Genomic Innovation:

Developing technologies and people to deliver translational impact 1993-2023

Introduction

Our work impacts science and society in numerous ways, including: publications, dissemination of knowledge, sharing tools and protocols, influencing policy and training genomic scientists. The Genomic Innovation team adds to these capabilities by helping to develop and apply our research in the form of new products or services. Our strategy includes developing technologies and people to deliver innovation, and embodies the following principles:

- Viewing innovation as a natural extension of our core science, and as a route to impact.
- Positioning translational activity as an opportunity for interested scientists, not a target.
- Defining impact broadly, by considering all the benefit that can be derived from our science:
    - On society – by working with industry partners and investors to apply our research to solve real world challenges.
    - On scientists – by providing opportunities to translate their research and make a difference, and by providing alternative career pathways.
    - On science – by using commercial routes when appropriate to efficiently disseminate our tools and technologies and therefore enable the research of others.

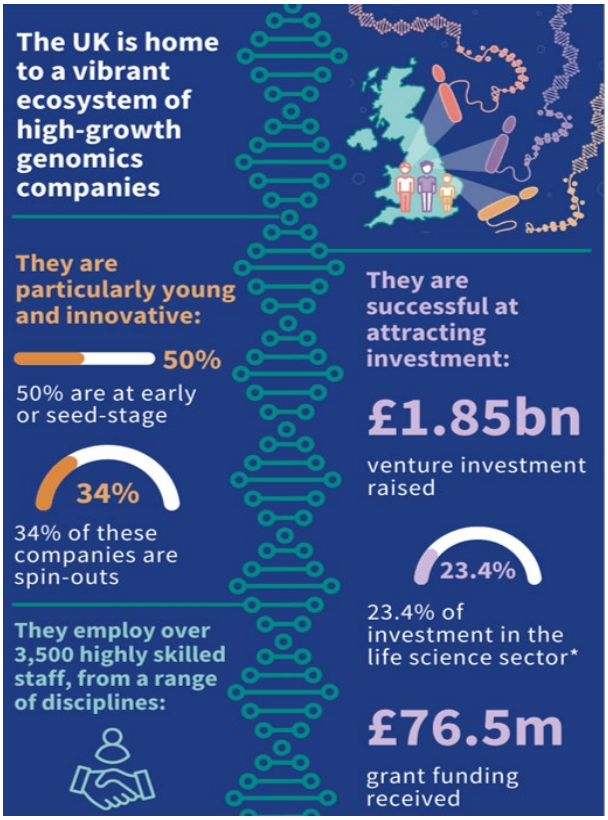
The innovation team works with our scientists to help select the most appropriate method to translate genomics and biodata and derive the maximum impact from a technology or resource developed at the Institute, including:

- Formation of spin-outs.
- Licensing of intellectual property (IP).
- Collaboration with commercial partners.

The UK context

The UK is a world leader in the development and application of genomics. It attracts more investment and is home to more genomics companies than any other country in Europe. This growing industrial ecosystem stems from early and visionary charitable investment in genomics that established foundational resources in the UK, and with it, capabilities to sequence, analyse and share the first human genome.

Thirty years on, the Sanger Institute still has a central part to play in this maturing ecosystem by undertaking research at a scale unavailable to most other institutions, By doing so, we are able to reveal the genetic factors and cellular processes that influence health and the development of disease, contributing insights that can change medicine.



Spinouts

An important measure of the relevance and impact of our research over the last thirteen years is provided by the new businesses that have been successfully spun out from the Sanger Institute, having raised £320.5M in investment in total. Below we highlight these companies and the health products and services they are developing and delivering.

Kymab – founded 2010

Kymab generates antibody-based treatments and vaccines from mice engineered to carry human antibodies, a technology developed at the Sanger Institute. Kymab has developed the Kymouse® platform, which has enabled the creation of a portfolio of therapeutic assets.

The first of Kymab's antibodies, KY1005, targets OX40L a molecule linked to several immune and inflammatory diseases, including atopic dermatitis, and the rejection of transplanted organs. Recently, KY1005 completed a Phase 2a trial studying moderate to severe atopic dermatitis patients whose disease is inadequately controlled with topical corticosteroids. The trial demonstrated that it has broad potential therapeutic application in multiple diseases caused by immune dysregulation, and its mechanism-of-action means that it could be applicable to a range of autoimmune and inflammatory diseases.

A second monoclonal antibody, KY1044, has been shown to promote a significant and long-lasting anti-tumour effect as a monotherapy or in combination with anti-PD-L1 checkpoint inhibitor. KY1044 has

progressed to early Phase 1/2 trial in development against solid tumours.

Kymab was the first biotech in the UK to achieve “unicorn” status when it was valued at over US $1 billion. In 2021, Kymab was acquired by Sanofi for £1.2 billion.

Congenica – founded 2014

Congenica is a world-leading digital health company that develops software and solutions to analyse and interpret genomic data to deliver automated analysis, diagnosis, and treatment solutions. Congenica was originally based on our Deciphering Developmental Disorders (DDD) study, which showed the value of using genomic technologies to improve the diagnosis of rare paediatric disease.

Congenica’s clinical decision software supports healthcare professionals globally in their interpretation of genomic data from whole genomes, exomes, or gene panels. In addition, Congenica is now developing HAPPY (Healthy Ageing Pharmacogenetics and Polypharmacy), a clinical decision software that uses genetic data to reduce adverse drug reactions. HAPPY will alert doctors to the risks of prescribing specific combinations of medicines for patients over 50 years old, enable them to investigate less harmful options.

In 2018, Congenica’s clinical decision support platform was selected as a provider of Diagnostic Decision Support Services by Genomics England to deliver the NHS Genomic Medicine Service. It has helped the NHS to increase its genetic diagnostic yield by 50%, while improving analysis times 20-fold. Using Congenica’s scalable processing pipeline enables the NHS to process over 200 genomes a day.

In 2022, the genomic interpretation software received the CE mark under the In Vitro Diagnostics Directive (98/79/EC), enabling it to transition from research to clinical use as a medical device in the UK and EU. The platform is the only CE marked in vitro diagnostic software based on genomic data. It can be fully integrated with existing medical records and laboratory management systems and is now used in over 25 countries.

Microbiotica – founded 2016

Microbiotica is a diagnostic and therapeutic company founded in 2016 to exploit the microbiome analysis platform and bacterial culturing capabilities developed at the Institute.

Microbiotica’s most advanced pipeline assets are two live bacterial therapeutic compositions, one developed for Ulcerative Colitis with a Phase1b trial is planned to start in 2024.

The second is an adjuvant for immunotherapy. The gut microbiome plays a key role in determining which cancer patients respond to Immune Checkpoint Inhibitor (ICI) therapy. Microbiotica identified the first microbiome signature that improved response to ICI in melanoma and developed a live bacterial therapeutic made up of nine key species from the signature. When used as a co-therapy with immunotherapy it showed potent anti-tumour efficacy in mice.

Mosaic Therapeutics – founded 2022

Mosaic Therapeutics applies a decade of groundbreaking work in our Translational Cancer Genomics Laboratory to identify selective vulnerabilities in cancer cells that can be exploited therapeutically. With initial seed investment from Innovate UK, MOSAIC is developing a world-leading target identification and prioritisation capability based upon patient-derived WGS-characterised cellular models, synthetic lethal CRISPR screens, and cutting-edge analytics. The company leverages Sanger’s expertise in generating and analysing large datasets from the manipulation of tumour models to identify new approaches to targeting difficult to treat cancers.

Conclusion

Developing both technologies and people to derive maximum benefit from our science is key to our innovation strategy. The Institute has a culture and history geared towards scaling technologies, and when suitable capabilities developed through our discovery research reach a critical mass, we have leveraged them to spin out companies that make a positive impact in the healthcare sector.

The 2022 **Bioindustry Association report** highlighted that 34% (41/121) of genomics companies with active high growth that are currently headquartered in the UK, are spinouts, compared to just 2.7% across all sectors in the same timeframe. These companies have raised a substantial amount of venture investment. However, the majority (61%) of the high growth companies have benefitted from public funding.

Our success in creating spinout companies based on our science highlights the Institute's commitment to translating research into applications that are advancing healthcare through genomics.

Genomic innovation at the Sanger Institute takes many forms, from improving a technology or a protocol, all the way through to the development of new platforms and therapeutics which become the cornerstones of biotech companies. Translating science at the Sanger Institute is about global impact and closing the gaps in technologies that will continue to drive improvements with the goal of maximising societal benefit. The spinouts provide one route of applying research to benefit society, and we will continue to be mission-driven in commercialising research in the pursuit of the greater good.