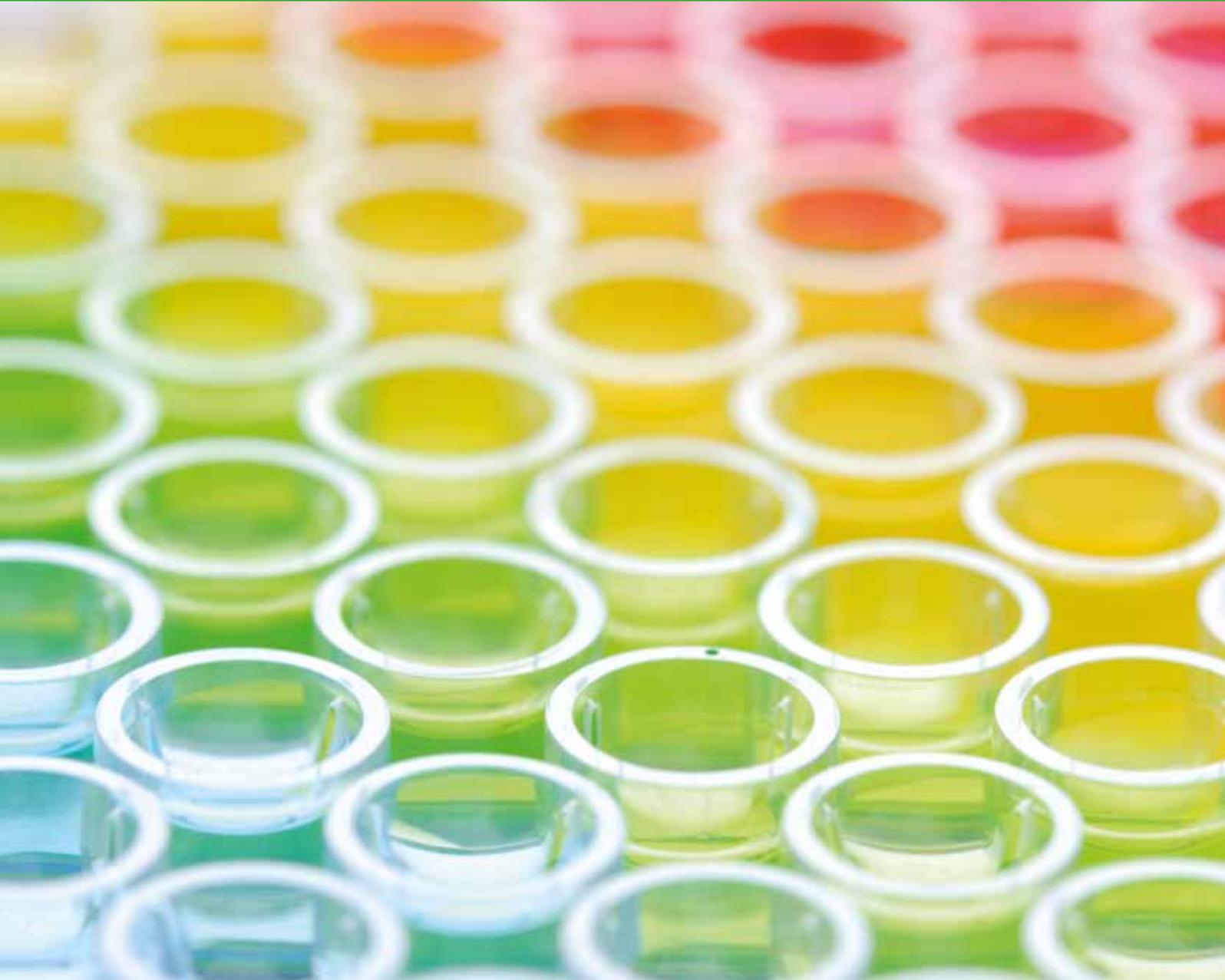


# TRANSLATING NOVEL SCIENCE TO PATIENT BENEFIT



[www.ucl.ac.uk/translational-research](http://www.ucl.ac.uk/translational-research)

## Impact Objectives

- Progress collaborative, interdisciplinary science and biomedical research discoveries along the development pathway to patient benefit
- Offer academics assistance with: defining development and regulatory strategy; building and managing links to available resources; funder liaising and reporting requirements; managing risks and issues; follow-on funding; and on-going project management and support
- Bridge the gap between academia and the commercial healthcare sector by de-risking UCL's novel science

# Translating novel science to patient benefit

*Dr Jane Kinghorn discusses how University College London (UCL)'s Translational Research Office (TRO) is working to encourage, facilitate and contribute to the translation of UCL's and its partner hospitals' research*



**Could you introduce yourself and your background?**

I am the Director of University College London's (UCL's) School of Life and Medical Sciences Translational Research Office (TRO), a role I took on from the previous director in 2016. Following my PhD, I went on to spend the next 16 years at GlaxoSmithKline in various roles, gaining a full understanding of the drug development process, particularly focused in the areas of neurology and inflammation. In 2009, I joined UCL to use my professional skills to complement those of the academics to drive forward their projects along a development path. One year later, together with two other colleagues, we formed the TRO, which now encompasses 20 people organised into three groups, a Translational Research Group, a Drug Discovery Group and a Business and Innovation Group.

**How do you work to make projects more appealing to translational funders?**

Demonstrating that a project has a clear and feasible regulatory and development path is critical to give the funders and investors confidence in the projects they are supporting. We use the joint expertise of the team, gained both in an industry setting and now from many successful projects at UCL, to ensure the project is planned, costed and articulated effectively.

**From your point of view, how has the relationship between industry and academia developed over the past decade?**

Arriving in academia from large pharma nine years ago, I felt very much like a fish out of water. Despite the aims of both being very similar in trying to deliver new solutions to improve patients' lives, the culture, philosophies and motivators were very different, leading to a certain mistrust and uncertainty of the benefits of working together. This situation has radically changed and the TRO's industry and academic expertise has significantly contributed to bridging the cultural divide.

The pharma industry has reorganised, cutting back on internal research to free-up resource to fund more open innovation and collaborative science. The government has also stimulated interest in translation and industry partnership through providing appropriate funding schemes to incentivise academics to participate more fully. There are many models of such partnerships in operation, with real potential to accelerate novel therapies to patients. The one thing they all have in common is mutually agreed goals reliant on harnessing the strengths from both parties – a true collaboration based on trust.

**How do you see the TRO progressing in the coming five to 10 years?**

The vision of the TRO is to encourage,

facilitate and contribute to all aspects of the translation of UCL's and its partner hospitals' life science and biomedical research discoveries along a development pathway towards clinical application and patient benefit. The era of big data and AI is upon us and is set to have a tremendous impact in diagnostics and therapeutic development, which we are well positioned to deliver. How we achieve this will evolve as we continue to learn through our achievements, and improve and adapt to the changes and challenges ahead.

The TRO will play an active leadership role in helping UCL, our NIHR Biomedical Research Centres (BRCs) and industry partners to continue to mature strategies to influence policies by forging new partnerships with regulators, NICE, NHS commissioners, the Office of Life Sciences and Industry associations such as MMIP, BIA and ABPI.

We anticipate a greater pull through to adoption of our novel therapies by working in partnership with our Academic Health Science Centre UCL Partners and with the Accelerated Access Review framework. In addition, working closely with our funders and their networks, we will play a leading role in sharing best practice on how to accelerate translation, overcoming bottlenecks and enhancing collaborations aimed at delivering greater impact to our patients. ●

# Bridging the gap

*The Translational Research Office (TRO) at University College London (UCL), UK, bridges the gap between academia and industry, helping Principal Investigators (PIs) to bring their research to fruition*

At University College London (UCL) in the UK, the School of Life and Medical Sciences Translational Research Office (TRO) is working to translate the research of UCL and its partner hospitals through their NIHR Biomedical Research Centres (BRCs). The TRO is directed by Dr Jane Kinghorn, who is responsible for a team of 20 applied scientists and business developers. The team is striving to fulfil UCL and its BRC's mission of: 'accelerating translation for health and wealth'. As such, TRO supports the development of early stage projects that are attractive for translational funding or further development with partner organisations (large and small).

## TRANSLATIONAL RESEARCH GROUP (TRG)

The TRG currently consists of a team of seven headed up by Dr Pamela Tranter (formerly of Novartis). 'As experienced project leaders we look to establish credibility with our academics, help them scope the projects' translational critical path, assist with internal and external networking to link to resources required, access funding and provide research project management services,' Tranter explains. 'The portfolio of funded translational milestone projects that we actively project manage has grown significantly since the beginning of the group, from three in 2010 to over 78 supported to date, with a cumulative value of £113million.'

The current active portfolio of 47 projects covers all modalities, with a particular emphasis on Advanced Therapies (cell and gene therapies) and Devices, which reflects the expertise at UCL and our BRCs. These projects cover all therapeutic

areas, including oncology, neuroscience, ophthalmology, rare diseases and range from pre-clinical studies to Phase II clinical trials. One example of the type of work undertaken by the TRG is the 'Development and clinical testing of a therapeutic antibody for a novel angiogenic target in wAMD' project, headed up by Professors John Greenwood and Stephen Moss. Neovascularisation and vascular remodelling are key clinical features of several pathologies, including neovascular age-related macular degeneration (AMD), diabetic retinopathy (DR) and solid tumours.

The project, which has been funded by the MRC, is to develop a humanised function-blocking monoclonal antibody to GMP and to test within a phase II trial. As well as helping to secure significant funding from the MRC (£7m), the TRG have advanced the project through managing all of the outsourced activities using their expertise in process development, manufacturing (GMP), toxicology (GLP) and regulatory requirements.

## DRUG DISCOVERY GROUP (DDG)

The DDG is responsible for progressing small molecule drug discovery based on UCL's novel targets for further investment. The group comprises a team of six 'drug hunters' with experience of working at the interface between academic and commercial science and is led by Dr Richard Angell (formerly GSK, Arrow Tx, AZ). 'We have successfully built a pipeline of small molecule projects by de-risking UCL and our partner hospital's science at every stage, which is now attracting significant interest from large pharma and investors,' Angell explains.

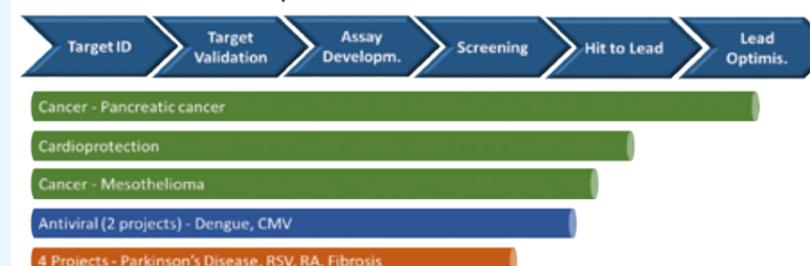
The DDG are skilled at converting fundamental scientific discoveries into small molecule drug discovery projects. The team build industry standard biochemical and cellular assays, carry out screening (including HT screening), as well as computational, medicinal and synthetic chemistry. They work closely with the originating academic (where the more complex cellular assays and animal models are generally run) and CROs, who generate pharmacokinetic data (what the body does to the drug) and biophysical screening data (how the drug interacts with the target) for projects as needed. Excellent links to the wider drug discovery community have benefitted several projects where advice and support from the community (pharma, biotech, investors and funders) has sharpened drug discovery questions and enabled the generation of data beyond local capabilities.

Currently, the team are running a portfolio of three lead projects with two back-up projects and a further four projects in early set-up (See Figure). The DDG provide small molecule drug discovery advice to all interested parties across UCL and its BRCs and seek to leverage their knowledge and contacts to help wherever they can.

## BUSINESS AND INNOVATION GROUP (B&IG)

The B&IG focus on the development of innovative approaches for the creation and management of strategic partnerships with industry and attract the kind of resources that only such external organisations can provide. Headed by Dr Maryam Atakhorrami (formerly Phillips), the team of four commercially experienced business developers work closely with academics and clinicians to map internal research capabilities and strengths across UCL and its partner BRCs through our Therapeutic Innovation Networks (TINs – all modalities including digital/AI). Through this activity they are able to identify potential opportunities for new strategic collaborations with a range of industry partners. The B&IG have helped to set up and maintain more than five long-term strategic alliances in addition have over 100 interactions with SMEs and large ►

## Small Molecule Pipeline





companies in the pharma, medtech, biotech and digitech sectors. Since its inception, the B&IG has helped attract over £40 million from industrial collaborations.

One of the flagship alliances for the B&IG and UCL is the relationship with Eisai, which has been operating for the last six years and is set to be renewed for a further five. The collaboration aims to develop UCL novel targets in the area of neurodegeneration and to work together to accelerate the discovery and development of disease-modifying therapies (small molecule and biologics). This is achieving significant results, with a number of projects progressing through multiple milestones.

‘The mix of expertise that currently resides within the B&IG and the wider TRO has grown in response to the changing external environment,’ Atakhorrami explains. ‘The pharma industry has significantly reorganised, cutting back on internal research to free-up resource to fund more open innovation and collaborative science. The Eisai/UCL collaboration is an excellent example of harnessing the capabilities of two organisations to truly accelerate drug development for patient benefit.’

#### CLOSE TIES

Collaboration has been key to the TRO’s success, not least its work with the National Institute for Health Research (NIHR)-funded Biomedical Research Centres (BRCs) at UCLH, Great Ormond Street and Moorfields Hospitals. ‘This has helped accelerate access to patients, ensuring their voice is heard at the earliest stage of development, through to greater recruitment into clinical trials and providing first class facilities to perform experimental medicine studies,’ Kinghorn highlights.

‘We have some of the best characterised cohorts of patients in the country and first

class clinical trials’ capability to facilitate proof of concept studies in humans,’ Kinghorn explains. ‘The depth of clinical engagement and culture of interdisciplinary collaboration and enterprise that comes through these partnerships is pivotal to translation at UCL. In addition to enabling and accelerating the progression path for emerging innovations to enter clinical evaluation, it informs and influences exploratory biomedical research at UCL, particularly in the discovery of potential targets for therapeutic intervention.’

Through the numerous connections it has established, the TRO expects to play a leading role in sharing best practice on how to accelerate research translation. This is exemplified through an EU-funded Teaming Grant that aims to establish a new Discoveries Research Centre (association of five leading Portuguese universities)\*. The UCL TRO, as the advanced partner, are advising the centre on how to accelerate their regenerative medicine therapeutics by establishing a Discoveries Translational Office.

The TRO has a breadth of knowledge to assist academia in developing their therapies, as Kinghorn explains: ‘I describe the TRO as “in-house consultants”, because the mix of skills, experience and networks gained from working in R&D in different organisations complements those of the academics supporting them to progress their project,’ she explains.

This is the first article in a series of three highlighting some of the translational infrastructure available to researchers at UCL, their BRCs and potential industry partners. The second (next issue) will explore how they organise to accelerate therapeutic translation and the third will focus on educating the next generation of translational scientists. ●

## Project Insights

### FUNDING

**TRO personnel are funded through a variety of mechanisms including:**

The School of Life & Medical Sciences • The NIHR UCLH Biomedical Research Centre • Wellcome Trust through their Institutional Strategic Support Fund • Academic Health Science Centre (UCL Partners) • Individual translational awards (see below)

**Individual translational project grants funded by:**

MRC • WT • NIHR • Innovate • Industry • Department of Health • EU Horizon 20:20 EU Discoveries Centre\* • UCL Technology Fund • NIHR Biomedical Research Centres (BRC) affiliated with UCL\*\*

*\*This project has received funding from the European Union’s Horizon 2020 research and innovation programme, under grant agreement No 739572.*

*\*\* Supported by researchers at the National Institute for Health Research University College London Hospitals Biomedical Research Centre, Great Ormond Street Hospital Biomedical Research Centre and Moorfields Hospital Biomedical Research Centre.*

### COLLABORATORS

**Translational Collaborators at UCL:** UCL Business • Research Services • Joint Research Office • Institute of Clinical Trials and Methodology • Clinical Research Facilities, Biomedical Research Centres (University College London Hospitals, Great Ormond Street Hospital, Moorfields Hospital) • UCL Partners • Innovation & Enterprise

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