A new narrative on value frameworks and partnership models

A final proposal to BIVDA

CRA International (UK) Limited ("CRA") is pleased to have the opportunity to present this draft proposal to the British In Vitro Diagnostics Association (BIVDA) on the UK genomics landscape and access to genomics technologies in the UK. Specifically, the objective would be to:

- Articulate and evidence the value framework for advanced in vitro diagnostics
- How new partnership models can facilitate improved patient access and the key enablers

Background

Over the last 10 years, the UK has developed a policy framework to encourage the development of advanced diagnostics. This included the delivery of the 100,000 Genomes Project in 2018, the development of 2020 strategy paper by Genome UK on the future of healthcare, which outlined a 10-year vision with 27 priority actions and most recently the UK government published its Genome UK: 2021 to 2022 implementation plan, setting out the steps it expects to take to build upon the UK's position as a global leader in genomic healthcare. The plan contained five high-priority actions[1] with priority actions for the financial year 2021 to 2022:[2]

- Data diversity: Genomics England will work to ensure datasets are more diverse and represent people from a wide range of ethnic backgrounds so that everyone can benefit equally from genomic healthcare. Particularly, improving the diversity of genomic data, addressing the historic under-representation of data from ethnic minority groups in genomic datasets, which results in health inequalities
- Whole genome sequencing for rare diseases and cancer: This means tests are being made available to patients for the first time through the NHS Genomic Medicine Service. The ambition is to sequence 500,000 genomes in the NHS and 500,000 in UK Biobank, creating the most advanced genomic healthcare system in the world
- Faster diagnosis and treatment of cancer: Genomics England will work with the NHS to find ways to improve the speed and accuracy of genomic testing for cancer. This includes integrating multiple data sources and new technologies to support faster and more comprehensive genomic testing for cancer in line with the NHS Long Term Plan.
- Our Future Health: This project (previously called Accelerating Detection of Disease) will begin recruiting up to five million people for the UK's largest-ever research programme. In 2021, Our

1 https://www.genomicseducation.hee.nhs.uk/blog/genome-uk-the-first-steps-in-a-grand-plan/

2 https://www.gov.uk/government/publications/genome-uk-2021-to-2022-implementation-plan/genome-uk-2021-to-2022-implementation-plan

Future Health will pilot participant recruitment processes to build towards their 5 million participant ambition.

- Data sharing: -The UK will develop global standards and policies for secure sharing of genomic and related health data, so that patients can trust their information is secure but can also benefit research. The National Institute for Health Research, Medical Research Council and Wellcome Trust will work on this over the next five years. This includes providing funding to the Global Alliance for Genomics and Health (GA4GH) to develop standards and policies for sharing genomic and related health data

The UK Genomics plan has been integrated into the UK Life Sciences Vision, attempting to join up with overall UK approach on supporting the life sciences industry. The UK has therefore made substantial progress compared to other European countries, with policy recognition and a strategic vision intended to create the most advanced genomic healthcare system in the world, delivering better health outcomes at lower cost, and the value of advanced diagnostics accepted in clinical practice. However, a strategy only delivers to patients if it is implemented and there are still considerable challenges regarding patient access to advanced diagnostics and a need for defined metrics that can be used to monitor the progress on the implementation.

The CRA proposal

The CRA proposal would have four key steps

- Step 1: A project kick-off
- Step 2: Develop a value framework
- Step 3: Assessing new partnership models
- Step 4: The development of a new industry narrative

Step 1: A project kick-off

The first step of the project will be a project kick-off meeting. Prior to this it would be useful to review any existing materials/research developed by BIVDA or the companies involved in the project steering group. The objective of the kick-off will be to:

- Agree the appropriate focus on national markets. We have discussed a focus on gathering information relevant to the UK, but with a focus on England given the size of the population.
- The definition of the value framework and a strawman structure
- Breadth of definition of partnership models
- A first discussion on case studies
- Agree timeline and key deliverables

This would be scheduled for the first weeks of the project.

Step 2: Develop a narrative on the appropriate value framework to ensure patient access

The purpose of this step is to set out the evidence on the benefits of genomic testing and the full value that these deliver to society and barriers that prevent these materialising.

We would develop UK case studies illustrating the benefits of the revolution in diagnostics testing and showing the magnitude of these benefits to society. This would include the significant benefits directly

delivered to patient's prognosis and treatment, and improving efficiency of the healthcare system and the broader value in terms of understanding diseases and preparedness through surveillance of infectious disease pathogens at scale. Beyond healthcare, it would be useful to illustrate the contribution to the economy indirectly through improved patient lives, and directly in terms of a vibrant, highly R&D intensive industry which Europe should be nurturing. For example, case studies could include how profiling provides invaluable information to physicians that can help them determine the best possible treatment for each patient, and even map their treatment journey to identify future courses of action should the disease progress.

In each element of the value framework we would set out the barriers to these materialising and the danger that the UK misses out on the economic and societal benefits this could deliver.[3] As the "advanced diagnostics" landscape is evolving, many genomic tests are facing hurdles before they can effectively reach the market and even once on the market diffusion and usage is restricted. One of these is the lack of funding and reimbursement which inevitably tends to inhibit access.[4]

The approach would be undertake:

- A literature review capturing the existing evidence for clinical benefits, but also at the benefits beyond those directly related to the patient [5]
- Interviews with BIVDA experts on the access challenges facing their technologies and how these have changed over the last few years. An option would be to include a small number of external interviews at this stage.
- A review of potential policy solutions that have been employed in other European markets and identification of high impact areas
- Development of metrics that show progress toward strategic objectives

The aim of the value framework is a tool to prioritise the key policy areas, - with a focus on high impact changes – and the development of associated metrics that will delivered increased utilisation of genomic technologies backed up by evidence. This would take 8 weeks to develop.

Step 3: Develop a narrative on new partnership models

Given the nature of genomic testing and the wide potential benefits, it is an ideal area for partnership models between innovators, regulators, and payers. The UK has a track record in the development of partnership models between individual companies, Genomics England, leading health providers and universities. For example, recent partnership models include:

- Genomics England and Illumina negotiated an agreement to deliver up to 300,000 whole genome equivalents over the next five years, with an option to increase to 500,000. Samples will be provided through the NHS Genomic Medicine Service and the network of seven genomic laboratory hubs across England, which were established in 2018.[6]

3 Aronson, S. J., & Rehm, H. L. (2015). Building the foundation for genomics in precision medicine. Nature, 526(7573), 336-342.

4 Vozikis, A., Cooper, D. N., Mitropoulou, C., Kambouris, M. E., Brand, A., Dolzan, V., ... & Macek Jr, M. (2016). Test pricing and reimbursement in genomic medicine: towards a general strategy. Public Health Genomics, 19(6), 352-363.

5 https://blogs.deloitte.co.uk/health/2022/02/how-genomics-is-transforming-healthcare-in-the-uk.html

6 https://www.genomicsengland.co.uk/news/genomics-england-and-illumina-sequence-whole-genomes-for-nhs-gms

- Oxford University partnered with Oracle to launch the Global Pathogen Analysis System, or GPAS, a digital genomic sequencing platform to analyse and compare different COVID-19 variants and highlight those that may pose an elevated threat to the population. This platform is intended to help scientists and governments make faster, more informed public health decisions and mitigate the risk of potentially dangerous variants. There are already plans to expand its capabilities to include other pathogens.[7]
- Guardant Health and The Royal Marsden NHS Foundation Trust agreed a partnership to establish the first liquid biopsy testing service based in the United Kingdom. This increased accessibility to Guardant Health's liquid biopsy testing will enable clinicians to personalize treatments more accurately for patients with late-stage cancer.[8]

However, there is no taxonomy of types of model used in the UK, agreement on the key enablers or an overarching framework on how best to develop partnership models in the future.

The approach would be to undertake:

- A catalogue of the different partnership models based on secondary research and any evidence of the benefits these deliver
- Interview companies involved in the development of the partnership, the most significant barriers and how these were overcome.
- Develop best practices on partnership development that delivers for patients

The objective will be to develop a description of the types of partnership model and how these help to deliver patient access. This would be developed by type of partnership model (horizon scanning, infrastructure, data sharing etc), with an explanation of how these can improve patient access and the implication for the policy framework to encourage these types of partnership.

The deliverable would a taxonomy on types of partnership model, the key enablers, and policies to support their use in the UK. Assuming 4-6 partnerships, this would take 3-4 weeks. This can over in parallel with Step 2.

Step 4: The development of materials to support a new industry narrative

The final step would be to develop materials that BIVDA could use to disseminate the developed narrative. These could take a number of different forms:

- A short (~10 page) document describing the narrative and key asks
- Appendix with case studies
- Two infographic describing the value framework and the benefits of different types of partnership model
- Supporting talking points

7 https://www.healthcareitnews.com/news/emea/uk-launches-implementation-plan-genomic-healthcare-system

8 https://investors.guardanthealth.com/press-releases/press-releases/2021/Guardant-Health-and-The-Royal-Marsden-NHS-Foundation-Trust-Announce-Partnership-to-Establish-First-Guardant-Health-Liquid-Biopsy-Testing-Service-Based-in-the-United-Kingdom/default.aspx

Project timeline and deliverables

The project would be completed over a three month period. After each step there would be a progress meeting to discuss the results and next steps.

The output of the project would be evidence-based analysis to characterise the benefits of advanced diagnostics to payers and healthcare systems, with clear policy recommendations for how to develop this ecosystem in the UK. The final deliverable would be a short 10-20 pages written paper. This would be delivering in word format along with corresponding slides.

Why Charles River Associates

Charles River Associates is an economic consultancy that specialises in public policy issues in the life sciences industry. CRA has undertaken many assessments of challenges facing particular healthcare technologies and the case for policy reform. CRA focuses on delivering high quality, robust analysis but in a compelling fashion that is accessible to the target audience. CRA has worked for the industry (through EFPIA, PhRMA, IFPMA, EUCOPE, EuropeaBio, Advamed), national trade associations, and individual companies on a wide range of issues over the last 15 years. The project team would include:

- Tim Wilsdon is a Vice President in CRA's London office. He is responsible for public policy related work on a global basis. He has led many projects for EFPIA, PhRMA, IFPMA and EuropaBio including assessing the economic footprint of the biotech industry, impact of health technology assessment, the experience of EUnetHTA, the future of managed entry agreements and a series of projects for PhRMA on the value of medicines and the role of innovation.
- Ryan Lawlor, an experienced project manager, who has been involved in a series of projects for EFPIA/EBE, PhRMA, IFPMA and EuropaBio including detailed study for the benefits of personalised medicines to patients, society and healthcare systems.
- They would be supported by 2-3 consultants in our London and Brussels offices. CRA consultants typically have 3-4 years consulting experience on policy projects and an advanced degree in economics or the natural sciences.

CRA has undertaken many projects for companies advising them on the development, market access consideration for transformative medicines, companion diagnostics, and complementary diagnostics. In terms of published public policy projects:

- *Fostering a European Advanced Diagnostics Ecosystem.* CRA developed a white paper for the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), describing the barriers, opportunities, and requirements for success. This review the evidence on the value of "advanced diagnostics" and the barriers that impede the development and uptake of advanced diagnostic technologies, and considered the need for an ecosystem that fosters the development of commercial advanced diagnostics and their uptake in the clinical setting.
- *The benefits of personalised medicines to patients, society and healthcare systems*: CRA conducted an evidence-based analysis to characterise the benefits of personalised medicine (PM) to patients, society and healthcare systems; and identify barriers and enablers to the development and adoption of PM in Europe. The report proposes policy recommendations for decision-makers to overcome these barriers and incentivise the development and adoption of PM in Europe.
- *Supporting the development of a manuscript on "Unlocking Access to Broad Molecular Profiling: Benefits, Barriers and Policy Solutions"* which develops a set of policy proposals that aim to

improve equitable patient access to genomics testing will need to consider new funding models, health technology assessment processes that capture both patient and systemic benefits, and appropriate regulatory standards to determine quality of genomic profiling tests.

- Publications on *innovative and adaptive strategies for new drug development* – CRA has released a number of publications on emerging challenges faced Gene & cell therapy including
    - How are cell and gene therapies changing drug development models?[9]
    - Building New Business Models to Support Cell and Gene Therapy R&D[10]
    - New approaches to market access and reimbursement for gene and cell therapies[11]

9 Colasante W, Diesel P, Gerlovin L (2018) How Are Cell And Gene Therapies Changing Drug Development Models? Cell & Gene.com, accessible at https://www.cellandgene.com/doc/how-are-cell-and-gene-therapies-changing-drug-development-models-0001

10 Colasante W, Gerlovin L (2018) Building New Business Models To Support Cell And Gene Therapy R&D, Cell & Gene.com accessible at https://www.cellandgene.com/doc/building-new-business-models-to-support-cell-and-gene-therapy-r-d-0002

11 Colasante W, Diesel P, Gerlovin L (2018) New Approaches To Market Access And Reimbursement For Gene And Cell Therapies, Cell & Gene.com accessible at https://www.cellandgene.com/doc/new-approaches-to-market-access-and-reimbursement-for-gene-and-cell-therapies-0001