Milner Therapeutics Symposium 2017

Monday 2nd October
Registration from 9.00am

Venue

West Road Concert Hall
11 West Rd, Cambridge
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Milner Therapeutics Symposium

October 2nd 2017

9.30-9.45 Introduction Tony Kouzarides

Session 1, Chair: Prof Richard Gilbertson

9.45-9.10.15 Prof Craig Mello (University of Massachusetts Medical School)

10.15-10.45 Dr David Reese (Amgen)

Coffee

11.15-11.45 Dr David Tapolczay (CEO, LifeArc)

11.45-13.00 Open Discussion

Session 2, Chair: Dr Kathryn Chapman

Panelists:
Dr Susan Galbraith,
Dr Cayetano Gonzales,
Dr Melanie Lee,
Prof Andy Neely,
Dr David Reese,
Dr David Tapolczay

Prof Michael Wakelam

Lunch

Session 3, Chair: Dr Greg Hannon

2.00-2.30 Prof Hans Clevers (Hubrecht Institute)

2.30-3.00 Dr Eoin McKinney (University of Cambridge)

3.00-3.30 Prof Ross Cagan (Mount Sinai)

3.30-6.00 Posters with drinks

Director

Tony Kouzarides, FMedSci, FRS
Deputy Director, Gurdon Institute, University of Cambridge

Session Chairs

Prof Richard Gilbertson
Head of Department of Oncology, Director, Cancer Research UK Cambridge Centre

Dr Kathryn Chapman
Executive Manager, Milner Therapeutics Institute, University of Cambridge

Dr Greg Hannon
Royal Society Research Professor of Molecular Cancer Biology, Cancer Research UK Cambridge Institute
Talk Title

Evidence for a temporal window of Argonaute surveillance during mRNA expression in the C. elegans germline

Science

Dr. Mello’s lab uses the nematode C. elegans as a model system to study embryogenesis and gene silencing.

Biography

Dr. Craig C. Mello is an Investigator of the Howard Hughes Medical Institute, the Blais University Chair in Molecular Medicine and Co-director of the RNA Therapeutics Institute at the University of Massachusetts Medical School.

His collaborative work with Dr. Andrew Fire led to the discovery of RNA interference (RNAi), for which they shared the 2006 Nobel Prize in Physiology or Medicine. Together they showed that when C. elegans is exposed to double-stranded ribonucleic acid – dsRNA, a molecule that mimics a signature of viral infection, the worm mounts a sequence-specific silencing reaction that interferes with the expression of cognate cellular RNAs. For the layperson, RNAi is the cell’s search engine; the Google of the cell. Using readily produced short synthetic dsRNAs, researchers can now submit their own RNAi search queries to silence any gene in organisms as diverse as corn and humans. RNAi allows researchers to rapidly “knock out” the expression of specific genes and to thus define the biological functions of those genes. RNAi also provides a potential therapeutic avenue to silence genes that contribute to disease.

Before the Nobel Prize, Dr. Mello’s work on RNAi was recognized with several awards including the National Academy of Sciences Molecular Biology Award, the Canadian Gairdner International Award, the Paul Ehrlich-and Ludwig Darmstaedter Award, and the Dr. Paul Janssen Award for Biomedical Research. He is a member of the National Academy of Sciences, the American Academy of Arts and Sciences, and the American Philosophical Society.
Talk Title
The Great Convergence: New Technologies and Translational Drug Development

Science
Dr. Reese's science focuses on early and translational development of novel treatments for serious disease, across therapeutic areas. His teams have published in journals such as Nature, Cell, and the New England Journal of Medicine.

Biography
Dr. David Reese is senior vice president, Translational Sciences, responsible for Medical Sciences, Comparative Biology and Safety Sciences, and Pharmacokinetics and Drug Metabolism. Prior to assuming this role, Reese was vice president of Translational Sciences and therapeutic area head for Oncology Global Development. Reese joined Amgen in 2005 and served in various roles in Global Development and Medical Sciences.

Prior to joining Amgen, Reese was director of Clinical Research for the Breast Cancer International Research Group (BCIRG) and a co-founder, president and chief medical officer of Translational Oncology Research International (TORI), a not-for-profit academic clinical research organisation. Reese is a graduate of Harvard College and the University of Cincinnati College of Medicine. He completed training in Internal Medicine and Haematology/Oncology at the University of California, Los Angeles (UCLA) School of Medicine, and subsequently served on the faculty at UCLA and the University of California, San Francisco.

Key References
Dr David Tapolczay

Talk Title

Science

Biography
Panelists

Dr Kathryn Chapman (Chair)
Executive Manager,
Milner Therapeutics Institute, University of Cambridge

David M. Reese
Senior Vice President, Translational Sciences,
Amgen

Melanie Lee
Chief Scientific Officer at BTG plc

Professor Michael Wakelam
Director of the Babraham Institute

Susan Galbraith
Senior Vice-President IMED Oncology at AstraZeneca

Professor Andy Neely
Pro-Vice-Chancellor for Enterprise and Business Relations, Head of the Institute for Manufacturing (IfM), University of Cambridge,

Cayetano Gonzalez
Group Leader, ICREA Research Professor, IRB Barcelona

Dr David Tapolczay
Chief Executive Officer, LifeArc
Hans Clevers is member of the Royal Netherlands Academy of Arts and Sciences (2000), of the American Academy of Arts and Sciences (2012) and the National Academy of Sciences of the USA (2014), the Academie des Sciences (2016) and the Orden pour le Merite der Wissenschaften und Kuenste (2017). He is the recipient of multiple awards, including the Dutch Spinoza Award in 2001, the Swiss Louis Jeantet Prize in 2004, the German Meyenburg Cancer Research Award in 2008, the German Ernst Jung-Preis für Medizin in 2011, the French Association pour la Recherche sur le Cancer (ARC) Léopold Griffuel Prize, the Heineken Prize (2012), the Breakthrough Prize in Life Sciences (2013), the 2015 ISSCR McEwen Award for Innovation and the Academy Professor Prize (2015), and the Körber European Science Prize (2016). He obtained two ERC Advanced Investigator grants (2008 and 2016). He is Chevalier de la Legion d’Honneur since 2005, Knight in the Order of the Netherlands Lion since 2012.

Throughout his career, he has worked on the role of Wnt signaling in stem cells and cancer. His discoveries include TCF as the nuclear Wnt effector, the role of Wnt in adult stem cell biology and of Wnt pathway mutations in colon cancer, Lgr5 as a marker of multiple novel types of adult stem cells and as receptor for the Wnt-amplifying R-spondins, and finally a method to grow ever-expanding mini-organs ('organoids') from Lgr5 stem cells derived from a range of healthy or diseased human tissues. This has led to over 600 publications and >70,000 citations.
Talk Title

Proving Pablo wrong: new targets from big data

Science

Using network transcriptomic analysis of purified leucocytes from patients with autoinflammatory disease, I aim to identify key patterns (‘signatures’) of genes correlating with detailed prospective clinical phenotyping. By understanding the genes comprising each signature, I aim to inform the biological mechanisms of each trait. Recent examples include the association of T cell exhaustion with favourable outcome in multiple immune-mediated diseases and identification of a novel pathway associated with outcome in multiple sclerosis. By identifying both shared and distinct signatures across multiple immune-mediated diseases this approach can provide both useful clinical tools and novel insight into mechanisms of disease progression in a wide range of contexts.

Biography

Eoin studied medicine at Oxford University before obtaining his PhD from Cambridge University. He is currently a Wellcome-Beit Research Fellow using systems immunology analysis of immune cell transcriptomes isolated from patients with a broad range of autoimmune, inflammatory and infectious diseases. The approach involves the application of network models to identify key ‘patterns’ or signatures of transcription associated with clinical traits. This is followed by interpretation of the biology driving those signatures, using further in silico analysis of both in vitro and in vivo models.

A major focus of recent work has been the identification of pathways driving and marking severe, relapsing autoimmune disease with a view to developing both predictive biomarkers and novel therapeutic targets. A key discovery is the association of T cell exhaustion with favourable outcome in multiple diseases including SLE, IBD and systemic vasculitis1,2. Eoin is now applying the approach to other immune-mediated diseases including multiple sclerosis and type 1 diabetes using samples collected by international clinical trial networks. A prognostic biomarker in IBD, based on the T cell exhaustion signature, has been developed into a whole blood prognostic biomarker, validated in multiple independent cohorts and is now the focus of a major biomarker-stratified clinical trial.

Increasingly, Eoin is also using both in silico and in vitro screens in combination with in vivo models to identify novel ways of modifying disease outcome-associated signatures. This is the focus of his current Wellcome fellowship, designed to identify new and improved methods of improving clinical outcomes in immune-mediated disease3.

Key References

Professor Ross Cagan

Talk Title
*A Fly Approach to Cancer Therapeutics*

Science
Dr. Cagan’s laboratory uses Drosophila to explore the biology of therapeutics for cancer and Mendelian diseases. Combining fly genetics with medicinal and computational chemistry, they have teamed with the Dar and Schlessinger laboratories to develop a new generation of multi-targeting lead compounds. In addition, development of ‘personalized fly avatars’ forms the basis of a fly-to-bedside clinical trial (NCT02363647) for colorectal and thyroid cancer patients.

Biography
Dr. Cagan received his Ph.D. from Princeton University. After a postdoctoral fellowship at UCLA, he achieved the rank of Professor at Washington University School of Medicine (1993-2007). He is currently (2007-present) Professor in the Department of Developmental and Regenerative Biology, Director of the Center for Personalized Cancer Therapeutics, and Senior Editor of the journal Disease Models and Mechanisms. He was Co-Founder and board member of the biotechnology company Medros Inc.

Dr. Cagan is an expert in utilizing Drosophila to explore cell-cell signaling and epithelial patterning. Moving to Mount Sinai to develop his interest in translational science, Dr. Cagan’s laboratory has helped pioneer the use of Drosophila to identify therapeutic leads for cancer (breast, lung, thyroid, colorectal), diabetes, and inherited genetic diseases. Taking advantage of a century of powerful genetic tools, his laboratory has developed complex, multigenic disease models designed to model aspects of the whole body complexity of human disease. This approach helped promote the first FDA-approved chemotherapeutic for Medullary Thyroid Carcinoma. Working with the chemist Arvin Dar and colleagues, his laboratory has developed a novel platform that combines genetics with medicinal and computational chemistry to build novel lead compounds that emphasize rational polypharmacology. Leveraging these new technologies, Dr. Cagan leads the Center for Personalized Cancer Therapeutics team that develops and treats thyroid and colorectal cancer patients through a personalized fly-to-bedside, open label clinical trial.

Key References


Davletov B

Nonparalytic botulinum molecules for the control of pain.

Davletov B, Mangione AS1, Obara I, Maiarú M, Geranton SM, Tassorelli C, Ferrari E, Leese C, Hunt SP.

Abstract
Local injections of botulinum toxin (Botox) have been reported to be useful not only for the treatment of peripheral neuropathic pain and migraine but also to cause long-lasting muscle paralysis, a potentially serious side effect. Recently, we synthesized a new botulinum-based molecule ("BiTox") that retains neuronal silencing capacity without triggering muscle paralysis. We examined whether BiTox delivered peripherally was able to reduce or prevent the increased nociceptive sensitivity found in animal models of inflammatory, surgical, and neuropathic pain. No motor deficits were seen and acute thermal and mechanical nociceptive thresholds were unimpaired by BiTox injections. Bitox treatment strongly reduced A-nociceptor-mediated secondary mechanical hyperalgesia associated with either complete Freund's adjuvant (CFA)-induced joint inflammation or capsaicin injection and the hypersensitivity associated with spared nerve injury. Taken together with recent clinical data the results suggest that BiTox should be considered for treatment of pain conditions in which A-nociceptors are thought to play a significant role.

Julian Ng

Better Labelling Through Chemistry

Ng J; Correa I; Lang K; Kashikar N; Pestana F; Cachero S; Sutcliffe B; Jefferis GSXE; Landgraf M

Abstract
The use of Fluorescent Proteins (FPs, such as GFP) and fluorophore conjugated immunoantibodies in optical microscopy is now standard practice as a way to visualise proteins in vivo and in fixed samples. However, current applications suffer from limited spectral flexibility and low photon emissions (FPs), or have limited penetration in thick tissues and requires prior fixation and permeabilisation (antibody staining). This is especially important when imaging thick brain tissues, where the current focus is to decipher the connectivity patterns of neuronal cells (spanning 10-100s of micrometers) and synapses (often in the 1000s and...
visualised at sub-micron resolutions) within an intact brain. Determining such connectivity information can help neuroscientists decipher the neurophysiological and behavioural properties of specific neural circuits and the basis of mood disorders and neurodegenerative pathologies in humans.

Over the past few years, we have developed genetically-encoded protein tags that incorporate small, soluble chemical ligands that are coupled to bright, organic fluorophores. We have shown this ultrafast and even labelling method works well when applied to Drosophila and mouse brain tissues, when investigating individual neuronal types and synaptic protein clusters (references below). Here we will present the latest iterations of this method and how it can be applied in biology and drug discovery settings.


Krishnaa Mahbubani

A novel high-yield lymphocyte cell extraction technology for cellular therapeutics

Krishnaa T Mahbubani, Olivia Tysoe, Kathleen Elliott, Nikola Dolezalova, Nikitas Georgakopoulos, Kourosh Saeb-Parsy

Abstract

Human peripheral blood mononuclear cells (PBMCs) isolated from living donors are the main source of lymphocytes for basic and translational research, including drug screening and the generation of cellular immunotherapies such as Chimeric Antigen Receptor (CAR) and regulatory T cells. The number of PBMCs isolated from each living donor is typically $10^8$ cells: insufficient to conduct extensive studies, reproduce pre-clinical or clinical studies, or generate a large supply of identical cells for patient use. Laboratory and drug screening studies must therefore be normalised, or cells pooled from multiple donors, often leading to inconsistent results with large errors owing to donor variability. The problem is compounded when rare subsets of lymphocytes are of interest.

We have developed and validated a method to extract splenic mononuclear cells (SpMCs) from the spleens of deceased organ donors obtained using appropriate ethical approval and informed consent. Our data confirm SpMCs are a viable, functional and well-characterised lymphocyte population with the potential to provide cell numbers up to 1000-fold higher per donor than typically available through PBMC collection. This novel source can ensure sufficient availability of lymphocytes for laboratory, clinical and pharmaceutical applications and can potentially remove the need for donor pooling.

Martin Zeidler

Repurposing low dose methotrexate for the treatment of myeloproliferative neoplasms

Kavitha Chinnaiya, Michelle Lawson, David Hughes, Antony Green, Jon R Sayers, John A Snowden, Martin Zeidler
Abstract
Ectopic activation of the JAK/STAT pathway is associated with many human diseases including the group of blood cancers collectively termed myeloproliferative neoplasms (MPNs). MPNs affect over 30,000 patients in the UK and while recently developed JAK inhibitors such as ruxolitinib have shown considerable benefit, access to ruxolitinib is restricted by its high cost. As such, a pathway inhibitor combining low-cost and proven safety has the potential to provide significant patient and health economic benefit. We recently identified methotrexate (MTX) as a potent JAK/STAT pathway inhibitor. We have shown that MTX levels consistent with current clinical usage strongly decrease constitutively activated JAK/STAT pathway activation. They are also sufficient to significantly reduce the elevated haematocrit, white blood cell counts and splenomegaly of murine polycythemia vera models. In silico modelling suggests that MTX may competitively bind within the ATP pocket of the JAK2 JH1 domain and case report data suggests that haematological values and constitutional symptoms of MPN patients can be improved by MTX treatment. Collectively, this data suggests that MTX may represent an effective treatment of human MPN patients. Progress on the development of Sheffield-led Phase IIa/b clinical trials seeking to repurpose this low cost generic drug will be presented.

Kin Suen

Proteomic analysis of the PIWI interactome in *C. elegans*

Abstract
The PIWI-interacting RNAs (piRNAs) are a class of small ncRNAs whose role in safeguarding the genome integrity is conserved throughout the animal kingdom, including humans. Accumulating evidence suggests that deregulation in the piRNA pathway is associated with infertility and various types of cancer. To further our understanding in how PIWI proteins function mechanistically, we used *C. elegans* as a model system to identify novel players in the piRNA pathway and characterise how PIWI proteins function as small RNA effectors molecularly. In a comprehensive proteomic study of the PIWI interactome in *C. elegans*, we identified DEPS-1 as a direct binding partner of PRG-1, the PIWI protein in *C. elegans*. DEPS-1 possesses motifs as those found in known Ago-binding proteins, suggesting that the PIWI pathway employs similar molecular strategy to function as the Ago-clade family members. We show that mutations in the *deps-1* gene leads to down-regulation of the downstream amplification signal in small RNA pathways, indicating that DEPS-1 plays a critical role in the piRNA pathway. DEPS-1 is also required for the proper formation of ribonucleoprotein (RNP) granules, suggesting a role as a shuttle protein that allows for communication between different types of RNP granules.

Silvia Basilico

Characterization of leukaemogenic regulatory networks in the early phases of Acute Myeloid Leukaemia development

Abstract
Acute Myeloid Leukaemia (AML) is a clonal neoplastic disorder, characterised by an increase in the number of myeloid cells in the bone marrow and an arrest in their maturation. AML is a genetically heterogeneous disease characterized by chromosomal rearrangements that produce fusion proteins with aberrant transcriptional regulatory activities.

The Gottgens group studies transcriptional networks in normal and malignant blood stem/progenitor cells. Mixed-lineage leukaemia (MLL) gene is a common
target for chromosomal translocations especially associated with infant cases of AML. The t(11;19) translocation constitutes one of the three most frequently found fusions in leukaemia cases, giving rise to the MLL-ENL fusion protein. The conserved transactivation domain of ENL donates transcriptional effector properties to the MLL-ENL which acts to constitutively maintain MLL target gene expression.

Our strategy entails the identification of primary targets responsible for the early phases of MLL-ENL leukaemic transformation. Pre-leukaemic state is established via retroviral infection of a mouse haematopoietic cell line with a GFP tagged MLL-ENL fusion gene. Dissection of pre-leukaemic events leading to a block in myeloid cell differentiation is captured within a time frame window after viral infection. Dysregulation of transcriptional network states are assessed via single cell experimental approaches like single cell RNA-Seq combined with the study of global chromatin modifications via ChIP-seq and ATAC-seq techniques.

The derivation of a mouse immortalised MLL-ENL expressing cell line allow us to identify genetic vulnerabilities via genome wide CRISPR-Cas9 screening. Thus, the pre-leukaemic and leukaemic models generated are powerful platforms to better understand how dysregulation of regulatory networks evolve during early phases of leukaemic transformation. Moreover, identification of altered regulatory states may contribute to the identification of potential therapeutic targets that might be used to block AML.

Sven Sewitz

Genome Organisation as the next Frontier in understanding Gene Regulation and Disease Mechanisms

Abstract

The linear DNA molecule of eukaryotic genomes has to be compacted into the nucleus by a factor of over several hundred thousand (>300,000 fold for humans), even during interphase. This is an astonishing level of compaction. At the same time, the genome remains highly organised on several scales\(^1\). It is now clear that this organisation is both non-random and vital. Long-range genomic interactions between thousands of spatially dispersed DNA regulatory elements and their target genes play a major role in eukaryotic gene regulation. These architectural relationships are vital to understand genome control and have direct implications for cell identity and disease. To investigate this, we develop a new approach, combining bioinformatic determination of chromatin states, dynamic polymer modelling of genome structure, quantitative microscopy and Hi-C to demonstrate that differential mobility of chromosome segments leads to self-organisation of the genome in three dimensions\(^2\).

2. Sewitz S. \textit{et al.}, Heterogeneous chromatin mobility derived from chromatin states is a determinant of genome organisation in \textit{S. cerevisiae}, \textit{bioRxiv} 2017
Affiliated Institutes

All logos go here
University of Pennsylvania: Penn Centre for Innovation

Established in 2010, the CRUK Oxford Centre (formerly the Oxford Cancer Research Centre) is a network and partnership between Oxford University, Oxford University Hospitals NHS Trust and Cancer Research UK, based on the University’s Translational Biomedical Research Campus. It harnesses Oxford’s world-leading cancer research with the core aim of facilitating collaboration to ensure rapid translation from scientific discovery to treatments for patients.

The ultimate aim of the Centre is to enhance cancer research activity to increase cancer cure rates. The Centre currently comprises over 500 members from 25 different Departments, Units, Institutes of the University as well as the NHS Trust. The partnership provides a cumulative investment of approximately £55m each year for science in Oxford for research to save and improve peoples’ lives.

The Oxford Centre is an inclusive network of organisations in Oxford for whom cancer research is a priority focus. We support and connect people working across a range of disciplines and aim to facilitate research collaboration on a local, national and international scale to speed up translation from scientific discovery to treatments in patients.

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University of Pennsylvania: Penn Medicine and the Penn Centre for Innovation (PCI). As the nation’s first medical school and home to the first teaching hospital, the Perelman School of Medicine (Penn Medicine) has a long tradition of academic excellence and scientific discovery. Building on this tradition, our innovative, interdisciplinary research programs continue today to pave the way for a future of new paradigms in cutting-edge science. As an internationally renowned community of scientists and physicians, we are dedicated to both advancing knowledge and fostering a culture of excellence in training the next generation of scientific leaders. Our faculty are at the forefront of the biomedical revolution, and we are committed to sustaining a vibrant intellectual environment, with the ultimate goal of translating ground-breaking discoveries into medical therapies that will eradicate disease and improve health care around the world. The Penn Centre for Innovation (PCI: www.pci.upenn.edu) helps to translate Penn discoveries and ideas into new products and businesses for the benefit of society by facilitating connections with the private sector. Whether the end result is a technology license, an R&D alliance, the formation of a new venture, or an integrated combination of these activities, PCI serves as a dedicated one stop shop for commercial partnering with Penn.

**Contact details:**
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Johns Hopkins Technology Ventures:

Johns Hopkins Technology Ventures (JHTV) is the intellectual property administration center of The Johns Hopkins University. In addition to serving as the licensing, patent and technology commercialization office for Johns Hopkins researchers and inventors, JHTV also supports the growth of startup companies in and around the university and is an active liaison to parties interested in leveraging university research or materials for academic or corporate endeavors.

JHTV aims to maximize the impact of The Johns Hopkins University’s research excellence by facilitating the translation and commercialization of discoveries into accessible technologies, products and services that benefit society. The JHTV website is: ventures.jhu.edu and can provide more details.

Contact Details:
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Yonsei University College of Medicine

The history of the Yonsei University College of Medicine starts from the opening of “Kwanghyewon” in 1885. Kwanghyewon was established to provide western-style medical treatment to the people of Chosun (Korea’s former name) suffering from disease, as well as to serve as a teaching facility for its youth to learn western medicine and sanitary science. Then in 1886, the Chejungwon Medical School was established and formalized medical education began. As Korea’s first institution of western medicine, our College of Medicine has been a leader in medicine here for the past 120 years.

In order to create a leading medical college, we are striving to provide an environment where researchers can passionately achieve their greatest potential. In addition, to further develop our education and research potential, we will expand the college’s essential support functions, as well as distribute and apply resources in the most efficient manner.

Ultimately, it is our goal to increase the level of medical education and services in order to become a hub medical institution in the world. We will do this by identifying the most capable individuals at our institution, supporting research and continuing our efforts to lead the way in the latest methods in clinical treatment.
The Spanish National Cancer Research Centre (CNIO) was founded in Madrid to create an international and comprehensive project for excellence in oncology research. In 2016, the CNIO employed 462 people, of whom 420 were scientific personnel and 17% of its staff are foreigners. Nurturing an ecosystem for translational research and innovation, the CNIO helps researchers maximize the value of their science and bring their discoveries to society as new technologies and therapies that can improve the lives of people. A strategy to accommodate different partnership models with industry and facilitate knowledge transfer is at the core of our goals. Our Basic and Translational Programmes are intertwined so creativity can flow in both directions and novel ideas can be identified for future alliances. In this framework of innovation, the CNIO acknowledges the key value of the human capital and its academic role in science, and strives to create a stimulating and dynamic environment to support excellent research and ethical training, interdisciplinary learning, professional growth, and cultural enrichment.

The CNIO has created a legacy of research excellence, which has placed the Centre as one of the top leading Cancer Research Institutes in Europe. The strategic objectives are:

- to develop research that will uncover novel diagnostic and therapeutic approaches in cancer;
- to translate scientific knowledge into clinical practice and ensure patients can rapidly benefit; and,
- to promote innovation effectively transferring CNIO’s knowledge and technology to industry.

**Contact Details:**

Contact: Carolina Pola, PhD, Director of International Affairs
cpola@cnio.es

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The UCL Cancer Institute is a £40 million investment in central London based at University College London (UCL), one of the world’s top universities and a founding member of the Crick Institute. The Institute draws together over 300 scientists working together to develop world-class basic and translational cancer research.

Through our associations with UCL Hospitals NHS Foundation Trust, Great Ormond Street Hospital and the Royal Free Hospital NHS Foundation Trust, the Institute has greater clinical links than any comparable centre in the UK and a significant impact on the delivery of clinical service to cancer patients. This concentration of excellence, access to patients and significant recent investment of cancer treatment and research infrastructure, makes UCL a global leader.

UCL Cancer Institute exemplifies UCL’s distinctive cross-disciplinary approach, bringing together varied disciplinary expertise and theoretical perspectives to advance our understanding of the complexities of cancer and the development of improved diagnostics and treatments.

**Contact Details:**

Tel: +44 (0)20 7679 6500 (Reception)
Email: ci.contact@ucl.ac.uk
The Medicines Discovery Catapult is a new, national centre of applied R&D expertise to promote and support innovative, fast-to-patient drug discovery in the UK through collaborative projects across the community.

We work with biopharma companies of all sizes, translational researchers, technology experts, patient groups and the contract research and finance sectors to help transform great ideas into commercial products and services for the wider health and wealth of the country.

By developing and validating new ways of discovering new medicines, and promoting key talent and expertise across sectors, we can help the UK maintain its heritage position as a global leader in this key industry.

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info@md.catapult.org.uk

Peter MacCallum Cancer Centre is Australia’s only public hospital solely dedicated to caring for people affected by cancer and is one of the world’s leading cancer research, education and treatment centres. We have over 2,500 staff, including more than 580 laboratory and clinical researchers. We aim to lead a new era of cancer prevention, care and discovery, supported by state-of-the-art facilities at our new home within the Victorian Comprehensive Cancer Centre building.

The Peter MacCallum Cancer Centre houses the largest group of laboratory-based cancer researchers in Australia working in close collaboration with multi-disciplinary teams comprising medical, surgical and radiation oncologists, nurses, radiation therapists and allied health professionals. We offer industry a range of opportunities for collaborative research and development across the spectrum from discovery through to clinical trials. Our laboratory scientists offer pre-clinical drug development expertise, with access to sophisticated animal models of cancer, cutting edge genomic facilities and a range of human tissue banks. Many of our laboratories have also pioneered new technologies in-house that are open to licensing and further development.

65+ years after our establishment, this sense of purpose and commitment to making life better for people affected by cancer continues at our centre today.

Contact Details:
Dr Shari Lofthouse, Head, IP & Business Development
shari.lofthouse@petermac.org
Cancer is a highly complex disease with more than 200 types, each caused by a different combination of genetic defects. Even among patients with a single type of cancer, such as breast or lung cancer, the genetic triggers vary from one person to the next. At The Institute of Cancer Research, London, we believe it is important to take into account those differences as we discover new cancer treatments – an approach known as personalised medicine.

Our scientific strategy focuses on three main areas, each designed to support the delivery of personalised cancer treatment:

- Our scientists work to identify the genes that cause cancer, and understand how a combination of genetics and the environment helps determine a person's cancer risk.

- We aim to understand the biology of tumours, by discovering the genes that drive cancers, and how tumours evolve within a person's body and shape their environment.

- We design new cancer treatments applying our biological knowledge about cancers to target their specific genetic weak points, and developing more focused radiotherapy to treat tumours more effectively while reducing side-effects.

The ICR works closely with our partner hospital, The Royal Marsden, in delivering our strategy, so we can take the results of our research as quickly as possible to patients in clinical trials. We also learn lessons from the clinical experience with new treatments, and adjust our research priorities accordingly.

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Central switchboard: +44 (0) 20 7352 8133

IRB Barcelona is a world-class research centre devoted to understanding fundamental questions about human health and disease. It was founded in October 2005 by the Government of Catalonia (Generalitat de Catalunya) and the University of Barcelona (UB), and is located at the Barcelona Science Park (Parc Científic de Barcelona). IRB Barcelona forms part of the Barcelona Institute of Science and Technology.

The Institute’s missions include conducting multidisciplinary research of excellence at the unique interface between biology, chemistry and medicine, providing high-level training in the biomedical sciences to staff, students and visitors, driving innovation through active technology transfer to the benefit of society, and actively participating in an open dialogue with the public through a series of engagement and education activities.

Exceptional scientific results deserve to be transferred to society. With this in mind, IRB Barcelona has devised a proactive strategy to ensure that the discoveries made in its labs are developed into products and technologies that serve the scientific and healthcare communities, as well as society at large.

Advised by an international Business Advisory Board, specialists from the Innovation Department work shoulder to shoulder with our researchers to identify results with commercial potential and to protect, develop and commercialize them, with the aim to establish strategic public-private sector collaborations, licensing agreements, and spin-off companies.

**Contact Details:**
Contact e-mail: innovation@irbbarcelona.org
The Centre for Genomic Regulation (CRG) is an international biomedical research institute of excellence, whose mission is to discover and advance knowledge for the benefit of society, public health and economic prosperity. The CRG believes that the medicine of the future depends on the groundbreaking science of today. This requires an interdisciplinary scientific team focused on understanding the complexity of life from the genome to the cell to a whole organism and its interaction with the environment, offering an integrated view of genetic diseases.

**Contact Details:**
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Cleveland Clinic was founded in 1921 on guiding principles of cooperation between diverse specialists to act as a unit. Since its founding, Cleveland Clinic has become one of the largest and most respected hospitals worldwide, with 4,435 beds and over 7 million total visits in 2016. Cleveland Clinic’s mission is to provide better care of the sick, investigation into their problems, and further education of those who serve. In addition to cutting-edge patient care facilities as well as surgical and medical experts, Cleveland Clinic has extensive innovations and translational research infrastructure. The Center for Clinical Genomics (CCG) was launched in 2015 under the direction of W.H. Wilson Tang, MD, with the mission to reduce the burden of disease by advancing clinical delivery of genomics and precision medicine. CCG empowers both patients and investigators by helping clinicians apply clinical genomics at the bedside and developing clinical use cases to expand knowledge in precision medicine across the institution. By partnering with information technology, laboratory medicine, basic science research, and medical genetics groups both within and outside Cleveland Clinic, CCG advances innovations in precision medicine for all patients.

**Contact Details:**
TANGW@ccf.org
Since its inception as a dedicated, comprehensive cancer center, then significantly supported by a naming gift from the Harcourt M. and Virginia W. Sylvester Foundation,

Sylvester Comprehensive Cancer Center (UM/Sylvester) has been the cancer brand for the University of Miami Leonard M. Miller School of Medicine. As the only university-based cancer center in South Florida, Sylvester has transformed cancer research and treatment in South Florida and beyond.

We seek to reduce the human burden from cancer and other serious illnesses through research, education, prevention, and the delivery of quality patient care.

Sylvester will become a fully integrated program of patient care, education, and research with an international reputation for excellence.

Sylvester will provide new hope for cancer patients in our extended community, which includes South Florida, the southeastern United States, the Caribbean, and South America.

Sylvester will promote efficient, community-responsive health care, and generate resources to sustain and enhance innovative cancer programs.

**Contact Details:**

(305) 243-1000  
Toll free: 800-545-2292
Affiliated companies & organisations – September 2017 -

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Abcam

As an innovator in reagents and tools, Abcam's purpose is to serve life science researchers globally to achieve their mission, faster. Providing the research and clinical communities with tools and scientific support, the Company offers highly validated biological binders and assays to address important targets in critical biological pathways.

Already a pioneer in data sharing and ecommerce in the life sciences, Abcam's ambition is to be the most influential company in life sciences by helping advance global understanding of biology and causes of disease, which, in turn, will drive new treatments and improved health. Two-thirds of the world's 750,000 life science researchers use Abcam's affinity binders, reagents, biomarkers and assays and the Company's products are mentioned in over 20,000 of the 56,000 peer-reviewed papers published each year in the life sciences.

By actively listening to and collaborating with researchers, the Company continuously advances its portfolio to address their needs. A transparent programme of customer reviews and datasheets, combined with an industry-leading validation initiative, gives researchers increased confidence in their results.

Abcam's twelve locations are in the world's leading life science research hubs, enabling local services and multi-language support. Founded in 1998 and headquartered in Cambridge, UK, the Company sells to more than 100 countries. Abcam was admitted to AIM in 2005 (AIM: ABC).

To find out more, please visit [www.abcam.com](http://www.abcam.com)

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Active Motif

Active Motif is the industry leader in developing and delivering innovative tools to enable epigenetics and gene regulation research. We are committed to providing the highest quality products along with superior service & support to the life science, clinical and pharmaceutical/drug discovery communities. Whether you are an expert in the field of epigenetics or a researcher interested in integrating epigenetics research into your studies, our comprehensive portfolio of experts will enable you to tackle your most difficult scientific challenges.

We provide:

- Innovative products for Chromatin Immunoprecipitation and DNA Methylation
- Epigenetic Services
- Antibodies for ChIP and ChIP-Seq
- Recombinant Proteins and substrates
- Multiplex Histone PTM Quantitation products and services
- Luciferase Reporter Assays

To find out more, please visit [www.activemotif.com](http://www.activemotif.com)

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Amgen is committed to unlocking the potential of biology for patients suffering from serious illnesses by discovering, developing, manufacturing and delivering innovative human therapeutics. This approach begins by using tools like advanced human genetics to unravel the complexities of disease and understand the fundamentals of human biology.

Amgen focuses on areas of high unmet medical need and leverages its expertise to strive for solutions that improve health outcomes and dramatically improve people’s lives. A biotechnology pioneer since 1980, Amgen has grown to be one of the world’s leading independent biotechnology companies, has reached millions of patients around the world and is developing a pipeline of medicines with breakaway potential.

For more information, visit www.amgen.com and follow us on www.twitter.com/amgen.

Agilent is a leader in life sciences, diagnostics and applied chemical markets. The company provides laboratories worldwide with instruments, services, consumables, applications and expertise, enabling customers to gain the insights they seek. Agilent’s expertise and trusted collaboration give them the highest confidence in our solutions.

The purpose of Agilent Research Laboratories is to power Agilent’s growth through breakthrough science and technology.

To complement our product line R&D, Agilent Labs looks beyond the evolution of current products and platforms to create the technologies that will underlie tomorrow’s breakthroughs, enabling Agilent customers to answer questions at the leading edge of life science, diagnostics and the applied markets. Our horizon is broad, encompassing synergies across Agilent and seeding new businesses to create competitive differentiation and compelling value for current and future customers and shareholders.

To accomplish these goals, we attract and retain top technical talent, collaborate extensively with global research leaders in academia, government and industry, and promote a culture of innovation and teamwork across our highly multi-disciplinary staff of life scientists, physical scientists and engineers. The majority of our research is located in the United States in Santa Clara, California, with additional locations in Europe and Asia.

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BioAscent

Founded in 2013, BioAscent is part of the BioCity group, and located within the BioCity Scotland site at Newhouse, Scotland. BioAscent operates a former MSD Automated Compound Management and Logistics facility. Our team comprise the highly skilled management, scientists and technicians who commissioned and managed the facility since its establishment in 2005.

The compound storage facility was initially responsible for delivering compounds to the Organon screening sites at Newhouse and Oss (The Netherlands), in addition to varied research partners.

The facility was dramatically expanded after acquisition by Schering Plough, to become the global repository site for the company. The facility successfully supported all drug discovery operations in Europe, the US, and Asia.

The Innovative Medicines Initiative (IMI) funded European Lead Factory (ELF) was launched on the 7th February 2013, with BioAscent selected as the “centre of excellence” for the Compound Management activities of the consortium, with responsibility for the collection, storage, distribution, HTS logistics (hit picking, support of follow-up studies) and inventory management of the Joint European Compound Collection.

We were able to recommission the facility and associated IT systems in order to deliver the compound management and logistics services for the European Lead Factory.

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Biomax

Biomax provides computational solutions that help clients to manage and use knowledge assets to improve decisions, develop innovative products and find solutions for complex problems.

Biomax Informatics AG was founded in 1997 as a spin out of the Max Plank Institute of Biochemistry (Martinsried, Germany) by Professor Hans-Werner Mewes, Dr. Klaus Heumann and Dr. Dmitrij Frishman. We are headquartered in Munich, with offices in London (UK), Vienna (Austria) and Madison, Wisconsin (USA). While Biomax has proven expertise across the life sciences, our solutions are increasingly applied to new areas by the most innovative organizations worldwide.

Our flagship product BioXM is a knowledge management system that can tie electronic medical records to NGS workflows, electronic lab notebooks and clinical studies along with patent information and give usable intelligence to the team. This is a small example of the diverse data types Biomax can work with. Databases are integrated by point-and-click, without additional programming. The semantic data model can be visualized using most commercial visual analytics tools.

In personalized medicine, Biomax has helped clinicians to achieve improvement of treatment outcomes with substantial and measurable reduction in treatment cost per patient. Besides patient stratification, Biomax software is being used in Systems and Synthetic Biology, Risk Assessment, and by any other team aiming to make sense of data stored in database ‘silos’ across their organisations.

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At BTG we are focused on bringing to market innovative products in specialist areas of medicine to better serve doctors and patients. Our growing portfolio of Interventional Medicine products is designed to advance the treatment of cancer, severe emphysema, severe blood clots and varicose veins, while our Specialty Pharmaceuticals portfolio offers antidotes that alleviate toxicity and treat rare conditions.

Healthcare is constantly evolving – so BTG never stands still. Inspired by a deep understanding of our customers’ needs, we’re working to meaningfully improve the lives of patients and their healthcare experience.

Our competitive advantage is our dedication to finding smart, often unconventional solutions to complex medical problems. Many of our products combine medicines, device technology and new techniques in order to deliver more targeted treatments. By providing more targeted therapies, treating the disease locally and minimising side effects, our minimally invasive procedures have the ability to improve patient care. We also invest in the clinical evidence to help demonstrate the value of our products to doctors, patients, and healthcare systems.

Whether developed in our own labs or in partnership with clinicians, academics and other companies, we believe passionately that medical innovation has the power to improve human health.

Cancer Research Technology (CRT) is dedicated to advancing discoveries to beat cancer. We develop and commercialise exciting new discoveries in cancer research, working closely with leading clinical and academic institutions, pharmaceutical companies and biotechs worldwide.

CRT is owned by Cancer Research UK (CRUK), the world’s largest charitable funder of cancer research and we are uniquely placed to capitalise on the connections of our parent organisation and work with the network of scientists that CRUK supports to translate their discoveries towards products and applications.

CRT also operates our own drug discovery laboratories - CRT-DL. CRT-DL translates cutting edge science into innovative new therapies for cancer patients and have pioneered an innovative operating model which brings together the best minds from industry and academia. They focus exclusively on establishing and prosecuting biologically-themed multi-project alliances with industry. CRT-DL currently has 5 major partnerships underway including alliances on the DNA Damage Response (Artios), Deubiquitinase enzymes (Forma Therapeutics), Cancer Metabolism (AstraZeneca) and with several new cancer biology themes/alliances being developed.

For more information on CRT visit www.cancertechnology.com

For more information on CRT Discovery Laboratories visit www.crtdiscoverylabs.com

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Cambridge Epigenetix’s mission is to harness the power of epigenetics to develop the next generation of diagnostics and therapeutics.

Spun out of the University of Cambridge in 2012, we are the industry-leaders in this cutting-edge area of precision medicine. Using our technologies, researchers have proven the link between epigenetics and disease.

Epigenetics has the potential to improve human health though the earlier diagnosis, improved patient stratification and the development of more effective, targeted therapies. Importantly, epigenetic modifications are also reversible, allowing physicians to identify and suggest lifestyle changes that are most beneficial for preventing disease.

Our innovative discovery platform is based on fundamental chemistry and enables the detection of novel epigenetic marks and signatures that can deliver next generation diagnostic tests and therapeutic targets.

We are currently applying our proprietary platform and know-how to biomarker and clinical assay development programmes for a number of diseases with significant unmet need — both internally and in partnership with leading biopharma companies. Together, we will make disease optional, not inevitable.

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Cell Guidance Systems develops technologies for regenerative medicine. Our primary focus is on the PODST technology. This generates co-crystals to provide sustained protein release. Proteins expressed using the PODST system are incorporated into polyhedrin crystals actually within the insect expression cell, during expression. These co-crystals protect the active cargo protein, providing it with sustained release. We are developing collaborations and partnerships with numerous groups to apply the technology to the treatment of a variety of diseases, including Osteoarthritis, Parkinson's disease and limbal stem cell disease. In addition, PODST technology has demonstrated significant potential for delivery of vaccines.

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Censo Biotechnologies Ltd is a stem cell technology company, with facilities in both Edinburgh and Cambridge, UK, providing human cells and contract research services for drug discovery in CNS, pain and neuro-inflammation, toxicity testing and cell banking.

We have the capabilities to source tissue samples from large cohorts of individuals, both healthy and patients with specific disease relevant mutations. From these tissue samples we generate induced pluripotent stem cells (iPSCs), using the potential of these cells to transform into a variety of different cell types, allowing the study and mimicking of disease. We can supply human iPSC for differentiation, assay ready samples of differentiated cells, or use our own capabilities to offer custom assay development and compound testing for our clients.

By developing human cell-based models of disease using cells from a diverse range of tissue donors, Censo supports the development of new targeted treatments for disease. Its major focus is the generation of novel data on drug efficacy and drug response variation for a given population.

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Discovery from Charles River is industry-proven in the successful development of novel therapies, with 300 patents and 65 candidates delivered to our sponsors in the past 16 years. Backed by more than 650 scientists, our comprehensive, integrated portfolio employs the latest technology and platforms to provide chemistry, biology, and pharmacology services that support clients from the earliest stages of hit identification all the way through to IND. Our client-focused, collaborative approach creates true partnerships that anticipate challenges, overcome obstacles, and move us forward together on the journey of getting new drugs to market.

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Cyclofluidic is using CyclOps™, its proprietary integrated drug discovery platform, to accelerate early stage discovery projects for its partners.

CyclOps™ connects chemistry, purification and biochemical assay with a powerful machine learning method to enable lead molecules to be assayed minutes, rather than weeks, after they are designed. Removing the latency between design and data, our unique approach puts molecular design, synthesis and screening and firmly back together in a single laboratory, allowing fast iterative exploration of large areas of diverse chemical space. Alongside the platform, Cyclofluidic expertise includes compound synthesis (flow and batch), analytical chemistry, assay design and optimisation making us an ideal partner for early stage small molecule hit-to-lead discovery projects.

Cyclofluidic has completed more than twenty-five projects with industrial and academic partners to deliver lead molecules through a unique combination of the CyclOps platform and a highly skilled, industry experienced team. CyclOps™ is the state-of-the-art lean approach to lead discovery, allowing us to optimise hits in real time, making and screening only those key compounds that progress a hypothesis towards the best possible lead molecules.

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Diagenode, based in Liège, Belgium and NJ, USA, is a leading global provider of complete solutions for epigenetics research, biological sample preparation, and diagnostics assays. The company has developed a comprehensive approach to gain new insights into epigenetics studies. The company offers innovative Bioruptor® shearing and IP-Star® automation instruments, reagent kits, and high quality antibodies to streamline DNA methylation, ChIP, and ChIP-seq, and RNA-seq workflows. The company’s latest innovations include a unique, full automation system, the industry’s most validated antibodies, the Megaruptor DNA shearing system for very long fragment sequencing, a ligation-free RNA-seq library prep method from 10 pg of inputs, and epigenetics assay services.

- Service options for ChIP-seq and DNA methylation
- Epigenetic biomarkers identification for drug discovery and therapeutic approach
- High-quality DNA and chromatin shearing devices
- Automation systems for ChIP, DNA methylation, and NGS library preparation
- Validated reagents and kits for epigenetics research including low input ChIP
- Ultra-validated ChIP (seq) antibodies that undergo stringent QC for guaranteed results

To find out more, please visit www.diagenode.com

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Definiens

We improve patient lives by unlocking the tissue phenome.

In oncology, therapeutic strategies have shifted from a direct assault on cancer cells to recruiting the immune system for that purpose. Our mission is to accelerate breakthroughs for this approach by helping scientists leverage Tissue Phenomics to deepen understanding of disease biology and immune system mechanisms, to bring multi-omics data into a cancer-relevant context, and to facilitate the translation of new insights into novel therapies and treatment strategies.

Definiens technology and services deliver outstanding biological insights to the immune activity landscape, this supports our clients to make key pipeline decisions and accelerate development. By creating a targeted biomarker signature based on an immuno profiling, we enable the potential to target specific cohorts and patient stratification based on response.

Our vision is to create unique patient profiles for an individualized standard of care, where patients experience fewer side effects and live longer.

Definiens’ Tissue Phenomics approach was awarded the 2013 Frost and Sullivan Company of the Year Award for Global Tissue Diagnostics and Pathology Imaging. For more information, please visit: www.definiens.com.

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DefiniGEN has world-leading expertise in the area of iPSC production and metabolic disease modelling. The company has a unique platform technology for generating phenotypically validated human cell disease models for a range of rare metabolic and liver diseases, to optimize preclinical drug discovery. DefiniGEN’s proprietary OptiDIFF platform generates terminally differentiated human cells of endodermal lineage from iPSCs. We provide iPSC-derived hepatocytes, pancreatic cells, cholangiocytes and intestinal organoids from healthy and diseased donors which closely resemble human primary cells. Off-the shelf products include:
- Alpha-1 antitrypsin deficiency
- Glycogen storage disease type 1a
- Familial hypercholesterolemia
- MODY3 Diabetes
- Neonatal Diabetes

The application of these technologies in drug discovery provides pharmaceutical companies with more predictive in vitro human cell products enabling safer and more effective treatments. This technology platform can be combined with cutting edge CRISPR gene-editing to produce a wide range of bespoke validated disease model cell products enabling pharmaceutical companies to effectively reprofile and reposition their drug libraries.

To find out more about our products and services, please visit: www.definigen.com

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Eagle Genomics’ award winning smart data management platform, the e[automated data scientist], allows scientists to bridge the gap between data and new insights in a rapid, systematic and traceable way. It is an AI augmented knowledge discovery platform putting state of the industry data science at the fingertips of biologists to radically reduce time and cost of research, thus enabling customers to achieve drastic productivity improvements and true data driven discovery.

Eagle Genomics are thought leaders in life sciences smart data management and analysis. Over the last decade, we have collaborated with a range of blue chip clients in the healthcare, personal care and agritech sectors, enabling them to deliver game changing products and technologies into their respective markets.

At Eagle Genomics, we innovate at the intersection of biology, data science and bioinformatics. We combine our knowledge in these fields with best in class enterprise software skills to achieve an audacious goal… to develop the enterprise information architecture for the genomics era.

We are also proud of our strong links to research and development in the critical emerging markets of human genomics and microbiomics. We are headquartered in the epicentre of genomic research at the Wellcome Campus in Cambridge and have close relationships with academics, hospitals and the European Bioinformatics Institute.

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Exonate Limited was founded in 2013 and is based in Nottingham and Cambridge in the UK. Exonate is a biopharmaceutical company focused on the discovery and development of small molecule drugs that modulate alternative mRNA splicing to address diseases of high unmet medical need. Alternative mRNA splicing is modulated in disease states resulting in pathological blood vessel formation (angiogenesis), which is a key driver of disease progression in ophthalmic conditions and cancer. Exonate’s founders have identified and generated a novel class of drugs, “SPHINXes,” that modulate alternative mRNA splicing. We have developed a pipeline of molecules that promise therapeutics for anti-angiogenic therapies in ophthalmology, oncology, analgesia, diabetes, neurodegeneration and renal disease.
Genedata Limited

Genedata is a leading provider of advanced software solutions for biopharmaceutical and biotech research and development. The firm’s United Kingdom subsidiary, Genedata Limited, is based in Duxford and is headed by Managing Director Kevin Teburi. Genedata transforms life science data into intelligence with a portfolio of advanced software solutions and scientific consulting. With award-winning platforms, combined with deep domain expertise, Genedata enables dramatic increases in productivity and quality of research, development, and production. Our precision medicine platforms are uniquely positioned in both translational and early clinical research settings.

Globally, Genedata partners with all 25 of the world’s 25 top pharma and biopharma companies as well as leading biotech organisations, and has built a strong portfolio of clients in the UK. Genedata Limited offers the full suite of enterprise software solutions and consulting services designed to maximize efficiency for clients in R&D as well as production, potentially saving millions of pounds and accelerating processes that traditionally require enormous investment of manpower and time to manage, process and analyse vast amounts of data. More information on Genedata software platforms and the types of data handled by these solutions is available at www.genedata.com.

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Healx

Healx is transforming drug discovery by using advanced machine learning and data analytics to repurpose drugs for rare diseases.

There are 7,000 identified rare diseases but only 400 have a pharmacological treatment available. De novo drug discovery for rare diseases not only faces the classical major challenges of the industry such as high attrition rate (with only 10% of drug candidates in phase II eventually approved) and rising time to market and development costs (they take an average of 14 years and at a cost of over $3b to reach the market) but also rare diseases challenges: there are no compelling incentives for the pharmaceutical industry to initiate drug discovery in this area.

New solutions are urgently needed for rare disease patients and Healx mission is to accelerate drug development for them. Drug repurposing offers many advantages over de novo drug development: they are safer, cheaper and faster to develop. They are also more likely to be approved (25% of repurposed candidates succeed from phase II to approval).

The accumulation of large volumes of data in the biological, chemical and clinical domains offers a new opportunity in our approach to drug repurposing. Using a battery of powerful algorithms developed from the University of Cambridge, Healx is able to query and make sense of this mass of data and establish new links between known drugs and rare diseases.
Horizon is a UK-based biotechnology company that combines a long scientific heritage in translational research with a world-leading gene-editing platform based upon homologous recombination. Horizon builds human disease models and reagents derived from genetically-engineered cells that can be used in your own lab or in-house by our scientists on your behalf. Our tools can be used to gain knowledge of the genetic drivers of disease, to develop novel drugs and companion diagnostics that predict patient response in the clinic.

Horizon offers products, research services and bioproduction capabilities built around its proprietary suite of gene editing tools able to alter almost any endogenous gene sequence of human or mammalian cell-lines.

We can provide:

- custom cell lines and in vivo models for research
- contract research and custom screening services including for target identification and validation, and for drug combination studies
- cell lines for bioproduction applications
- over 23,000 cell line catalogue products
- quantitative molecular reference standards

For more information visit www.horizondiscovery.com

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Linguamatics transforms unstructured big data into big insights to advance human health and wellbeing. A world leader in deploying innovative natural language processing (NLP)-based text mining for high-value knowledge discovery and decision support, Linguamatics’ solutions are used by top commercial, academic and government organizations, including 18 of the top 20 global pharmaceutical companies, and leading US healthcare organizations.

Linguamatics I2E is used to mine a wide variety of text resources, such as scientific literature, patents, Electronic Health Records (EHRs), clinical trials data, news feeds, social media and proprietary content. I2E can be deployed as an in-house enterprise system, or as Software-as-a-Service (SaaS) on the cloud.

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MedAnnex

A privately-owned, preclinical-stage biopharmaceutical company, MedAnnex was established in 2009 by serial life sciences entrepreneur Professor Chris Wood. Chris has founded, managed and successfully exited four biotechnology companies including Bioenvision Inc, which he grew from a small entrepreneurial venture to a leading biotech company (NASDAQ: BIV) with a market capitalisation of €315m. MedAnnex's lead product, a monoclonal antibody, annexuzlimab™, has shown significant activity and therapeutic potential in experimental models of rheumatoid arthritis, multiple sclerosis and systemic lupus erythematosus (SLE). Annexuzlimab modulates undesirable over-expression of annexin-A1 which is characteristic of chronic inflammation. MedAnnex is developing annexuzlimab as a novel agent for the treatment of complex autoimmune conditions and other T-cell mediated pathologies. MedAnnex is a member of the Scottish Life Sciences Association and has its headquarters in Edinburgh, Scotland.

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Metrion Biosciences

Metrion Biosciences is a UK-based Contract Research Organization (CRO) focussed on delivering a range of high quality ion channel drug discovery services. Metrion is located on Granta Park within the Cambridge bio cluster, one of Europe’s largest bioscience research hubs, and our state of the art laboratories house high quality facilities for culture and maintenance of native cell lines, engineered cell lines and induced pluripotent stem cells. We also have a comprehensive suite of automated patch clamp platforms, multiple manual patch clamp rigs, fluorescence-based screening capability and industry standard laboratory data management and reporting systems.

Our highly expert and dedicated team of ion channel specialists support a diverse range of ion channel drug discovery projects and modern safety assessment research for our clients. We combine in-depth target class knowledge and high-quality screening assays with experienced and collaborative project managers to keep your research focused upon meeting both objectives and timelines.

Metrion provides highly skilled electrophysiology screening services to support client medicinal chemistry optimisation programmes, CiPA compliant cardiac safety profiling assays, plus both neuroscience and translational assays. Working closely with our collaborators Assay.Works and Concept Life Sciences we also offer an expert integrated drug discovery service.

Metrion offers flexible business models according to the scope of each programme, with both fee-for-service and collaborative options available.

Further information regarding the full range of our capabilities can be found at www.metrionbiosciences.com.
ModiQuest

For over 10 years ModiQuest Research specializes in the generation of monoclonal antibodies against difficult targets for R&D, diagnostic and therapeutic applications. Using our proprietary ModiVac™ (hyper-immune stimulatory cell immunization), ModiFuse™ (electrofusion-based hybridoma generation), ModiSelect™ (antigen-specific B-cell selection) and ModiPhage™ (phage display) technologies, we can generate monoclonal antibodies from multiple species against virtually any target. Besides lead antibody generation, we also provide antibody engineering such as affinity maturation and humanization (ModiTune™), and production in transient and stable mammalian cell systems (ModiXpress™). All services are offered on a fee-for-service basis.

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Nemesis Bioscience

Nemesis Bioscience is a biopharmaceutical company developing Symbiotics© –DNA therapeutics administered before or with antibiotics to inactivate anti-microbial resistance in bacterial pathogens.

These Nemesis Symbiotics will make existing antibiotics work again, prevent the spread of resistance genes, and protect the efficacy of new antibiotics. The technology is applicable to all antibiotic classes, all known resistance mechanisms, and all bacteria. To deliver the Symbiotics, Transmids®, our novel vectors, are encapsidated in a bacteriophage coat. Transmids can also spread directly between bacteria by conjugation. Other applications include reduction of chemotherapeutic toxicity, inactivation of virulence factors, and in vivo synthesis of biofuels and therapeutics. Our current Symbiotics, use RNA-guided endonuclease technology to inactivate multiple beta-lactamase (bla) resistance genes –so resurrecting sensitivity to beta-lactams. We have validated the efficacy of both Transmid delivery routes and of consequent AMR inactivation in mouse models (i) therapeutically in a bacterial infection and (ii) also prophylactically inactivating AMR in the gut flora. Our multi-functional gene targeting systems may obviate the need for prior diagnostic screens for antibiotic resistance and used generally as a companion biological therapeutic together with well-established antibiotics for therapeutic treatment of infection as well as for prophylactic treatment to prevent the spread of AMR.

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O2h

O2h has developed an engine for seeding a pipeline of early stage life science assets through research collaborations, in-licensing, spin-outs and investment. We have the in-house capability to execute hit-lead-optimisation programs leading into patent and IND filing from our state-of-the-art biotechnology incubator with expertise in discovery chemistry, biology/pharmacology and the on-going project management of pre-clinical development.

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PhoreMost

Advances in genomics and basic research are providing a long list of clear causes that lie at the heart of a range of debilitating diseases. This has the potential to open the door to many new and improved targeted therapies. However, many of these newly characterized disease targets are intractable to current drug discovery technologies. PhoreMost is aiming to remove these barriers to new drug development with its novel SITESEEKER® technology, a live cell phenotypic assay system that can rapidly identify unexpected, or “cryptic” druggable sites in specific disease driving targets and pathways that can’t be readily seen using conventional non-cell based analytical methods.

The core of the SITESEEKER® platform is the search for new unexpected points of therapeutic intervention. The process consists of uncovering such nodes of disease-relevance and designing small-molecule drugs for them. This is achieved using a diverse and highly complex (ca. 108) library of small 3-dimensional protein-fragment shapes, which interact with intracellular proteins to describe new druggable sites. This approach, called Protein Interference (PROTEINi) differs fundamentally from other genome-based target screening technologies, such as RNAi and CRISPR, in operating directly at the protein level, so that new druggable space can be defined as an inherent part of the target-function screening process.

For more information: http://www.phoremost.com

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Promega

Promega is a global biotechnology company offering over 3,500 tools and technologies in the life sciences. Here, the values of science, business and human well-being intersect, cultivating a supportive environment for employees, customers and community.

Researchers, technicians and analysts in academic, industrial and government settings worldwide rely on innovative solutions and technical support provided by Promega to advance knowledge in the fields of genomics, protein analysis and expression, cellular analysis, drug discovery, human identification, and applied biotechnology.

Founded in 1978, the company is headquartered in Madison, WI, USA with branches in 16 countries and over 50 global distributors serving 100 countries.

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Proximagen

Proximagen and its predecessor companies have a long heritage in discovery and development of novel small molecule therapeutics, in particular in the areas of CNS, pain and inflammation.

Proximagen’s focus is on the discovery of novel small molecule therapeutics for diseases of the central nervous system (CNS), pain and inflammation and related areas. Located on the Babraham Research Campus in Cambridge, U.K., Proximagen’s integrated drug discovery and early development unit has in-house capabilities in all core drug discovery disciplines with small teams enabling close interaction and rapid decision-making. Our teams have a long and successful legacy in small molecule drug discovery with experience in:

- Medicinal chemistry
- Biology
- Drug metabolism and pharmacokinetics (DMPK)
- Early stage Development

Proximagen has a strong track record of collaborations and our commitment to partnerships continues Our current collaborations include a partnership with Roche where we are collaborating to conduct further Phase II trials on a VAP-1 inhibitor discovered by the team at Proximagen.

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Quid is a San Francisco based technology company that focuses on unstructured data analytics. Quid's proprietary platform analyses the world's text information and is being used by business leaders across the world to generate data-driven insights to guide strategic decisions. Quid has been recognized as one of the World Economic Forum’s Technology Pioneers and was listed as one of the top 50 most disrupted companies in the world in 2017.

Quid’s healthcare team has developed a strong presence in the pharmaceutical and biotech markets by helping senior leaders better understand their patients, physicians, and competitors. Market leading healthcare companies have leveraged Quid’s solution to generate insights across various business functions such as R&D, marketing, competitor intelligence, and business development.

For more information on Quid, please visit our website: www.quid.com

Learn more about our work on our blog: https://quid.com/feed

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Repositive is connecting the world of genomic data: We help research organisations find the data they need to power their research and help data providers making their data collections visible and accessible to their target audience.

Our public platform at http://repositive.io presents the largest ever global search portal specialised in genomic datasets. Our users benefit from browsing the contents of worldwide data sources with a single search, discuss data utility, form research collaborations and request data from the research community. Better visibility and accessibility of human genomic data, combined with a collaborative community can power research, aid faster and better diagnosis of diseases and the development of precision medicine and treatments.

Repositive offers data access services to biopharmaceutical organisations, for example with our Patient-Derived Xenograft (PDX) Consortium which we initiated together with AstraZeneca. Our PDX consortium now includes global biopharma companies like Boehringer Ingelheim and PDX-providing CROs like Horizon Discovery. Together we are creating a pre-competitive resource to speed up pre-clinical oncology research.

For more information visit: https://repositive.io
Macrocycles are an emerging class of drugs that can address the challenging interactions and extended binding sites traditionally targeted by biologics. However, unlike protein therapeutics, they are both orally available and able to penetrate biological membranes. They are being developed as new therapeutics for the inhibition of protein-protein interactions (PPIs), such as are present in many diseases including cancer, auto-immune and inflammatory conditions. There are believed to be at least 300,000 different PPIs occurring in human biology.

Ripptide Pharma has developed a chemoenzymatic technology for the generation of novel and diverse macrocycles. Our technology uses a set of enzymes, originally isolated from marine micro-organisms, but engineered to increase activity and to have a broad substrate specificity. Our biotransformation tool-kit comprises enzymes capable of cyclising linear substrates, introducing heterocycles into the ring, and further tailoring of the macrocycle through oxidation and prenylation. Ring sizes of 4 – 12 residues are possible. The scope is almost unlimited, and we have so far shown the incorporation of L-, D- and unnatural amino acids, N-methyl amides, non-peptidic groups such as triazoles, alkyl chains, sugars, glycols and aminobenzoate. Each of these can be introduced into the linear substrate using standard peptide coupling with bespoke reagents. Having heterocycles and/or N-methyl amides in the final macrocycle has been shown to increase cell permeability.

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Selcia is a leading global CRO offering the full range of drug discovery services; integrated drug discovery, assay development, medicinal chemistry and ADME, with expertise in macrocycles, natural products and their simplification and optimisation for drug use. Selcia has a unique peptidyl prolyl isomerase (PPIase) inhibitor screening platform.

Selcia Radiolabelling specializes in 14C GMP radiolabelled API for clinical trials and in 14C custom radiolabelling for DMPK, dermal penetration and environmental fate studies.

To support regulatory submissions, Selcia offers GLP analysis; including GLP NMR (500MHz), mass spectrometry, HPLC (analytical and preparative) and specialised purification capabilities from analytical (µg) to multi-100g scale.
Sphere Fluidics (SF) is an established Life Sciences/Medtech Tools company that is focussing on developing proprietary single cell analysis systems for therapeutic discovery. The Company is commercialising a range of novel instruments and consumables (microfluidic Cyto-Cartridges® and specialist chemicals and bioreagents) that, within several hours, can automatically screen millions of single cells for secreted protein (e.g. antibody) production in miniaturised aqueous compartments called picodroplets (i.e. compartments of picolitre volume). Sphere Fluidics has recently developed and launched the world’s first integrated bench-top platform (the Cyto-Mine® system) that performs high-throughput single cell analysis and monoclonality assurance. The system is protected by 31 patents (23 are granted) and 16 trademarks. The picodroplets are assayed for single cells that secrete valuable protein (e.g. antibodies) and imaged (for monoclonality assurance) during subsequent dispensing of “hits” into individual wells of microtiter plates. Cyto-Mine® is enabling and reduces costs dramatically for Biopharmaceutical Discovery and Cell Line Development. The platform is also now being extended to automatically perform high-throughput single cell engineering using Synthetic Biology or Genome Editing techniques. Sphere Fluidics has 21 employees based in labs and offices in Babraham (Cambridgeshire, UK) and Trenton (New Jersey, USA) and has signed multiple international distributorships. The Company’s technology has had about £25 million invested in its development and Sphere Fluidics is a revenue-generating business.

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Storm Therapeutics is a spin out of the Gurdon Institute at the University of Cambridge, created to commercialise the work of founders Professors Tony Kouzarides and Eric Miska in RNA epigenetics.

Several large families of RNA-modifying enzymes have been identified that impact key biological processes by changing the activity of RNA through catalysing epigenetic RNA modifications. Storm is working at the forefront of this new field, collaborating closely with our scientific founders and their research groups at the Gurdon Institute to elucidate the functional role of diverse RNA modifications.

Advances in the understanding of RNA modification and its role in the development of cancer offer the prospect of identifying novel therapeutic targets. Using cutting-edge techniques such as CRISPR screens, chemical biology, RNA-Seq and RNA mass spectrometry, we have established a unique target discovery platform for the identification of small molecule modulators of RNA modification pathways.

Since inception in May 2015, Storm has raised £12.6m in seed and Series A funding. The company uses the proceeds to establish a pipeline of drug discovery programmes to develop novel, first-in-class drugs for the treatment of specific cancers with high unmet medical need.

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Strem Chemicals UK has had a presence in the UK and Ireland for over twenty-five years distributing Strem Inc’s specialty chemicals of high purity in a timely fashion. Strem have a history of assisting in marketing new products from within the research community, its aim is to bring new and interesting products to the research field as quickly as possible.

The pharmaceutical industry can rely on key product lines such as catalysts, ligands (especially phosphines), organometallics and N-heterocyclic carbenes for organic synthesis. More recently Strem has introduced a growing range of nano materials.

Strem Chemicals UK Ltd offers:

- High Purity Materials
- High quality products and service;
- Trustworthy procedures and documentation, including description of chemical’s colour and form.

For more information visit www.strem.co.uk

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TCRS

Niche CRO providing a full range of clinical development services to pharma, biotech and academia.

The UK is a global centre of research excellence with world class hospitals, scientists and research clinicians. However, at times, the bureaucratic process associated with running clinical trials in the UK can be frustrating. TCRS offers our sponsors the ability to expertly navigate around these challenges.

Because TCRS was created by a team of senior hospital researchers, we have an in-depth understanding of NHS systems, processes and people.

We offer a highly developed network of contacts in the UK healthcare system and work closely with hospitals to ensure they have the facilities, personnel and infrastructure to deliver your requirements in full. This makes us uniquely qualified to select and manage the very best UK hospital sites for your study.

Our hands-on experience of the NHS infrastructure means we can deliver ultra-rapid study start-up by expertly managing the regulatory, ethics and hospital approval process.

A supporting team of highly experienced CRAs and Project Managers ensures that every aspect of your project is delivered in an efficient and timely manner. Whether you are seeking full support for every aspect of your clinical development programme or just for one part of a study, TCRS offers flexibility, momentum and value.

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Ubiquigent offers a range of tools, services and integrated programmes to enable and supports its client’s ubiquitin system targeted drug discovery projects. The targeting of ubiquitin system family proteins has been recognised as offering significant new opportunities across a range of therapeutic areas. Of particular interest is the deubiquitylase (DUB) enzyme sub-family but Ubiquigent also provides a range of tools and services addressing other members of the ubiquitin system.

Recently Ubiquigent launched its first novel hit-finding compound library DUBtarget™-001 addressing the DUB enzyme family and earlier this year was awarded an Innovate UK grant to develop next generation libraries. Coupled with its industry standard DUBprofiler™ enzyme screening and selectivity profiling service, Ubiquigent is now uniquely placed to enable and support its client’s DUB targeted drug discovery programme ambitions.

Ubiquitylation, like phosphorylation, describes a reversible post-translational protein modification. Ubiquitylation may control the protein substrate’s destiny or its signalling functionality. It is a process that refers to the covalent attachment of a small protein called ubiquitin to the epsilon-amino group of a lysine residue or the N-terminal methionine residing within a substrate protein – which may also be another ubiquitin molecule. This results in either mono- or poly-ubiquitylation of the substrate target protein. The structure of the resulting ubiquitin chain determines how ubiquitylation regulates protein degradation, coordinates cellular localisation, activates or inactivates proteins or can modulate protein-protein interactions.

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