Horizon Scanning Series
The Future of Precision Medicine in Australia

International actions, alliances and initiatives

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i. Global Alliance for Genomics and Health

The Global Alliance for Genomics and Health (GA4GH) was established in 2013 to accelerate progress in human health through responsible sharing of genomic and clinical data (Global Alliance for Genomics and Health 2017a). GA4GH is comprised of four Working Groups that address key aspects of genomics: Clinical, data, security, and regulatory and ethics. GA4GH members, including organisational members from 45 different countries, identify the issues addressed by the Working Groups. Australia has 27 organisational members, including CSIRO, Australian Genomics Health Alliance, and Murdoch Children's Research Institute.

Each of the Working Groups has a series of work products, including a catalogue of global genomics activities, policy documents, and frameworks for sharing data. Additionally, the Working Groups run several demonstration projects (Global Alliance for Genomics and Health 2017b), including:

- Beacon Project: an open internet service that gauges the willingness of international groups to share genetic data. This project is being implemented on the websites of leading genomic organisations.
- BRCA Challenge: combines information on sequence variation, phenotype, and scientific evidence on BRCA genetic variants from around the world, with the aim of increasing understanding of breast cancer and other cancers.
- Cancer Gene Trust: an online network that brings together somatic cancer genomic and clinical data from medical institutions worldwide, and makes the data publically accessible for cancer research.
- Matchmaker Exchange: a collaborative project between a number of genomics groups that is working towards a federated platform to facilitate matching of cases with similar phenotypic and genotypic profiles (Philippakis Azzariti, D.R., Beltran, S., Brookes, A.J., Brownstein, C.A., Brudno, M., Brunner, H.G., Buske, O.J., Carey, K., Doll, C., Dumitriu, S., Dyke, S.O.M., den Dunnen, J.T., Firth, H.V., Gibbs, R.A., Girdea, M., Gonzalez, M., Haendel, M.A., Hamosh 2015).

ii. Europe

International Consortium for Personalised Medicine

The International Consortium for Personalised Medicine (ICPerMed) began in 2016 as a result of several workshops run by the European Commission, and is funded by the European Union's Horizon 2020 Research and Innovation Program (ICPerMed 2017). It is a platform of over 30 European and international organisations created to encourage collaboration in personalised medicine research, funding, and implementation. The high level of participation of personalised medicine groups allows ICPerMEd to map the work taking place throughout Europe, which enables them to develop framework for infrastructure, resources, and regulatory procedures to facilitate the development of personalised medicine.

The ICPerMed Action Plan was published in March 2017 and identifies 22 research activities and 8 research-supporting activities that are ready to be implemented at national, European, and international scales (Aaviksoo Ballensiefen, W., Bauer, H., Bußhoff, U., Frenzel, M., Guglielmi, G., Andersen, P.H., Julkowska, D., Kuhlmann, K., Molnar, M.J., O'Driscoll, M. & ICPerMed 2017). This forms the basis of the work programme for ICPerMed members and stakeholders to 2020.

Genomic Medicine Alliance

The Genomic Medicine Alliance is an international network of genomic medicine researchers that was established in 2014. It is a platform for collaboration between academics, researchers, regulators, and members of the public interested in genomics and personalised medicine, with the aim of advancing genomic medicine (Genomic Medicine Alliance 2017).

In addition to encouraging collaboration, The Genomic Medicine Alliance facilitates the introduction of genomics technologies into mainstream medicine, produces genomics best practice guidelines and recommendations, and develops educational activities throughout Europe. Their key outputs to date include a genomics summer school, workshops, and publication of genomic research in their official scientific journal *Public Health Genomics*.

European Personalised Medicine Association

The European Personalised Medicine Association (EPEMED) was founded in 2009 to accelerate adoption of personalised medicine in Europe by providing recommendations on regulations and reimbursement, promoting the creation and application of advanced diagnostic tests, and designing education and training programs for personalised medicine stakeholders (European Alliance for Personalised Medicine 2017).

To deliver on these objectives, EPEMED have produced white papers, public fora, research studies, and activities led by subcommittees that focus on regulatory, economic, and educational issues associated with personalised medicine in Europe. In April 2017, EPEMED announced it will direct its remaining funds towards funding personalised medicine research through scholarships/fellowships and grants over five years, rather than continuing to produce high-level recommendations and programs (The European Personalised Medicine Association 2017).

Biobanking and BioMolecular Resources Research Infrastructure European Research Infrastructure Consortium

Biobanking and BioMolecular Resources Research Infrastructure - European Research Infrastructure Consortium (BBMRI-ERIC) is a European network of distributed personalised medicine infrastructure. BBMRI was established in 2008 with funding for three years from the European Commission. In December 2013, BBMRI was awarded ERIC status to facilitate collaboration of biobanks and biomolecular resources into a pan-European collaborative facility (BBMRI-ERIC 2017). BBMRI-ERIC is increasing access to quality biobank and biomolecular resources in order to facilitate high quality precision medicine research throughout Europe.

BBMRI-ERIC members consist of Austria, Belgium, Czech Republic, Estonia, Finland, France, Germany, Greece, Italy, Latvia, Malta, the Netherlands, Norway, Poland, Sweden, and the United Kingdom. In mid-2017 there were 1,379 biobanks or biomolecular resource centres throughout Europe listed in the BBMRI-ERIC directory (Holub Swertz, M., Reihs, R., van Enckevort, D., Müller, H., Litton, J-E. 2016).

European Alliance for Personalised Medicine

The European Alliance for Personalised Medicine (EAPM) was established in 2012 with the aim of improving public health research and the regulatory environment for personalised medicine. Their key actions include running educational events for personalised medicine stakeholders, summer school for health care professionals, and publishing guidelines and recommendation documents (European Alliance for Personalised Medicine 2017).

iii. Asia

GenomeAsia 100K

GenomeAsia 100K is a non-profit organisation that aims to create reference genomes and identify key alleles for the Asian population. The initiative is hosted at Nanyang Technological University in Singapore, with support from founding partners Macrogen and MedGenome. The first stage of the project aims to sequence 10,000 people to generate reference genomes from all major Asian ethnic groups. This will be followed by sequencing of 90,000 additional individuals. Genomic data will be combined with clinical, microbiome, and phenotype information to allow greater analysis of disease causation. The initiative was announced in 2016, with an aim to complete the database by 2020 (Genome Asia 100K 2017).

China Precision Medicine Initiative

Announced in March 2016, the China Precision Medicine Initiative is a US$9.2 billion 15-year initiative run by the Chinese Academy of Sciences to fund Chinese precision medicine research. The initial project will involve collection of genetic data from 2,000 individuals and will be carried out by a cross-disciplinary team coordinated by the Beijing Institute of Genomics (Russell 2016).

Indian Department of Biotechnology Human Genetics and Genome Analysis Program

The Indian Department of Biotechnology (DBT) is driving genomics research and engagement in India through their Human Genetics and Genome Analysis Program (Department of Biotechnology, 2017). In 2013, the Indian Government, through DBT, commenced its five-year plan to promote human genomic and genetic research. Their strategy involves acquisition of genomic technologies, the creation of five new genomic research centres, and establishment of technology transfer organisations and incubators to commercialise new genomic technologies (Padma 2016). Their major outputs to date include establishment of 21 genetic diagnosis and counselling units, implementation of a consortium project at several Indian medical research institutes, and the formation of three advisory taskforces:

- Task Force on Genome Engineering Technologies and their Applications
- Task Force on Human Genetics and Genome Analysis
- Priority areas of Human Genetics and Genome Analysis Task Force.

The DBT also established the National Institute of Biomedical Genomics (NIBG) in 2010, which was the first institution in India solely devoted to biomedical genomics. NIBG aims to accelerate genomic medicine by conducting and communicating leading genomics research, establishing world-leading infrastructure, and facilitating faster uptake of novel genomic technologies through greater operational understanding. Their key outputs include workshops, conferences, policy documents, and publications in scientific journals (National Institute of Biomedical Genomics 2017).

iv. Africa

Human Heredity and Health in Africa Initiative

The concept for the Human Heredity and Health in Africa (H3 Africa) Initiative was developed during a Frontiers Meeting of the African Society of Human Genetics, the US National Institutes of Health, and the Wellcome Trust in 2009, which identified the need for a large-scale research program in Africa. H3 Africa was announced in 2010 and is supported by the National Institutes of Health and the Wellcome Trust. The aim of the initiative is to enhance understanding of disease susceptibility and drug responses in African populations through innovative genomics research in a pan-continental network of laboratories.

H3 Africa manages biospecimen collection from 22 African countries through its Biorepository Program at three biorepositories in Uganda, Nigeria, and South Africa. The data from these specimens is available for genomics research. In addition to this program, H3 Africa runs personalised medicine training events, and produces guidelines and policy documents (Human Heredity and Health in Africa 2013).

v. United Kingdom

Innovate UK Precision Medicine and Catapult

The Catapult Programme is a network of collaborative research centres designed to increase innovation in key areas, and help drive economic growth for the UK. The Precision Medicine Catapult (PMC) was

established in 2015 and was based in Cambridge. Its aim was to harness UK expertise to become a world leader in developing precision medicine tests and therapies (University of Glasgow 2015).

In June 2017, Innovate UK announced some aspects of the scientific aims of the PMC would be transferred to the Medicines Discovery Catapult, and the PMC has now closed. The funding for the PMC is instead being directed into providing funding for grants for businesses and precision medicine regional centres of excellence throughout the UK (Innovate UK 2017).

100,000 Genomes Project

The 100,000 Genomes Project was launched in late 2012 by the Department of Health, and is run by Genomics England. The project aims to sequence the genomes of 100,000 National Health Service (NHS) patients and combine this genetic information with medical records. Participants are NHS patients with a rare disease, their families, and patients with cancer (Genomics England 2017a).

The main goals of the project are: to create an ethical and transparent program based on consent; to benefit patients and set up a genomic medicine service for the NHS; to facilitate new scientific discovery and medical insights; and to encourage the development of a UK genomics industry. As of August 2017, the 100,000 Genomes Project has sequenced 32,642 genomes (Genomics England 2017b).

Cancer Research UK Genomics Initiative

The Cancer Research UK Genomics Initiative commenced in 2011 and provided funding for genomics research in cancer. The initiative is funding nine projects for two years, all of which included genome sequencing and subsequent analysis of the genomic data. Data from each of the projects is published in an open-access database so it is available for genomics researchers worldwide (Cancer Research UK 2011).

Transforming Genetic Medicine Initiative

The Transforming Genetic Medicine Initiative (TGMI) is a £5.3 million 4-year program funded by the Wellcome Trust. It was established in June 2016 to undertake collaborative research, development and promotion required for large-scale genome sequencing to be integrated into mainstream medicine. The TGMI has four key aims:

1. To build a gene-disease map that provides information on the association between genes and disease in humans.
2. To develop a clinical annotation reference system to provide standards and tools for consistent recording and reporting of genomic data.
3. To develop techniques that allow access of data from multiple sources worldwide to deliver fast, automated, large-scale, high-throughput gene analysis required for successful genomics.
4. To develop processes that maximise the research and clinical utilities of genetic testing.

TGMI works to deliver projects, tools and resources. Their key outputs to date include a sequencing dataset and gene data analysis software (Transforming Genetic Medicine Initiative 2017).

vi. United States of America

Precision Medicine Initiative

The Precision Medicine Initiative (PMI) was launched by President Obama in his 2016 Federal Budget with an investment of $215 million. The initiative is run by the National Institutes of Health (NIH) in conjunction with the Food and Drug Administration (FDA), and the Office of the National Coordinator for Health Information Technology (ONC) (The White House 2016).

The key component of the PMI is the National Institutes of Health All of Us Research Program, which is an initiative to gather genomic data from one million or more people living in the US to accelerate precision medicine. Participant enrolment for anyone over the age of 18 living in the United States is planned to commence during 2017, and children will also be able to participate in the coming years.

Through a series of funding awards, NIH has established the essential elements of the All of Us Research Program. Funding has gone towards the establishment of biobanks, data and research centres, health care provider organisations to collect data, participant centres, participant technology systems centres, and communications and engagement (National Institutes of Health 2017).

Million Veteran Program

Million Veteran Program (MVP) is a national voluntary research program funded by the Department of Veterans Affairs (VA) Office of Research and Development. The program collects genomic and health information from veterans receiving care in the Veteran Affairs Healthcare System. The Department of Defence also partners with VA to facilitate the enrolment of active duty participants into MVP. Data will be used to gain a better understanding of disease and military-related illnesses (Office of Research and Development 2017).

California Initiative to Advance Precision Medicine

The California Initiative to Advance Precision Medicine (CIAPM) was announced in April 2015 and received $13 million funding from the California state government. CIAPM is a collaborative initiative between the state, the University of California, and several public and private organisations.

The aim of the initiative is to provide infrastructure and resources necessary to support precision medicine activities in California. The program is building an inventory of the state's precision medicine initiatives, including research projects, clinical studies, databases, and analysis platforms. In addition to this, CIAPM funded two precision medicine demonstration projects in 2015 and a further six projects in 2016 (California Initiative to Advance Precision Medicine 2016).

Innovative Genomics Institute and Initiative

The Innovative Genomics Institute (IGI) was created in 2014 as the Li Ka Shing Centre for Genetic Engineering, from a donation from the Li Ka Shing Foundation. The centre expanded in 2016, and relaunched as the Innovative Genomics Institute.

The Innovative Genomics Initiative is a partnership between the University of California, Berkeley, and the University of California, San Francisco, utilising the IGI. The focus of the initiative is to fully understand CRISPR-based genome editing and apply this to improve healthcare. Achievements to date include improving the efficiency of gene replacement, and initial work towards a treatment for sickle cell disease.

To complement its research, the institute also runs conferences, workshops, and public engagement on genome engineering (Innovative Genomics Initiative 2017).

Personalised Medicine Coalition

The Personalised Medicine Coalition (PMC) is an educational and advocacy organisation that was launched in 2004 by 20 organisations representing all sectors of the healthcare system. PMC brings together researchers, patients, and healthcare providers to promote the understanding and uptake of personalised medicine technologies, services and products by addressing regulatory, reimbursement and clinical adoption issues critical to the field's advancement. Their key outputs to date are a number of policy documents and public events (Personalised Medicine Coalition 2017).

vii. Middle East

The Nancy and Stephen Grand Israel National Centre for Personalised Medicine

The Nancy and Stephen Grand Israel National Centre for Personalised Medicine (G-INCPM) was established in 2012 by the Weizmann Institute of Science. It is named after Nancy and Stephen Grand, who donated US$50 million to establish the centre. G-INPCM is a collaborative research facility comprised of genomics, protein profiling, drug discovery, and bioinformatics research platforms, and is accessible by Israeli academic and industry researchers. The centre aims to support personalised medicine by providing access to cutting-edge infrastructure for researchers, and promoting a culture of collaboration and knowledge sharing (G-INCPM 2014).

Saudi Human Genome Program

The SAR300 million Saudi Human Genome Program was launched in December 2013 and aims to sequence the genomes of 100,000 Saudi Arabian people to identify the genetic basis for a range of diseases. The first phase of the project involved standardising sequencing laboratories. This was followed by a period of training and knowledge transfer to increase the national genomic skills base, which will support the ultimate aim of genome sequencing and data analysis. Saudi leaders focused US$40 million on a pilot project to diagnose patients with single-gene diseases who are recruited by physicians at Saudi research institutions (Kaiser 2016; Saudi Human Genome Program 2013).

Qatar Biobank

Qatar Biobank was launched in 2012 in collaboration with the Ministry of Public Health and Hamad Medical Corporation. The project aims to establish a national biobank for biological samples and health information to enable research into advancing precision medicine in Qatar by recruiting more than 60,000 participants by 2019. The project's pilot phase concluded in February 2016 with the official opening of the Qatar Biobank. Qatar Biobank recorded 2006 participants in the first three years of the project (Qatar Biobank 2017).

viii. Canada

Personalised Medicine Initiative

The Personalized Medicine Initiative (PMI) was established in 2014 with the goal of introducing technologies for personalised medicine into the Canadian healthcare system. PMI is a collaborative community with members from all the technological, preclinical and clinical healthcare communities in British Columbia and receives funding from a number of clinical, academic, government, and industry partners.

PMI holds weekly meetings at which the perspectives of personalised care stakeholders in government, industry, healthcare and academia are presented. PMI also sources funding, prepares teams, and assists in project management for a range of genomic projects, supports product commercialisation for new technologies, and runs an annual summit on personalised medicine (Personalised Medicine Initiative 2017).

Genome Canada

Genome Canada is a not-for-profit organisation funded by the Canadian Government, founded in 2000. They support large-scale genomics research projects, and provide Canadian scientists with access to the most advanced technologies and expertise, through a network of Science and Technology Innovation Centres (STICs). Genome Canada's aim is to advance genomics through research translation, facilitating collaboration, and investment in large-scale precision medicine science and technology (Genome Canada 2017).

Genome Canada also supports and funds the Precision Medicine Policy Network, which brings together top Canadian genomics researchers to address four key policy themes:

1. Research ethics
2. Health economics and health technology assessment
3. Knowledge transfer and implementation in healthcare systems
4. Intellectual property and commercialisation

Key outputs from the Precision Medicine Policy Network include publications in scientific journals, presentations, workshops, and policy documents (Precision Medicine Policy Network 2017).

Orion Health Canada

Orion Health Canada have developed a Care Coordination Tool (CCT), which allows patients to digitally create, update and share their personalised care plan that can be accessed by all of a patient's healthcare providers. Using this tool, care-givers receive up-to-date patient data, allowing them to monitor the patient's past and present healthcare information in one location, and enabling greater collaboration between healthcare providers (Orion Health 2017).

Genomics R&D Initiative

The Genomics R&D Initiative (GRDI) was established in 1999 to fund genomics research in eight federal science departments and agencies, covering areas such as health, agriculture, forestry, aquaculture, and environment. The GRDI has provided funding for a number of health genomics research projects to date, in topics such as foodborne illnesses, immunotoxicogenomcis, and developing safer stem cell treatments (Government of Canada 2017).

ix. Mexico

National Institute of Genomic Medicine

The National Institute of Genomic Medicine (INMEGEN) was established in 2004 by a consortium consisting of the National Autonomous University of Mexico, the National Council for Science and Technology, the Ministry of Health and the Mexican Health Foundation. INMEGEN's key actions focus on genomic research, education and outreach, technology development, and development of the institute through strategic alliances. Their main outputs include a postgraduate course in genomic medicine, a number of scientific publications, and educational workshops (Instituto Nacional de Medicina Genomica 2013).

x. New Zealand

Precision Driven Health Initiative

The Precision Driven Health Initiative was established in March 2016 by Orion Health, University of Auckland, and Waitemata District Health Board, with support from the Ministry of Business, Innovation and Employment. The initiative is investing NZ$38 million over seven years to provide world-leading research in precision medicine. It is also encouraging precision health research through funding of postgraduate scholarships, summer research scholarships, and travel grants (Precision Driven Health 2017).

Precision Driven Health focuses on four key themes:

1. Making new data sources available to broaden the scope of precise healthcare
2. Utilise a range of big data sources for predictive modelling in a healthcare setting
3. Utilise disparate data sources, analyses, and technologies to enable precise healthcare
4. Leverage technology to encourage self-management of healthcare.

Orion Health, Medtech and CSC

In late 2015, Orion Health, Medtech and CSC announced they were collaborating to deliver an innovative precision medicine solution for New Zealand. The organisations stated they would be working together to link hospital and primary care data in a single digital platform, with the aim to integrate this information with genomics, microbiomics, proteomics and other new health data in the future to provide a truly personalised healthcare system for New Zealanders (Orion Health 2015).

xi. Commercial companies

DeCODE Genetics

DeCODE Genetics was established in 1996 by Dr Kari Stefansson to map the unique genomes of the Icelandic population. This population was chosen because there are relatively few ancestors that account for the current Icelandic population, meaning biomarkers of genetic disease are more easily found that in other more heterogeneous populations. The company's work has provided genetic information for Alzheimer's disease, type 2 diabetes, cardiovascular disease, and schizophrenia, among other health issues (DeCODE Genetics 2017).

23andMe

California-based 23andMe was founded in 2006 by Anne Wojcicki, Linda Avey, and Paul Cusenza, and launched its Personal Genome Service one year later. In April 2017, 23andMe had genotyped over 2 million customers worldwide and were granted first ever FDA approval to market direct-to-consumer Genetic Health Risk reports, including tests for Parkinson's and Alzheimer's disease. In addition to providing genomic services to customers, 23andMe also contributes their customers' genomic data, with the individual's permission, to a number of scientific studies; on average, one customer contributes their genomic data to over 200 studies. 23andMe have published over 75 peer-reviewed studies in scientific journals, and have produced a number of White Papers on their discoveries (23andMe 2017).

In late 2013, the US FDA banned 23andMe from offering genetic screening for health information because the company had not provided evidence of the accuracy of its detection methods or standard error information (Gutierrez 2013). 23andMe recommenced providing consumers with genomic-based health information again in late 2015, after gaining FDA approval (23andMe 2017).

Futura Genetics

Futura Genetics is a Canadian company founded in 2014 by Auro Pontes and Efi Binder. They provide a genetic test that can be used worldwide designed to assess an individual's risk of developing each of the 28 most common conditions, including cancer, Alzheimer's disease, and obesity (Futura Genetics 2017).

Veritas Genetics

Veritas Genetics was founded in 2014 by Prof George Church, Mirza Cifric, Dr Preston Estep, and Dr Jonathan Zhao, and is headquartered in Massachusetts, USA. In March 2016, Veritas launched their gene testing kits for US$999, which includes screening, analysis, and genetic counselling. In addition to whole genome sequencing, the company tests for several types of cancer, and for hereditary diseases in newborns and pregnant women (Veritas Genetics 2017).

Counsyl

Counsyl is a DNA testing and genetic counselling service founded in 2007 by Ramji Srinivasan, Dr Eric Evans, and Rishi Kacker, with US$102 million in funding from private investors (Counsyl 2017). Their first genetic testing product was launched in 2009, and today they have three different tests:
Foresight Carrier Screen: targeted at couples who are planning to have children to assess if they carry certain genetic diseases.

Prelude Prenatal Screen: tests a baby in utero for chromosome conditions such as Down syndrome.
Reliant Cancer Screen: assesses an individual's risk of developing nine different cancers.

myDNA

myDNA, previously known as GenesFX, is a Melbourne-based genetic interpretation company founded in 2007 by Assoc. Prof Leslie Sheffield. myDNA is a team of pharmacologists and molecular and clinical geneticists who provide gene testing and interpretation of genetic data to explain its relevance to the individual and their doctor, with a focus on medication and diet (myDNA 2017).

Verge Genomics

Verge Genomics was founded in 2015 by Alice Zhang and Jason Chen, with US$4 million in private funding. Based in California, Verge uses genomic data to develop better drugs to treat brain diseases, including Alzheimer's, ALS, and Parkinson's (Verge Genomics 2017).

Foundation Medicine

Foundation Medicine was founded in 2010 and is based in Massachusetts, USA, and launched their first product, FoundationOne, in 2012. Foundation focus on identifying genetic risk of developing cancer, and now have four genome tests, including the first FDA-approved companion diagnostic assay for the treatment of ovarian cancer with rucaparib (Foundation Medicine 2017).

Rosetta Genomics

Rosetta Genomics was established in 2000 by Dr Isaac Bentwich and is headquartered in New Jersey, USA, with offices in the US and Israel. Rosetta develops diagnostic tests to differentiate between various types of cancer to enable accurate diagnosis, prognosis, and improved patient care (Rosetta Genomics 2017).

Color Genomics

California-based Color Genomics was founded in 2013 by Othman Laraki, Taylor Sittler, Nish Bhat, and Elad Gil and provides a physician-ordered genetic test to assess hereditary cancer risk. Color also contributes anonymised data to public genomic databases to assist in genomic research (Color Genomics 2017).

Intellia Therapeutics

Intellia Therapeutics a genome editing company founded in 2014 by Dr Nessan Bermingham, and has headquarters in Massachusetts, USA. The company's aim is to develop techniques to cures to diseases using the genome editing technology CRISPR/Cas9. Intellia's in vivo programs focus on liver diseases, and their ex vivo focus is on receptor T cells and hematopoietic stem cells (Intellia Therapeutics 2017).

Editas Medicine

Massachusettes-based Editas Medicine is a discovery-phase genome editing company founded in 2013. They aim to use CRISPR/Cas9 technology to develop curative gene editing techniques, with a focus on eye, muscle, blood, lung, and liver diseases, and cancer (Editas Medicine 2017).

CRISPR Therapeutics

CRISPR Therapeutics is a gene-editing company founded by Dr Rodger Novak, Dr Emmanuelle Charpentier, and Shaun Foy in 2013 with initial funding from Versant Ventures. The company was established in Basel, Switzerland, and subsequently opened R&D operations in Massachusetts, USA, and business operations in London, UK. CRISPR Therapeutics licensed the foundational CRISPR/Cas9 patent estate for human therapeutic use from their scientific founder, Dr. Emmanuelle Charpentier, who co-invented the application of CRISPR/Cas9 for gene editing (CRISPR Therapeutics 2017).

Caribou Biosciences

Caribou Biosciences is a California-based gene editing company that was founded in 2011 by Dr James Berger, Dr Jennifer Doudna, and Dr Martin Jinek. Caribou is using CRISPR/Cas9 gene editing technology, which Dr Jennifer Doudna co-founded, with applications in human and animal therapeutics, agricultural biotechnology, biological research, and industrial biotechnology (Caribou Biosciences 2017).

Genus plc

Genus plc is a biotechnology company founded in 1994 and is headquartered in Basingstoke, UK. They focus on the application of gene editing to the porcine, dairy, and beef sectors. Genus is currently working with the University of Edinburgh to demonstrate how CRISPR can remove a molecule in pigs that makes them susceptible to porcine reproductive and respiratory syndrome (Genus 2017).

DuPont Pioneer

DuPont Pioneer is an agricultural biotechnology company that was founded in 1926 and is headquartered in Iowa, USA. DuPont Pioneer is using CRISPR-Cas9 gene-editing technology to maximise productivity and profitability of a range of agricultural products. The first commercial product DuPont developed was waxy corn hybrids, which are expected to be available to grow in the US within four years, pending trials and regulation (PRWeb 2016).

US CRISPR-Cas9 patent dispute

The application of CRISPR-Cas9 for gene editing was invented by Dr Jennifer Doudna and Dr Emmanuelle Charpentier in 2012 at the University of California (UC), Berkeley. They used the tool to cut and rearrange viral DNA. In 2013, Feng Zhang, a bioengineer from MIT and Harvard's Broad Institute, created a procedure for using CRISPR-Cas9 specifically for eukaryotic cells, including in humans. Both teams applied for patents for their procedures, and Broad Institute was awarded its patent first, despite UC being the first to apply for a patent on the technology.

UC Lawyers filed for an 'interference' proceeding in January 2016 in an effort to reverse the patent award to the Broad Institute, arguing that the use of CRISPR-Cas9 in eukaryotes overlapped with the UC invention. In February 2017, patent judges ruled that Broad's invention was distinct from that of UC and the patent would stand (Ledford 2017).

xii. Academy studies

US National Academies of Sciences and Medicine Human Gene-Editing Initiative

In May 2015, the US National Academies of Sciences and the National Academy of Medicine announced the launch of their initiative to guide decision making about human gene-editing research. The initiative includes an international summit, which took place in late 2015, a public meeting in Paris in April 2016, and a comprehensive study of the scientific foundations of human gene-editing technologies. The Academies convened a panel of experts to carry out the study, which culminated in a report *Human Genome Editing: Science, Ethics, and Governance* in early 2017 (National Academies of Sciences Engineering and Medicine & Press 2017). The key recommendations from the report are:

1. Promoting well-being: Pursuing applications of human genome editing that promote the health and well-being of individuals, and ensuring a reasonable balance of risk and benefit.
2. Transparency: Openness and sharing of information in accessible ways, including a commitment to disclosure of information to the fullest extent possible and meaningful public input into the policy-making process.
3. Due care: Research involving participants should proceed cautiously and incrementally, under appropriate supervision and in ways that allow for frequent reassessment.

4. Responsible science: Adherence to the highest standards of research. Researchers should undertake high-quality experimental design and analysis, appropriate review and evaluation of protocols and resulting data, transparency, and correction of false or misleading data or analysis.
5. Respect for persons: Research should include a commitment to the equal value of all individuals, respect for and promotion of individual decision making, and a commitment to preventing recurrence of the abusive.
6. Fairness: Like cases should be treated alike, and risks and benefits should be equitably distributed. Equitable access should be given to the benefits of resulting clinical applications of human genome editing.
7. Transnational cooperation: A commitment to collaborative approaches to research and governance while respecting differing national policies, coordinating regulatory standards, and ensuring transnational collaboration and data sharing among different scientific communities and responsible regulatory authorities.

Leopoldina (Germany) Individualised Medicine

In 2014, the German Academy of Sciences, Leopoldina, published a report *Individualised Medicine: Prerequisites and Consequences* (German National Academy of Sciences Leopoldina Union of the German Academies of Sciences and Humanities, 2014). The report focuses on the genetic and pharmacological aspects of oncology. The key recommendations are:

1. Our understanding of the generally complex causes of disease must continue to improve.
2. The sensitivity and specificity of biomarkers for diagnosis and therapy must be improved.
3. Accompanying research in the areas of economics, ethics and law should be strengthened.
4. Biobanks ought to be harmonised and standardised.
5. Anamnesis and phenotypic data must be collected in a standardised way.
6. Clinical studies should be adapted to new demands.
7. High-throughput bioanalytical procedures should be established at university hospitals.
8. Expanding and networking IT infrastructure and bioinformatics are overdue.
9. Statutory data protection provisions must be observed.
10. The rights and duties of non-medical scientists must be regulated.
11. Appealing framework conditions should be created for the development of companion diagnostics.
12. Developing strategies for risk-adapted prevention should be supported.
13. University hospitals must have sufficient resources for clinical research and medical care based on that research.
14. We must meet the increasing need for information and counselling.
15. Basic and advanced training and continuing education must be adapted to the requirements of Individualised Medicine.
16. Individualised Medicine requires structural adaptation and adequate funding in research and care.

Alliance Nationale France *Médecine Génomique 2025*

The report *France Médecine Génomique 2025*, produced in June 2016 by the National Alliance for Life Sciences and Health (Aviesan) for the French government, is a vision for delivery of national genome sequencing capacity sufficient to deliver 235,000 genomes a year by 2020. The plan was developed by experts from research, health and industrial sectors, and ministerial headquarters. It includes the creation of a national network of clinical centres, a national network of molecular genetics laboratories, and next generation sequencing and bioinformatics facilities to ensure the rapid and ethical emergence of the French genomic medicine sector (Inserm Press Office 2016).

Royal Society of New Zealand gene-editing technologies panel

The Royal Society of New Zealand has convened a panel of experts to consider the research, ethical, social, legal, regulatory, environmental and economic considerations of gene-editing technologies. The panel will also consider this in the context of New Zealand's unique cultural perspectives. The panel will aim to raise

awareness of genome technologies, and provide advice on the potential implications of gene-editing uptake in New Zealand (Royal Society of New Zealand 2017).

UK Academy of Medical Sciences Realising the Potential of Stratified Medicine

In 2013, the UK Academy of Medical Sciences report *Realising the Potential of Stratified Medicine* focused on providing solutions to barriers to implementing precision medicine in the UK (The Academy of Medical Sciences, 2013). The report drew evidence from discussion papers presented at a symposium held by the Academy in 2012, discussions at the symposium, case studies, and the expertise of the group. The key findings of the report were:

- Collection, analysis and use of biomedical and health data should be enhanced.
- Changes to regulation and pricing systems are required as they currently do not provide adequate incentives for the development of stratified medicine products.
- Influencing clinical practice will be critical for stratified medicine to be embedded in healthcare.
- Collaboration will be crucial to accelerate the development and adoption of stratified medicine.

The Academy followed up on the findings of the report by hosting a symposium *Stratified, personalised or P4 medicine* in May 2015 in conjunction with the Medical Research Council, Science Europe, and the University of Southampton. The symposium discussed the ongoing challenges of implementing personalised medicine (The Academy of Medical Sciences 2017).

References

23andMe 2017, 'About Us', , vol. 2017, no. 8 August, retrieved from <https://mediacenter.23andme.com/about-us/>.

Aaviksoo Ballensiefen, W., Bauer, H., Bußhoff, U., Frenzel, M., Guglielmi, G., Andersen, P.H., Julkowska, D., Kuhlmann, K., Molnar, M.J., O'Driscoll, M., A & ICPerMed 2017, *The ICPerMed Action Plan*, retrieved from <http://www.icpermed.eu/media/content/ICPerMed_Actionplan_2017_web.pdf>.

BBMRI-ERIC 2017, 'Biobanking and biomolecular resources research infrastructure - european research infrastructure consortium', , vol. 2017, no. 8 August, retrieved from <http://www.bbmri-eric.eu/BBMRI-ERIC/about-us/>.

California Initiative to Advance Precision Medicine 2016, 'About CIAPM', , vol. 2017, no. 8 August, retrieved from <http://www.ciapm.org/about-ciapm>.

Cancer Research UK 2011, 'Cancer Research UK launches nine high-tech gene projects', retrieved from <http://www.cancerresearchuk.org/about-us/cancer-news/press-release/2011-11-18-cancer-research-uk-launches-nine-high-tech-gene-projects?_ga=2.107003914.962089662.1501648155-638791041.1501044100>.

Caribou Biosciences 2017, 'Origins', , vol. 2017, no. 8 August, retrieved from <http://cariboubio.com/origins>.

Color Genomics 2017, 'Our mission', , vol. 2017, no. 8 August, retrieved from <https://www.color.com/mission>.

Counsyl 2017, 'Company info', , vol. 2017, no. 8 August, retrieved from <https://www.counsyl.com/press/info/>.

CRISPR Therapeutics 2017, 'About us', , vol. 2017, no. 8 August, retrieved from <http://www.crisprtx.com/about-us/overview.php>.

DeCODE Genetics 2017, 'About decode genetics', , vol. 2017, no. 8 August, retrieved from <https://www.decode.com/company/>.

Editas Medicine 2017, 'Company overview', , vol. 2017, no. 8 August, retrieved from <http://www.editasmedicine.com/company-overview>.

European Alliance for Personalised Medicine 2017, 'European Alliance for Personalised Medicine: What we do', , vol. 2017, no. 8 August, retrieved from <https://www.euapm.eu/what-we-do.html>.

Foundation Medicine 2017, 'About us: A culture dedicated to advancing cancer care', , vol. 2017, no. 8 August, retrieved from <https://www.foundationmedicine.com/about>.

Futura Genetics 2017, 'About Us', , vol. 2017, no. 8 August, retrieved from <https://futuragenetics.com/en/about/>.

G-INCPM 2014, 'About G-INCPM', , vol. 2017, no. 10 August, retrieved from <http://g-incpm.weizmann.ac.il/about-g-incpm>.

Genome Asia 100K 2017, 'Genome Asia 100K', , vol. 2017, no. 8 August, retrieved from <http://www.genomeasia100k.com/>.

Genome Canada 2017, 'Genome Canada: About us', , vol. 2017, no. 8 August, retrieved from <https://www.genomecanada.ca/en/about-us>.

Genomic Medicine Alliance 2017, 'Genomic Medicine Alliance: Aims', , vol. 2017, no. 8 August, retrieved from <http://www.genomicmedicinealliance.org/2013-02-19-14-51-27/aims>.

Genomics England 2017a, 'The 100,000 Genomes Project', , vol. 2017, no. August 8, retrieved from <https://www.genomicsengland.co.uk/the-100000-genomes-project/>.

Genomics England 2017b, 'The 100,000 Genomes Project by numbers', retrieved from <https://www.genomicsengland.co.uk/the-100000-genomes-project-by-numbers/>.

Genus 2017, 'Strategic framework', , vol. 2017, no. 8 August, retrieved from <http://www.genusplc.com/about/strategic_framework.aspx>.

German National Academy of Sciences Leopoldina Union of the German Academies of Sciences and Humanities, acatech – NA of S and E 2014, *Individualised Medicine – Prerequisites and Consequences*,.

Global Alliance for Genomics and Health 2017a, 'Global Alliance for Genomics and Health: How We Work', ,

vol. 2017, no. 8 August, retrieved from <http://genomicsandhealth.org/about-the-global-alliance/how-we-work>.
Global Alliance for Genomics and Health 2017b, 'Work Products & Demonstration Projects', , vol. 2017, no. 8 August, retrieved from <http://genomicsandhealth.org/work-products-demonstration-projects>.
Government of Canada 2017, 'About the Genomics R&D Initiative (GRDI)', , vol. 2017, no. 8 August, retrieved from <http://grdi-irdg.collaboration.gc.ca/eng/about/initiative.html>.
Gutierrez, A 2013, 'U.S. Food and Drug Administration warning letter to Ann Wojcicki ' H and H Services (ed), retrieved from <https://www.fda.gov/ICECI/EnforcementActions/WarningLetters/2013/ucm376296.htm>.
Holub Swertz, M., Reihs, R., van Enckevort, D., Müller, H., Litton, J-E., P 2016, 'Biopreservation and Biobanking' BBMRI-ERIC (ed), retrieved from <https://directory.bbmri-eric.eu/menu/main/dataexplorer?entity=eu_bbmri_eric_collections&mod=data&attrs%5B%5D=country&attrs%5B%5D=biobank&attrs%5B%5D=collection&hideselect=true>.
Human Heredity and Health in Africa 2013, 'About H3 Africa', , vol. 2017, no. 8 August, retrieved from <http://h3africa.org/about>.
ICPerMed 2017, 'ICPerMed International Consortium: About ICPerMed', , vol. 2017, no. 8 August, retrieved from <http://www.icpermed.eu/en/icpermed-about.php>.
Innovate UK 2017, 'Additional responsibilities for Medicines Discovery Catapult', *Catapult to be centre for medicines; diagnostics; biomarkers; trial support.*, retrieved from <https://www.gov.uk/government/news/additional-responsibilities-for-medicines-discovery-catapult>.
Innovative Genomics Initiative 2017, 'Overview', , vol. 2017, no. 8 August, retrieved from <https://innovativegenomics.org/overview/>.
Inserm Press Office 2016, 'Presentation of the French Plan for Genomic Medicine 2025', retrieved from <http://presse.inserm.fr/en/presentation-of-the-french-plan-for-genomic-medicine-2025/24328/>.
Instituto Nacional de Medicina Genomica 2013, 'INMEGEN', , vol. 2017, no. 10 August, retrieved from <http://www.inmegen.gob.mx/es/inmegen-english/>.
Intellia Therapeutics 2017, 'Overview', , vol. 2017, no. 8 August, retrieved from <http://www.intelliatx.com/about-us/overview/>.
Kaiser, J 2016, 'Saudi gene hunters comb country's DNA to prevent rare diseases', *Science*, retrieved from <http://www.sciencemag.org/news/2016/12/saudi-gene-hunters-comb-countrys-dna-prevent-rare-diseases>.
Ledford, H 2017, 'Broad Institute wins bitter battle over CRISPR patents', *Nature*, retrieved from <http://www.nature.com/news/broad-institute-wins-bitter-battle-over-crispr-patents-1.21502>.
myDNA 2017, 'Out story', , vol. 2017, no. 8 August, retrieved from <https://www.mydna.life/our-story/>.
National Academies of Sciences Engineering and Medicine & Press, TNA 2017, *Human Genome Editing: Science, Ethics, and Governance*, Washington DC.
National Institute of Biomedical Genomics 2017, 'About us', , vol. 2017, no. 10 August, retrieved from <http://www.nibmg.ac.in/?q=ourvismis>.
National Institutes of Health 2017, 'All of Us Research Program: About', , vol. 2017, no. 8 August, retrieved from <https://allofus.nih.gov/about>.
Office of Research and Development 2017, 'Million Veteran Program (MVP)', , vol. 2017, no. 8 August, retrieved from <https://www.research.va.gov/mvp/>.
Orion Health 2015, 'Medtech and CSC team with Orion Health to deliver a world-first precision medicine solution for New Zealand', retrieved from <https://orionhealth.com/au/about-us/press-releases/medtech-and-csc-team-with-orion-health-to-deliver-a-world-first-precision-medicine-solution-for-new-zealand/>.
Orion Health 2017, 'Orion Health's Care Coordination Tool (CCT)', , vol. 2017, no. 8 August, retrieved from <https://orionhealth.com/ca/products/coordinate/cct-powered-with-new-functionality-and-enhancements/>.
Padma, T V 2016, 'India's budget keeps dream of genomics hub alive', *Nature*, vol. 531, no. 7592, retrieved from <http://www.nature.com/news/india-s-budget-keeps-dream-of-genomics-hub-alive-1.19469>.
Personalised Medicine Coalition 2017, 'Mission', , vol. 2017, no. 8 August, retrieved from

<http://www.personalizedmedicinecoalition.org/About_Us/About_PMC>.

Personalised Medicine Initiative 2017, 'Our approach', , vol. 2017, no. 8 August, retrieved from <http://personalizedmedicineinitiative.ca/approach/>.

Philippakis Azzariti, D.R., Beltran, S., Brookes, A.J., Brownstein, C.A., Brudno, M., Brunner, H.G., Buske, O.J., Carey, K., Doll, C., Dumitriu, S., Dyke, S.O.M., den Dunnen, J.T., Firth, H.V., Gibbs, R.A., Girdea, M., Gonzalez, M., Haendel, M.A., Hamosh, AA 2015, 'The Matchmaker Exchange: A Platform for Rare Disease Gene Discovery', *Human Mutation*, vol. 36, pp. 915–921.

Precision Driven Health 2017, 'Precision Driven Health: Our Partnership', , vol. 2017, no. 8 August, retrieved from <http://www.precisiondrivenhealth.com/about/>.

Precision Medicine Policy Network 2017, 'About the Precision Medicine Policy Network', , vol. 2017, no. 8 August, retrieved from <http://precisionmedicinepolicynetwork.org/about/>.

PRWeb 2016, 'DuPont Pioneer Announces Intentions to Commercialize First CRISPR-Cas Product', retrieved from <http://www.prweb.com/releases/dupont-pioneer-seed/crispr-cas-corn/prweb13349828.htm>.

Qatar Biobank 2017, 'Qatar Biobank: About us', , vol. 2017, no. 8 August, retrieved from <http://www.qatarbiobank.org.qa/about-us>.

Rosetta Genomics 2017, 'About us', , vol. 2017, no. 8 August, retrieved from <https://rosettagx.com/about>.

Royal Society of New Zealand 2017, 'Gene-editing technologies', , vol. 2017, no. 8 August, retrieved from <https://royalsociety.org.nz/what-we-do/our-expert-advice/our-expert-advice-under-development/gene-editing/>.

Russell, G 2016, 'China plans leading role in "precision medicine" field', *Reporting Health & Medicine*, retrieved from <http://jmsc.hku.hk/reportinghealth2016/2016/04/06/china-plans-leading-role-in-precision-medicine-field/>.

Saudi Human Genome Program 2013, 'Saudi Human Genome Program: Summary', , vol. 2017, no. 8 August, retrieved from <http://shgp.kacst.edu.sa/site/Summary.html#>.

The Academy of Medical Sciences 2017, 'Stratified Medicine', , vol. 2017, no. 8 August, retrieved from <https://acmedsci.ac.uk/policy/policy-projects/Stratified-medicine>.

The European Personalised Medicine Association 2017, 'Make Personalised Medicine and Diagnostics a Reality for European Patients', , vol. 2017, no. 8 August, retrieved from <http://www.epemed.org/online/www/content/79/80/ENG/index.html>.

The White House 2016, 'The Precision Medicine Iniitiative', , vol. 2017, no. 8 August, retrieved from <https://obamawhitehouse.archives.gov/node/333101>.

Transforming Genetic Medicine Initiative 2017, 'Transforming Genetic Medicine Initiative: The plan', , vol. 2017, no. 8 August, retrieved from <http://www.thetgmi.org/about/the-plan/>.

University of Glasgow 2015, 'Scotland takes aim at precision medicine breakthroughs with catapult', retrieved from <http://www.gla.ac.uk/news/archiveofnews/2015/october/headline_429623_en.html>.

Verge Genomics 2017, 'About us', , vol. 2017, no. 8 August, retrieved from <https://www.vergegenomics.com/about-us-1/>.

Veritas Genetics 2017, 'About Veritas', , vol. 2017, no. 8 August, retrieved from <https://www.veritasgenetics.com/about>.