



A Framework for Industry Engagement

GENOMICS ENTERPRISES PROSPECTUS

Introduction

Genomics Enterprises recognises that pharma, biotech and diagnostic company engagement with the 100,000 Genomes Project will be critical for the success of the programme. This industry engagement will be the most effective way of accelerating the development of new diagnostics and treatments. When aligned to an NHS that is well prepared for a future of genomic medicine, this will undoubtedly result in better care and improved outcomes for patients – not just in the UK but across the world. This Prospectus outlines the proposed approach for achieving this goal.

Background

In December 2012, the UK Prime Minister announced a programme of whole genome sequencing as part of the UK Government's Life Sciences Strategy. The principal objective of the Project is to sequence 100,000 whole human genomes from patients with cancer and rare disorders and to link the sequence data to a standardised, extensible account of diagnosis, treatment, and outcomes.

By the end of 2017, the major legacies of the Project will be:

- Advanced genomic medicine integrated into the NHS enabling the UK to lead the world in this field
- Accelerated insights and understanding of the genetic basis of rare diseases and cancer leading to improved diagnostics and precision medicines that benefit NHS patients
- Thriving private sector investment and commercial activity in genomics
- An increased public knowledge and support for genomic medicine and science

To this end, the UK Department of Health has established Genomics England as a wholly owned, limited company. Genomics Enterprises is the arm of Genomics England responsible for engaging and managing the relationship with commercial organisations.

On September 30th and October 10th, Genomics Enterprises held meetings in Cambridge, Massachusetts and London, UK respectively with twenty of the largest pharmaceutical companies in the world. Other meetings have also been held with individual biotech companies and venture funds. The purpose of each of these meetings was to obtain feedback from industry on what the 100,000 Genomes Project would need to deliver in order to be perceived to be of value by industry. This Prospectus is a response to the feedback that we received.

The Genomics Enterprises Proposed Value to Industry

The following propositions are based on industry requirements, as expressed in the workshops and various meetings. These are put forward as a series of value propositions from Genomics Enterprises to industry. They are intended as areas to be developed and explored in partnership with industry and relevant stakeholders – and should not be taken, at this stage, as guarantees:

- Genomics Enterprises will provide pharma, biotech and diagnostic companies with the ability to access whole human genomic sequences and to link these sequences to longitudinal patient data (medical records and outcomes) on a scale that has not been undertaken anywhere else in the world¹
- Genomics Enterprises will provide access to the latest bioinformatics tools and capabilities developed internally at Genomics England and in partnership with Bioinformatics Platform Partners. This will involve a range of services, tools and processes involved in the interpretation of genomic data for clinical purposes from sequencing through to diagnosis and reporting
- Industry will be able to recruit and follow-up with patients through their physicians based in NHS Genomic Medicine Centres. Access will be provided to specific patient cohorts and this could dramatically speed up recruitment for clinical trials. Requests could be made to further sequence certain patients where considered appropriate
- In working with Genomics Enterprises, industry will have access to a streamlined Institutional Review Board (IRB) and ethics approval process
- Genomics Enterprises will manage an appropriate industry interface to the Genomics England Clinical Interpretation Partnerships (GeCIP) involving a network of researchers and clinical (NHS) disease experts focused on analysing and constantly refining the clinical interpretation of the 100,000 Genomes dataset. GeCIP is aiming to create a thriving, sustainable research community to support a future where advanced genomic medicine becomes part of everyday clinical practice
- Through Genomics Enterprises, industry will also have the opportunity to link to other UK data repositories and initiatives as available and appropriate e.g. we will explore the potential to link to UK Biobank with their data and samples from 500,000 people across the UK
- Industry will have an opportunity to work together with key decision makers from across the UK healthcare marketplace, including regulators, payers and the clinicians who ultimately will look to use new diagnostics and therapeutics to deliver better care and improved outcomes for patients

¹ A key feature of the Genomics England programme is that individual level data will not be “released” but will instead be analysed within a secure, monitored environment akin to a reading library (where books cannot be taken away but must be read in situ in a monitored environment). Access to this environment will be granted only for specific, approved purposes.

The above benefits have a potential impact across the breadth of the pharma, biotech and diagnostics business - from the discovery of new targets in research; to accelerating clinical development; through to assessing the health impact/outcomes associated with pre-launch and in-market products. We would look to jointly develop specific industry use cases / success stories around these areas to demonstrate the impact and value of industry engagement with the 100,000 Genomes Project.

What makes the Genomics Enterprises Value Proposition Unique?

Whilst there are other gene sequencing initiatives currently ongoing or under consideration around the world, none of these offers the breadth and scope available with the 100,000 Genomes Project. The unique features include:

- The ability to link sequence data to longitudinal patient records from across primary, secondary and tertiary care for the whole of the diverse population of a country
- Engagement with a single healthcare system the size of the NHS with its depth of expertise and coordinated approach to care
- The opportunity to engage with national regulators and payers who are open to discussing the new approaches that will be required in a world of genomic medicine
- A healthcare system that is already preparing itself through huge investment in training specialists and creating systems to support a future of personalised medicine, health and care

Our aim is to make the UK one of, if not the best place for industry to engage and learn the lessons needed to prepare itself for the appropriate business models of the future. In return, the UK is aiming to demonstrate intellectual leadership in genomics, to secure clinical gain for NHS patients and to encourage inward investment into the UK.

A Strategy for Action

As a core part of the future strategy, Genomics Enterprises is proposing the following:

- To scope out, manage and deliver an Industry Trial
- To establish an industry Genomics Expert Network for Enterprises (GENE) Consortium to manage the Industry Trial

Industry Trial

Genomics Enterprises is proposing to run an Industry Trial during 2015, open to all pharma, biotech and diagnostics companies prepared to invest monetary and FTE resources to understand how best to realise the value from working with Genomics England, our Bioinformatics Platform Partners and the wider NHS.

Through the Trial, the specifics of industry engagement will be worked through in practice and key questions related to each of the benefits outlined above – and indeed other questions e.g. related to IP and ownership - will be answered. Additionally, the Industry Trial will aim to identify the use cases of highest potential value that will be utilised to inform future commercial options.

GENE Consortium

To enable the smooth running of the Industry Trial and to ensure that industry is able to steer it in the way that it considers most appropriate, Genomics Enterprises is also proposing to establish a GENE Consortium. This Consortium will involve a cross-industry group of participating companies working collaboratively with Genomics Enterprises and Bioinformatics Platform Partners to determine the questions to be asked, to work through the processes to uncover the answers and to share the learnings amongst Consortium members. This is an opportunity for the participating members of the Consortium to set the agenda and manage the working environment as they would like to see it best working for them. This will involve active participation on a day-to-day basis from each company. As part of this process a virtual collaborative model will be supplemented with in-person meetings held at regular intervals. This model could also evolve into a permanent physical network of linked centres of expertise. In effect, the GENE Consortium will be the means by which industry creates an appropriate management interface to the 100,000 Genomes Project and to the UK payer, regulator and clinical practice expertise of the NHS as they prepare for a future of genomic medicine.

Industry Trial Deliverables

Participating companies will have full access to the output from the Industry Trial through active participation in the GENE Consortium. The Industry Trial will be scoped to ensure it is of sufficient size to be able to answer the key questions of relevance to industry and give an insight into viable future commercial models. A suggested trial cohort involving a selection of 5000 whole genome sequences across both cancer and rare disease patients is proposed. Genomics Enterprises will develop a services offering alongside the core data asset coming from Genomics England to enable the following:

- Access to whole genome sequences linked to relevant phenotype data through an appropriate technology platform
- Access to the bioinformatics expertise available from Genomics England and our Bioinformatics Platform Partners
- Clarity on the process for patient recruitment and follow-up through Genomic Medical Centre physicians with insight into specifics e.g. around speed of response to requests
- Engagement in the streamlined IRB and ethics approval process to understand exactly how it works
- Creation of an appropriate industry interface to GeCIP with agreement on an operating model
- Agreement on which of the other data repositories and initiatives would constitute useful interfaces and how this will work in practice
- Confirmation of the relevant relationships and approaches with key decision makers across the UK healthcare marketplace to explore e.g. the regulation of new targeted treatments

The Business Model

Industry Trial

Our proposal is to charge pharma, biotech and diagnostics companies a fee of £250,000 per company to become a member of the GENE Consortium for 2015. In order to ensure active participation in the GENE Consortium we are also asking each company to commit at least one named FTE team member to the programme.

The agenda for this first year will be focused on creating and managing the Consortium itself and ensuring the delivery of the services associated with the Industry Trial. We see the contribution as an option payment on the unique set of deliverables that will give an insight into the long-term commercial value of the

100,000 Genomes Project.

Direct Engagement

We appreciate that smaller pharma, biotech and diagnostics companies (defined as those with a market capitalization below one billion dollars) will have a far more focused agenda. Thus our proposal for these companies is to allow access to a narrower range of services to be agreed further to Direct Engagement with Genomics Enterprises. Our proposed fee for such services will start at £25,000 for 2015 and an evaluation of the success of the approach will run alongside an evaluation of the Industry Trial and will be used to inform future commercial options. In return for a lower access fee we want to make clear that products developed from information gained from access to Genomics England data will be subject to a royalty fee.

The approaches outlined above will be the foundation upon which we hope to build a mutually beneficial and sustainable commercial model for the future. At a minimum, our aim is to use the output from the Industry Trial and Direct Engagement to clarify the range of commercial options for 2016-17. Those companies that engage early and contribute most will not only help to create the models of the future but will also be offered early and preferential terms in accessing them.

