talent, ideal time-zone

By providing access to rich networks of expertise and financial support, these programmes draw on and further encourage Cambridge, London and Oxford's collaborative, cross-disciplinary culture of open innovation. The golden triangle already benefits from the UK's lower-than-average corporation tax rates; the region is also ideally located geographically between the major time-zones of the US and Far East. Add to that the compelling need for most of the world's best scientists to learn English, and it's easy to see why the region assembles an "incredible pool of scientific and medical people," according to Cell Medica CEO, Gregg Sando.

In conclusion, the golden triangle offers unmissable opportunities for its scientific and business communities, as well as for their actual and future partners.

The golden triangle's office hours overlap with those countries that collectively account for 99% of the world's GDP.



*OAG

A snapshot of what is happening in specialist cell therapy institutes across the region

The Cell Therapy Catapult

The Cell Therapy Catapult, based at Guy's Hospital in London, is an innovation centre translating cell and gene therapy research projects into life-saving medicines. Set up in 2012 to support cell therapy businesses, the Catapult provides clinical trial, technical, manufacturing, regulatory and market access expertise.

The Catapult also develops key UK infrastructure capabilities and technological innovations, such as the cell therapy manufacturing centre in Stevenage. This secure, compliant, 7200m^2 facility will allow developers from across the world to reliably ship UK-manufactured cell-lines and cell-based products into the European Union within 24 hours.

Partners such as Tokyo Electron rely on the Cell Therapy Catapult to find the optimal location for their businesses. "Tokyo Electron chose to locate its Stem Cell Technology Centre (STC) in the UK because of the industry support the Cell Therapy Catapult provides, and the world-leading scientific research conducted in the country," confirms Bob Honda, Director of STC and Tokyo Electron Europe Limited (TEE).

Under an agreement with iPS Academia Japan, the Cell Therapy Catapult is permitted to sub-license, manufacture and commercialise iPS cell lines for use in early-stage research and clinical trials.

"Thanks to the Cell Therapy Catapult, the Wellcome Trust and other public bodies, as an entrepreneur in London, you feel the wind is behind you"

Gregg Sando, CEO Cell Medica

Use Blippar to unlock a video

Oxford: Collaborating to uncover stem cell opportunities

Oxford, just one of the dynamic clusters of scientific excellence within the golden triangle, boasts a dynamic network of stem cell focused researchers, institutes and innovation.

The Oxford Stem Cell Institute brings together over 40 laboratories across the city; several of its members have already established ties with the University of Tsukuba, Japan, on the Life Science Innovation Program. This comprehensive training program assembles both academic and industrial partners, offering the UK closer ties with Japanese stem cell researchers and life sciences companies.

As well as breaking down geographical barriers, the Oxford Stem Cell Institute also recognises the need for cross-disciplinary research – overcoming taxonomic barriers between cancer, regenerative medicine and traditional drug development.

Oxford is also the lead academic institution within the five year StemBANCC research programme, a €55.6 million European project funded by the Innovative Medicines Initiative. StemBANCC assembles partners from 11 countries, and aims to generate human-induced pluripotent stem cells (iPSC) as a platform for drug discovery, in particular in brain diseases and diabetes. The project is riding on the wave of international iPSC research, driven most notably by Japanese researchers and Shinya Yamanaka's work.

Reflecting the translational excellence across the region, a new company, OxStem, has been set up to spin out cell therapy focused companies from the University of Oxford. OxStem will leverage both stem cell and medicinal chemistry expertise within the University and other partners to create and spin-out a series of daughter companies focused on different therapy areas, such as dementia or macular degeneration in the eye. These 'Stem' companies will ultimately deliver small molecule therapeutics that work by activating repair mechanisms already present within the body, rather than manipulating cells in vitro before re-insertion. Acting as a parent holding company, OxStem funds the spin offs, as well as licensing its IP.

Cambridge: Novel, cross-disciplinary approaches to treat osteoarthritis

Scientists at the University of Cambridge Stem Cell Institute, funded by the Wellcome Trust/Medical Research Council, are ideally placed to collaborate and network with neighbouring disciplines, research institutes and industry, both within Cambridge's rich biomedical talent pool and beyond. They are exploring stem cells' fundamental properties, function and their role in disease, as well as seeking to harness endogenous stem and progenitor cells for repair and regeneration.

The Institute works with affiliate scientists whose research intersects with stem cell biology and medicine. One of these is Andrew McCaskie, Professor of Orthopedic Surgery at the University of Cambridge. McCaskie and Cambridge Stem Cell Institute Director, Professor Austin Smith, are investigating whether stem cells and related regenerative techniques could allow damaged joints in patients with osteoarthritis to be repaired, thereby radically changing the way the condition is treated.

"The conventional approach is to treat osteoarthritis when the joint is extensively damaged by using a joint replacement. We want to treat the condition at an earlier stage, using repair and regenerative techniques to prolong the use of the patient's own joint and therefore defer joint replacement," says McCaskie.

McCaskie leads two other multicentre, collaborative efforts – each with a strong focus on translating great science into great products. The Arthritis Research UK Tissue Engineering Centre, assembling universities and hospitals across the UK, focuses on both cell and cell-free approaches to regenerative therapies in osteoarthritis.

The SmartStep consortium ("Stepwise Translational Pathway for Smart Material Cell Therapy"), funded via the UK Regenerative Medicine Platform, an initiative that is addressing the key translational challenges of regenerative medicine, is exploring ways to stimulate the patient's own repair mechanisms by targeting different cell populations. This will have a particular focus on novel biomaterials and bioactive molecules that may help recruit and differentiate resident stem and/or progenitor cells within the joints. SmartStep includes investigators from across the UK and the Netherlands.

Cell therapy in action

Osaka University and King's College London: Shared Innovation

A London-Osaka partnership is tackling painful skin diseases with bone marrow cells that can transform into skin cells to repair tissue.

The international collaboration between King's College London and Osaka University has discovered the 'distress signal' released by damaged skin – a protein that mobilises cells from bone marrow and directs them to where they are needed. The discovery opens up the potential to persuade bone marrow to mobilise an increased number of cells to improve skin repair, according to Professor John McGrath, who leads the research at KCL.

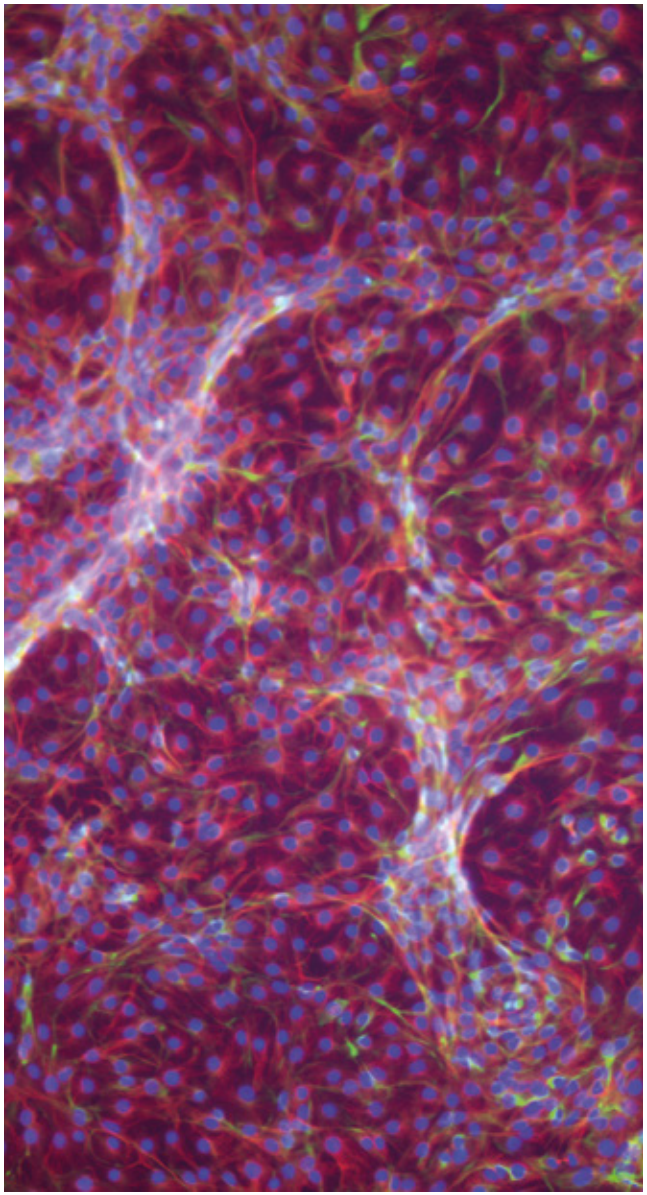
"It's a bit like dealing with your bank if the roof blows off your house," he explains. "The bank may only be inclined to give you £500 to fix the basic problem. If you can persuade it to give you £5,000 to make thorough improvements, you have a much sounder, longer-lasting structure. For damaged skin, this means being able to get the repairs done quickly when you need to."

"We have an extraordinary level of support in the UK to facilitate clinical trials through the National Institute for Health Research and Biomedical Research Centres that really speeds up the process, whereas in Japan the infrastructure for clinical trials is not so well set up.

What is shared by researchers in the UK and Japan, however, is a tremendous energy to innovate and to deliver new health benefits for patients, wherever they may be."

The team is now carrying out clinical trials in London and Osaka to develop a cell therapy that will repair skin and heal wounds, improving quality of life for patients and saving money for healthcare systems. The work initially targets inherited skin diseases such as Epidermolysis Bullosa, which causes painful blisters. However, Professor McGrath believes it will have much broader applications over the longer-term, including wound healing for conditions such as varicose vein ulcers, which currently require dressings for many months.

The partnership between KCL and Osaka is based on common protocols for research and clinical trials for cell and gene therapy between the two institutions, and Professor McGrath is enthusiastic about the benefits of joint working. He adds, however, that the experience of establishing clinical trials in the UK and Japan has given him a renewed appreciation for the NHS.



Use Blippar to unlock a video

Takeda partners locally with world-class talent

Earlier in 2015, Japan's largest pharmaceutical company Takeda joined forces with University College London to find new targets to treat a range of neurodegenerative diseases including Huntington's, Parkinson's and Motor Neuron diseases. The partners will use established cell lines from both patients and their healthy relatives to better understand the mechanisms of various CNS disorders, and to discover improved treatments.

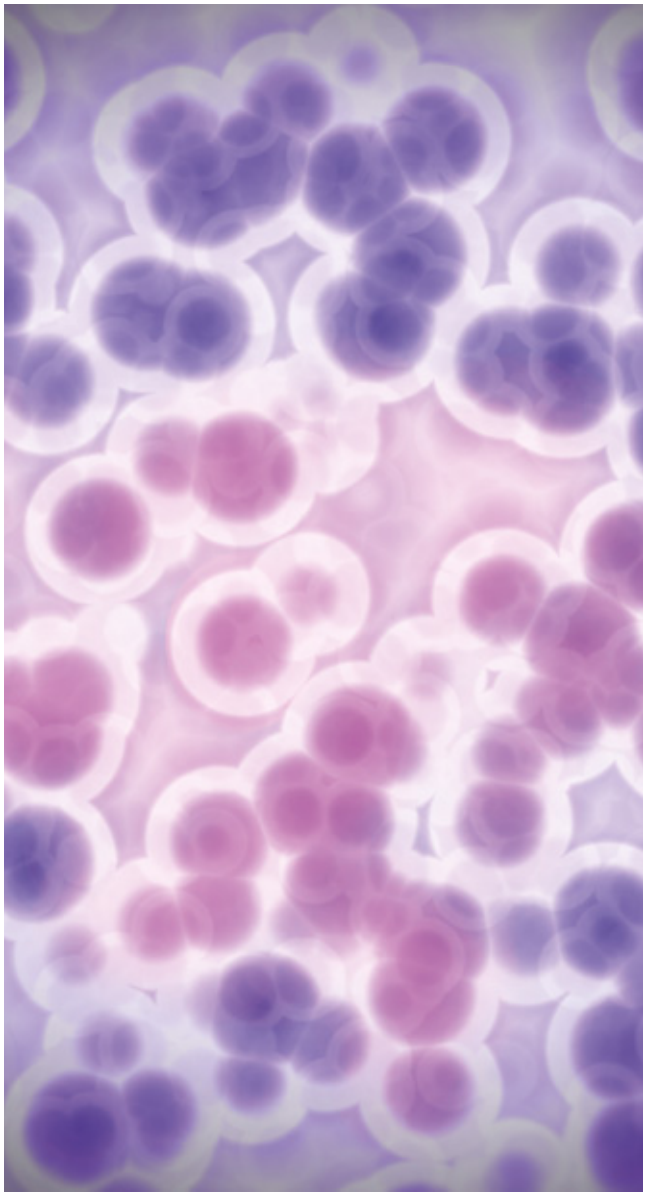
The collaboration was driven from Takeda's Cambridge-based UK research hub, and made possible by the Japanese group's established presence in the golden triangle, including clinical development offices in London. "Cambridge is only a 50 minute train ride from London," remarks Mark Carlton, President of Takeda Cambridge. "When University College London collaborators come up here, they often have ex-PhD students and other colleagues that they can also meet with." Takeda Cambridge has doubled its headcount to around 150 highly skilled scientists, thanks to the rich pool of talent, willing to work in an area with many employment opportunities across biotech, academia and pharmaceuticals.

Takeda selected University College London after scrutinising a range of potential partners. What set the London-based group apart was the level and depth of coordination and professionalism within their neurology group, Mark Carlton recalls, as well as the quality of their research proposals. Technologies and data banks were another big plus: "When they collect patient tissue, they also collect from healthy relatives, so you can compare genetically closely-aligned cell types. That offers a powerful control reducing the 'noise' in the dataset," says Mark Carlton.

"When they collect patient tissue, they also collect from healthy relatives, so you can compare genetically closely-aligned cell types. That offers a powerful control"

Carlton is excited about the promise of CNS research in the era of Big Data and genomics in particular. "We're at a turning point in CNS research," he says, describing the beginnings of patient and disease stratification. Joint government and industry-funded initiatives such as Genomics England, whose aim is to sequence 100,000 genomes of people with conditions including cancer, CNS disorders and rare diseases, will provide valuable data to allow a improved understanding of causal events driving these conditions. "We'll see important changes in the coming years, in imaging, patient stratification, and in clinical trial design," he predicts.

Takeda's interest in cell and gene therapy research extends well beyond CNS; the group has also been working since 2014 with University College London on using induced pluripotent stem cells to treat muscular dystrophy. This partnership, run out of Takeda's US-based New Frontiers group, is also looking at novel cell and gene therapy strategies using artificial human chromosomes and novel biomaterials.



Cell Medica: Showcasing UK science, trials, funding and support

Cell Medica's evolution illustrates the UK's dynamic research and clinical trial environment, its commercial focus, and its extensive funding and support networks.

The London-based company was set up in 2007 around an adoptive T-cell immunotherapy project that was already undergoing early trials in an academic setting. The start-up's goal was to take the programme to the next level – into market-focused, confirmatory trials. Adoptive T-cell immunotherapy involves transferring a virus-specific T-cell from a healthy donor, to restore immunity to that virus in a bone marrow transplant patient, for example, a patient with leukemia or another haematological malignancy.

Researchers at University College London, the Royal Free Hospital and the University of Birmingham had been pioneering this new paradigm for treating patients so "the UK was a natural place to set up the company," explains CEO Gregg Sando. Their product, Cytovir CMV, for the prevention of infections in bone marrow transplant patients, has since been the subject of two randomised clinical trials across 15 UK transplant centres, part-funded by the Wellcome Trust. "We had a good experience of running clinical trials in the UK," concludes Gregg Sando. "Nearly every centre that we asked to join a trial said yes, because it was interesting science, and they wanted to be involved."

Imperial Innovations engages in technology transfer, licensing, company incubation and investment, with a focus on IP generated at or associated with four top institutions in the UK's golden triangle: Imperial College London, Universities of Oxford and Cambridge, and University College London. The group has invested over £1 billion in UK innovation.

Cell Medica has also benefited from financial support from Innovate UK, which provided £2 million for the company to develop, in partnership with University College London, a more cost-effective system for manufacturing T-cell products as well as funding development and testing of a new product targeting adenovirus infection in children receiving transplants. It is also working with the Cell Therapy Catapult to achieve NHS reimbursement for Cytovir CMV.

In November 2014, Cell Medica raised £50 million in a Series B that included three of the UK's most significant life sciences investors: Imperial Innovations, Invesco Perpetual and Woodford Investment Management.

Pfizer and the Global Medical Excellence Cluster (GMEC)

Pfizer has over 20 years' experience in rare diseases and is dedicated to discovering more new medicines. They have two operations in London supporting this goal.

Both work on the principles of collaboration. Pfizer believes that no one person has all the answers – by working together, they can achieve more and faster.

The Genetic Medicine Institute is a new research facility dedicated to the development of genetic medicines at pace and scale. They are exploring how to use viruses as tools to replace or repair faulty genes. Located in the heart of London's science community, the Genetic Medicine Institute supports the free flow of knowledge, ideas and best practice between industry and academia.

Pfizer has also joined with fifteen of the UK's leading universities to create the Rare Disease Consortium (RDC). Initially a partnership between Pfizer and the Global Medical Excellence Cluster (GMEC), which includes University of Cambridge, Imperial College London, King's College London, University of Oxford, University College London and Queen Mary University of London, the consortium has expanded to include the N8 research group in North England as well as the University of Dundee.

In a recent government report, Pfizer was recognised as the most active collaborator in UK university research, headquartered outside the UK.

The RDC has recently initiated research into read-through disorders with the University of Dundee, in haematology and neuromuscular disorders with the University of Oxford and neuromuscular disorders with Oxford, Imperial College London and University College London.

What next?

Japan and the golden triangle share the challenge to improve the health of their populations, a world-leading scientific pedigree, a history of effective partnerships in life sciences and flourishing research and development in cell and gene therapy, making them natural collaborators.

If you would like to explore the opportunity to work with organisations and institutions in the golden triangle, both MedCity and London & Partners can help you access this thriving life sciences region.

London & Partners is the official promotional company for London. For practical information on how to set up your business in London, visit our website or contact us to see how we can help.

business@londonandpartners.com
+44 (0)20 7234 5800
invest.london

@L_Pbusiness

MedCity can help you navigate and access different parts of the academic, NHS and industrial life sciences and healthcare environment across the golden triangle of Cambridge, London, Oxford and the greater south east region of England.

contact@medcityhq.com
+44 (0)20 7691 3588
medcityhq.com

@MedCityHQ

GREAT BRITAIN & NORTHERN IRELAND

LONDON & PARTNERS

MedCity