Genomics informed medicines optimisation - what's in the pipeline?

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Overview

Genomics and Medicines Optimisation

- The NHS Genomic Medicine Service
- National Genomic Test Directory
- Accelerating Genomic Medicine in the NHS strategy for England
- Genomics Informed Medicines Optimisation

ATMPs and Gene Therapies

- Introduction to ATMPS and gene therapies
- Gene therapies already in use by the NHS
- Commissioning of ATMPS
- Horizon scanning
- Future ATMPs on our workplan

NHS Genomic Medicine Service (GMS)

NHS Genomic Medicine Service infrastructure

NHS England Genomics Unit
→ *Provides national oversight, coordination, commissioning, and funding to the NHS GMS infrastructure*

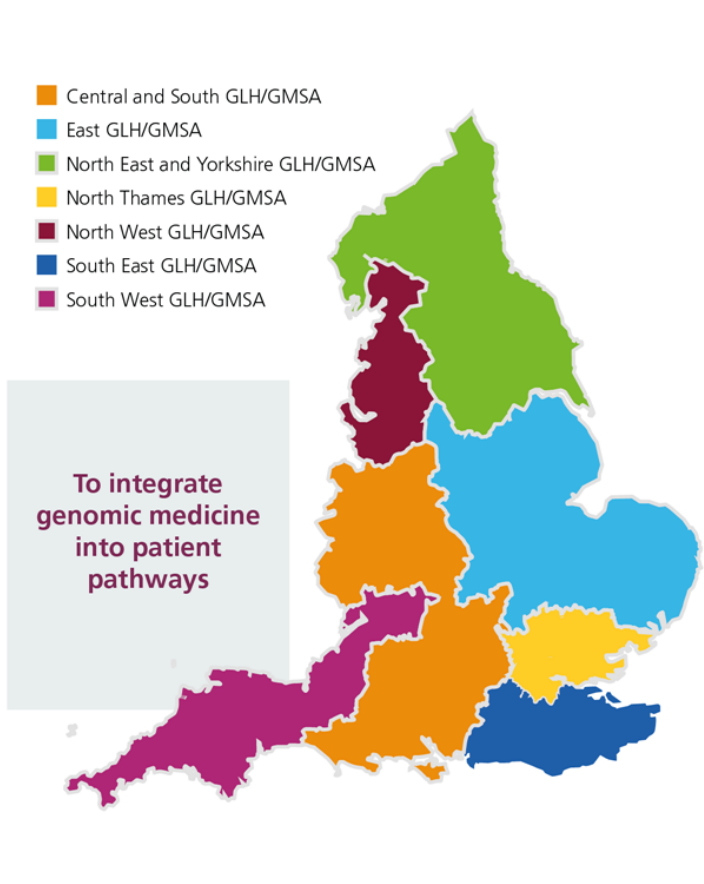
7 NHS Genomic Medicine Service Alliances (GMSAs)
→ *Provide multidisciplinary clinical leadership to embed genomic medicine across end to end pathways*

7 NHS Genomic Laboratory Hubs (GLHs)
→ *Deliver genomic testing outlined in the National Genomic Test Directory*
→ *Provide multi-disciplinary clinical and scientific leadership across referrals, analysis, interpretation with MDTs and reporting to clinicians*

17 NHS Clinical Genomics Services→ *Deliver comprehensive clinical genomic and counselling to direct diagnosis, risk assessment and lifelong clinical management of patients and their families*

Genomics England
→ *Supports national provision of WGS, including bioinformatics and underpinning information platform. Supports genomics research through the National Genomic Research Library (NGRL) and research initiatives*

Serving all patients and the public



Embedding Pharmacy Expertise

National Pharmacy Genomics team embedded in NHSE Genomics Unit and linked to Office of the Chief Pharmaceutical Officer

GMS Alliance Pharmacy Leads

Four pillars of practice – clinical practice, leadership, education, and research

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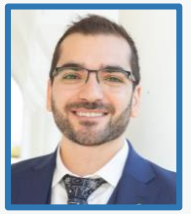

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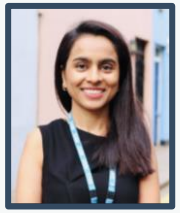

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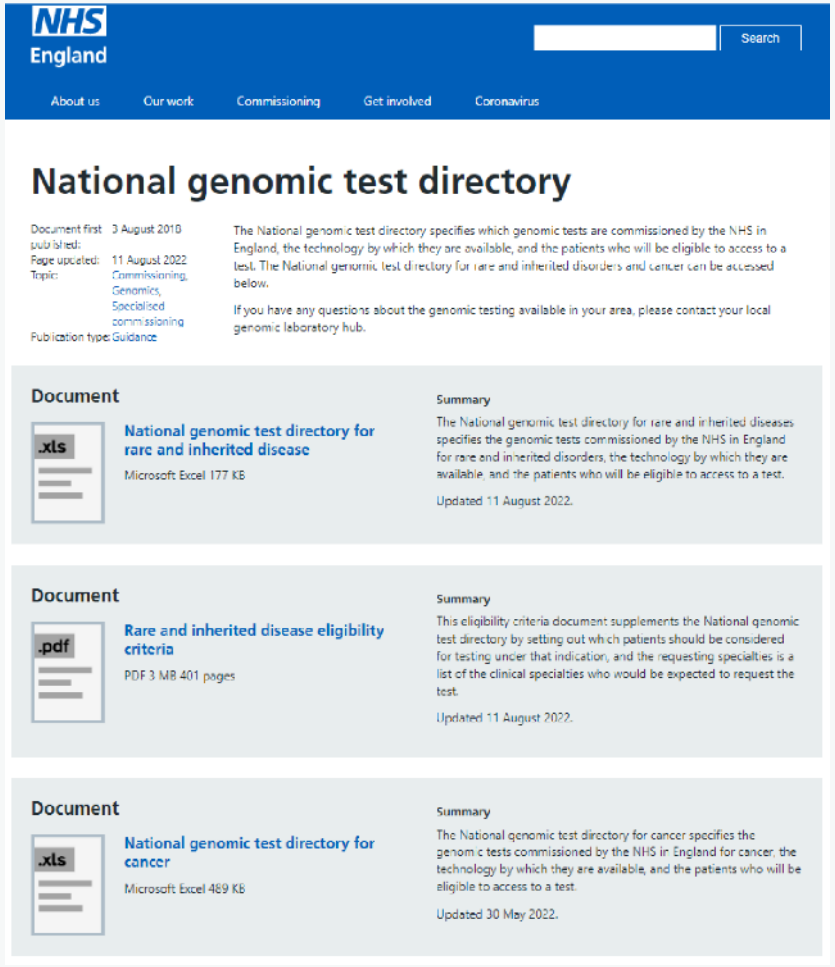
Driving the use of precision treatments and optimising the use of medicines

Gene therapies	• Use cutting-edge technology to deliver tailor-made genetic material into a patient's cells to treat disease
New targeted treatments	• Based on an increased understanding of the genomic basis for disease & diagnosis
Pharmacogenomics	• Guide treatment decisions and dosing using genomic data to predict drug response
Repurposing	• New indications for existing medicines
Predicting	• Drug resistance and pathogen resistance

Test Evaluation

National Genomic Test Directory – a national offer

We drive **equitable access** through a single mandated National Genomic Test Directory focused on: (1) **Rare and inherited disease**; (2) **Cancer ;** (3) **Pharmacogenomics** These support a standardised offer of funded testing across England and a new pricing model



- Covers a full repertoire of testing through a multi-modal approach (single gene, panel tests, WES, WGS)
- Fast track application process which can respond to new developments, for example to reflect licensing decisions including for Olaparib and NTRK inhibitors.
- Approximately 3,200 rare diseases and over 200 cancer clinical indications
- Updated annually through the Test Evaluation Working Groups. Through the Test Evaluation process to date, recent publication included tests such as whole genome sequencing for Sudden Unexplained Death in Childhood.
- Cements WGS as central to service delivery

Pharmacogenomics in Practice

DPYD

- Fluorouracil (5-FU) and capecitabine are chemotherapies used frequently in the treatment of patients with colorectal, breast, oesophageal, hepatic, and head and neck cancers.
- 5-FU and capecitabine are metabolised by the dihydropyrimidine dehydrogenase (DPD) enzyme. The enzymatic activity is influenced by inherited genetic variability in the *DPYD* gene.
- Patients with complete DPD deficiency are at risk of life-threatening toxicity and this class of drug is contra-indicated. Patients with a partial DPD deficiency are at increased risk of toxicity and should have a reduced dose.
- *DPYD* gene testing is available in the NHS GMS for patients who are planned to receive fluoropyrimidine treatment to identify patients with an increased risk of toxicity.

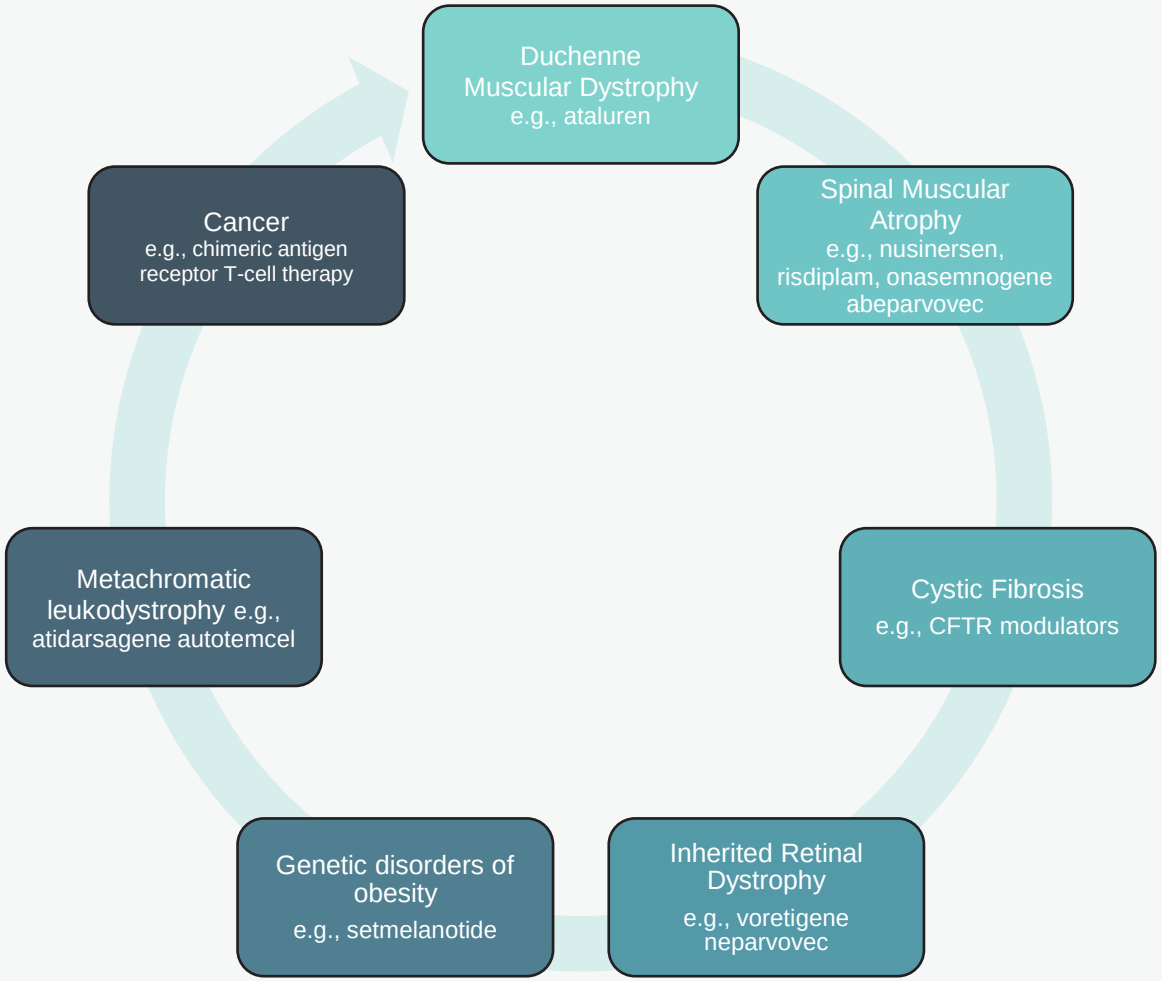
MT-RNR1

- Aminoglycoside antibacterials (amikacin, gentamicin, tobramycin) can cause ear and labyrinth disorders such as ototoxicity and hearing loss.
- 1 in 500 patients have the most common *MT-RNR1* variant m.1555A>G and will experience ototoxicity after auricular (ear) exposure to aminoglycosides.
- *MT-RNR1* gene testing is available in the NHS GMS for patients with a predisposition to infections requiring aminoglycosides and patients with hearing loss who have been exposed to aminoglycosides.

TPMT and *NUDT15*

- Purine analogue based drugs (e.g. mercaptopurine, thioguanine) are commonly used in the treatment of acute lymphoblastic leukaemia.
- Thiopurine S-methyltransferase (TPMT) and nudix hydrolase 15 (NUDT15) are key enzymes in the metabolism and inactivation of these drugs. The enzymatic activity of TPMT and NUDT15 is directly influenced by inherited genetic variability in the *TPMT* and *NUDT15* genes.
- Patients with lower enzymatic activity are more likely to experience toxicity such as severe bone marrow suppression.
- *TPMT* and *NUDT15* gene testing is available in the NHS GMS for patients with a confirmed diagnosis of acute lymphoblastic leukaemia and proposed treatment involving purine analogue based drugs to identify patients with an increased risk of toxicity.

Precision medicine in practice



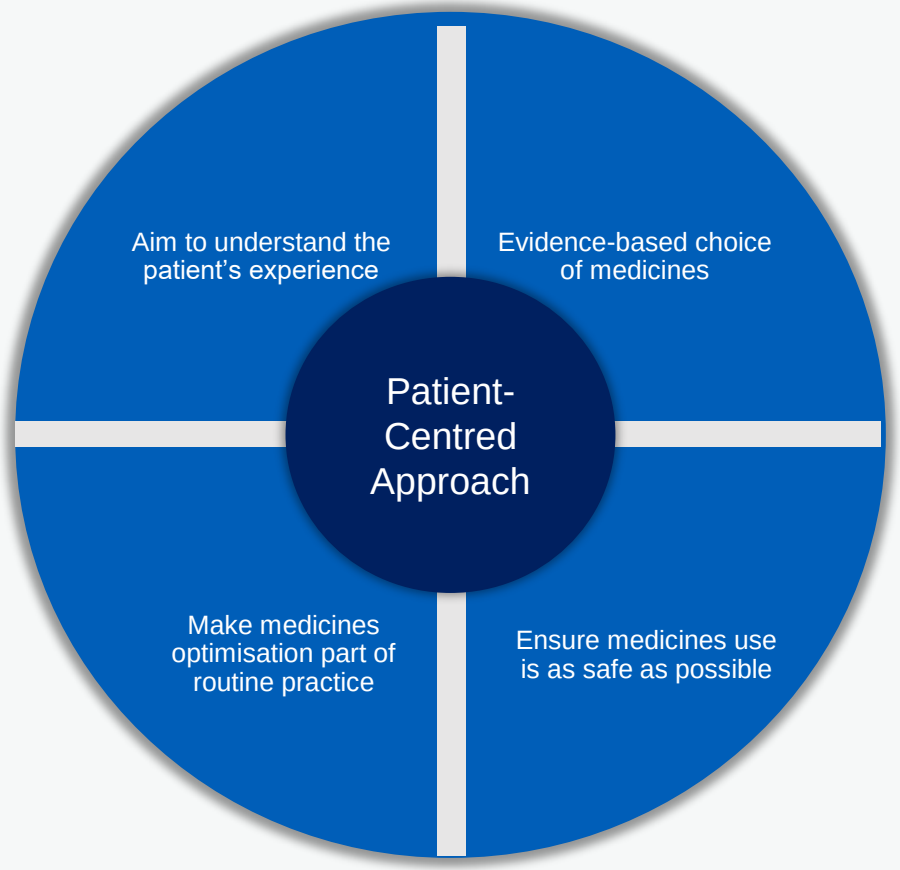
Genomics informed medicines optimisation

Linking genomics with medicines optimisation

Improved patient outcomes

Ensure access to cutting-edge screening and diagnostic technologies to offer personalised medicine choices to patient's through a shared-decision making approach

Equitable access to precision treatments and optimisation of existing medicines in view of patient choice and experience



Linking to the Commercial Medicines Pathway and horizon scanning, through to evidence-based use of genomic testing and optimised use of medicines

Link medicines access pathways to the National Genomic Test Directory and NHS Genomic Medicine Service Research Collaborative

Embed genomics into routine practice at a national, regional and local level within medicines optimisation governance structures and clinical pathways

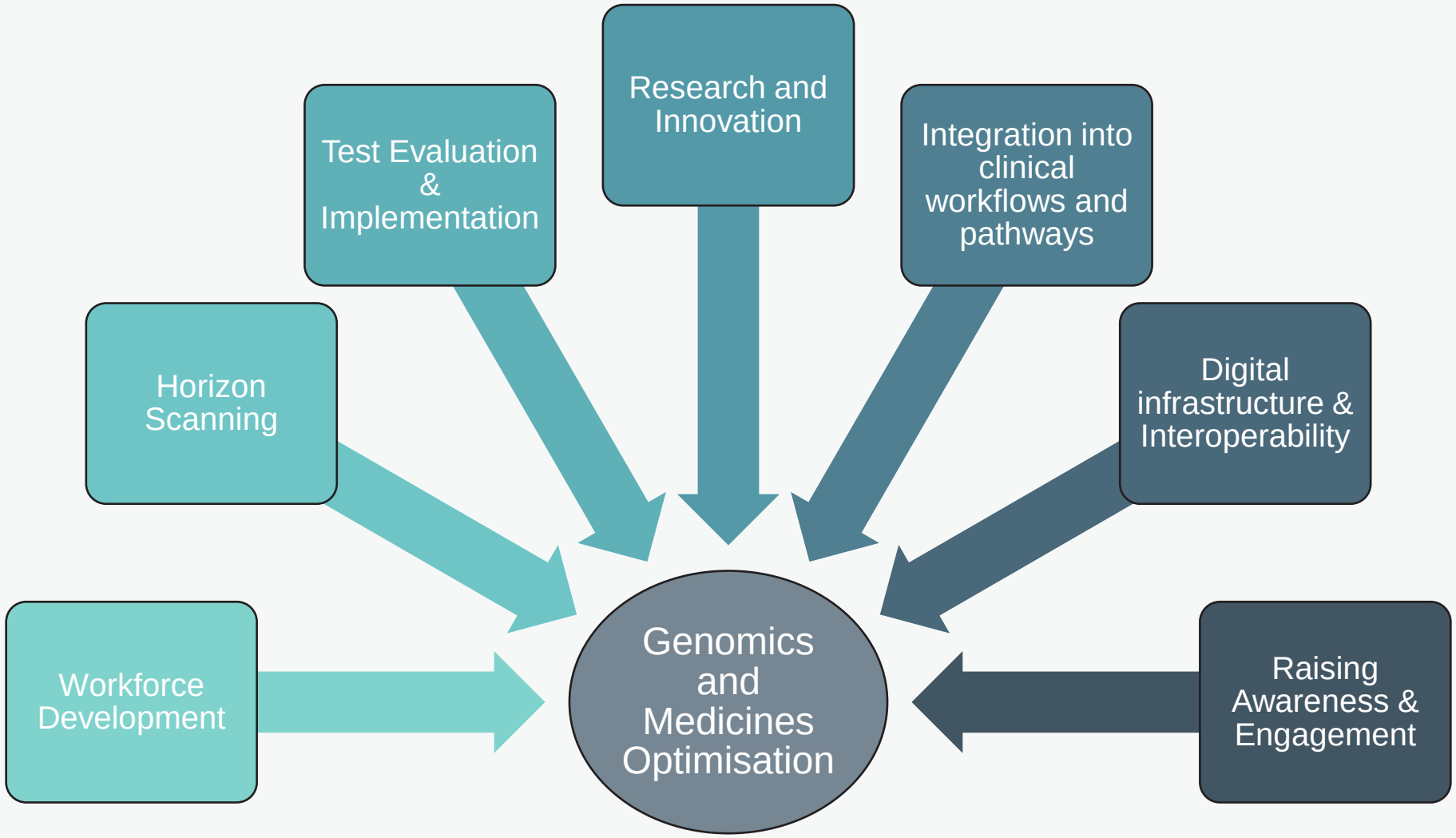
National standardised infrastructure and delivery via integrated national programmes, Genomic Medicine Service Alliances and local champions

Use genomics to target treatments and predict risk of toxicity to ensure the safe use of new and existing medicines

E.g., DPYD testing to prevent chemotherapy-related toxicity and other tests and processes introduced to support the safer use of medicines

Aligned measurement & monitoring of medicines optimisation

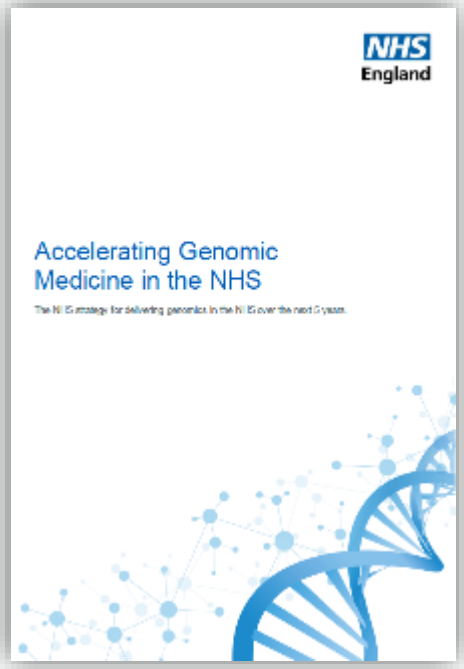
Optimising the use of medicines through genomics



Accelerating genomic medicine in the NHS

NHS Genomics Strategy – published in 2022

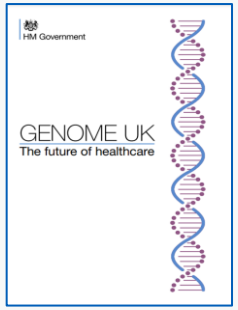
Our vision is that the power of genomics in **predicting, preventing and diagnosing disease, and targeting treatment** is accessible to all as part of routine care in the NHS. The strategy sets out a 5 year action plan



Key themes include:

1. **Embedding genomics across the NHS**, through a world leading innovative service model from primary and community care through to specialist and tertiary care
2. **Delivering equitable genomic testing for improved outcomes in cancer, rare, inherited and common diseases** and enabling precision medicine and reducing adverse drug reactions
3. **Enabling genomics to be at the forefront of the data and digital revolution**, ensuring genomic data can be interpreted and informed by other diagnostic and clinical data; and
4. **Evolving the service driven by cutting-edge science, research and innovation** to ensure that patients can benefit from rapid implementation of advances

UK Life Sciences Vision sets 10-year strategy for sector to solve some of the biggest healthcare problems of our generation including in cancer.



Genome UK; the future of healthcare sets out a 10 year vision how we will achieve progress in genomic medicine across Diagnosis & Personalised medicine, Prevention and Research

NHS Long Term Plan genomics commitments aligned to other policies for example cancer, cardiovascular, diabetes.

Priority 1 – Embedding genomics in the NHS through a world leading innovative service model

Priorities

1. **Co-creating services, infrastructure and an operating model with patients and the public.**
2. **Developing a sustainable infrastructure across testing, clinical services and research and innovation.**
3. **Building greater clinical and professional leadership and developing the capacity and capability of the workforce.**
4. **Developing national and international collaborations and partnerships.**

Key areas of progress

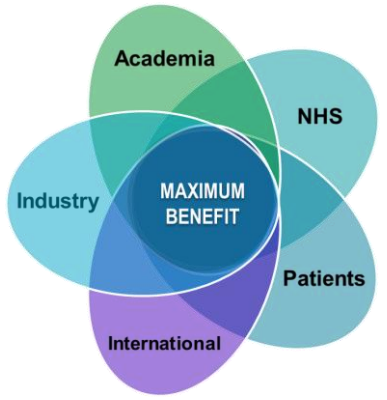
Integrated governance and networks

Implementing a price x activity model
Working with the NHS GLHs to be able to develop a cost for activity and a new model for payment

Publish activity data
From 2023 we will publish activity data and turnaround times publicly.

Workforce development and Genomics Training Academy
Carrying out workforce profiling to understand demands on the service and capacity. Will then inform the NHS GMS Workforce Strategy and People Plan for England.

NHS GMS People and Communities Forum
Feeding in patient views across the NHS GMS



Working with partners
Building strong relationships across a range of sectors

Priority 2 – Delivering equitable genomic testing for improved prevention, diagnosis, and precision medicine

Priorities

1. **Systematically introducing new clinical indications for genomic testing and embedding comprehensive genomic testing within end-to-end clinical pathways.**
2. **Driving the use of precision treatments and optimising the use of medicines through genomics.**
3. **Enabling the rapid evaluation and adoption of affordable, efficient, and innovative genomic technologies**

Key areas of progress

Aligning clinical trial targets with standard of care NHS testing
Identifying ~50 gene targets for inclusion in cancer gene panels

Ratification of end-to-end turnaround times in cancer genomics following a series of workshops with Royal Colleges

Aligning on priorities with NICE and regulatory pathways

Evolving the genomic testing strategy
Including new clinical indications and introduction of inclusion of gender-neutral language and running pilots for example ctDNA testing

Genomics Informed Medicines Optimisation Board

Chaired by CSO and CPhO

Priority 3 – Enabling genomics to be at the forefront of the data and digital revolution

Priorities

1. **Developing an interoperable informatic and data infrastructure that enables the NHS to use and share genomic data appropriately to improve patient care.**
2. **Putting the NHS at the forefront of using genomic data alongside other health data to drive health improvements for individuals and populations.**
3. **Enabling the NHS to use cutting-edge analytical tools and up to date variant databases to maximise diagnosis, access to precision medicine and efficiency.**

Key areas of progress

Genomic Data and Digital Board – Data and Digital Board established with high level stakeholders within NHS England

Data and Digital Framework

Implementation plan – Priorities identified including a digital Test Directory, Genomic Order Management and Unified Genomic Record

Priority 4 – Evolving the service through cutting-edge science, research and innovation

Priorities

1. **Enabling patients to make informed choices on the use of their data for research and innovation.**
2. **Enriching existing and developing new NHS GMS relationships to support innovation and the generation of evidence for adoption and improvements in health and care.**
3. **Ensuring ongoing alignment with clinical trials and national life sciences projects and supporting the growth of life sciences in the UK**

Key areas of progress

Establish NHS Genomic Networks of Excellence - to work with life science partners to rapidly adopt innovation – currently operationalising the Networks of Excellence

Working with industry to signal the needs of the NHS GMS

Evolving the NHS GMS Research Collaborative: An audit of research is underway, expected for return by the end of September, to understand the scope and breadth of the >900 research projects being supported through the NHS GMS.

Supporting the Cancer Vaccines Launch Pad, including the introduction of Cellular Pathology Genomic Centres

Supporting life sciences initiatives e.g. newborn screening, diverse data, Our Future Health

biobank uk
Improving the health of future generations

+ Our Future Health

Genomics england

Future Outlook

NHS Genomic Networks of Excellence

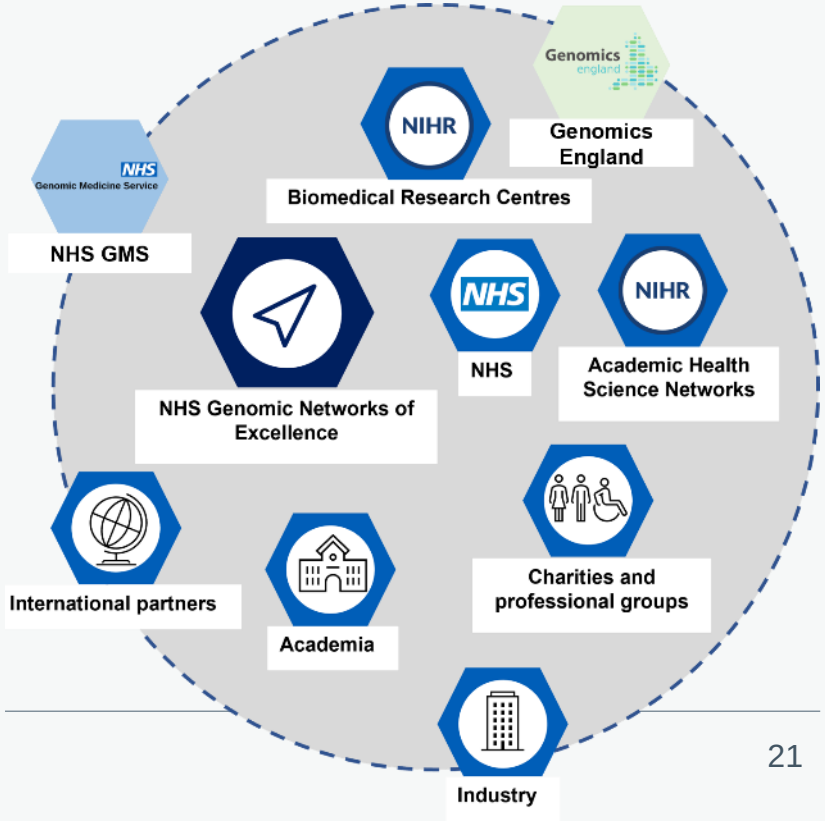
During 2023/24 the NHS will, as part of the evolving NHS GMS Alliance infrastructure, establish 'NHS Genomic Networks of Excellence'

DISCOVERY | TRANSLATION | ADOPTION | DIFFUSION

NHS Genomic Networks of Excellence will be partnerships between the NHS, academia, the third sector and industry to **generate evidence and models of adoption for new technology and testing** and, clinical and laboratory practice in defined topic areas of **strategic importance.**

NHS England has confirmed funding for:

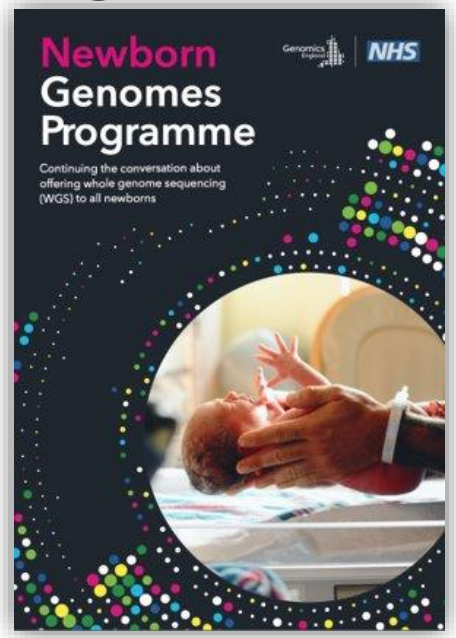
- **Prenatal** Genomic Medicine Network of Excellence
- **Circulating Tumour Biomarker** testing for rapid, effective cancer diagnostics and monitoring for Cancer of Unknown Primary, advanced metastatic Breast Cancer, Paediatric Cancer, circulating miRNA test for Germ Cell Tumours and CSF testing for primary CNS lymphoma.
- **Haemato-Oncology** NHS Genomic Network of Excellence
- **NHS Rare and Inherited Disease** Genomic Network of Excellence
- **Severe Presentation of Infectious Disease** Genomic Network of Excellence
- Improving the identification and outcomes for individuals with **inherited and acquired cardiovascular disease** NHS Genomic Network of Excellence
- **Pharmacogenomics** and Medicines Optimisation NHS Genomic Network of Excellence
- **Genomics Artificial Intelligence (AI)** NHS Genomic Network of Excellence



Early intervention: Newborn Genomes Programme

Research study **led by Genomics England** to:

- **Identify rare disease in babies -** Study will evaluate the utility, feasibility and impact of WGS newborn screening programme
- **Enable research -** improve our understanding of rare disease and how genomic testing can be integrated into newborn screening.
- **Create a lifetime resource.** Study will investigate benefits of storing an individual's genome over their lifetime.



Current progress

Following extensive engagement there are currently 523 gene condition-pairs proposed to be included in the study

All underpinned by clinical discussion and NHS England governance

Proposed conditions likely to include Bare lymphocyte syndrome C, Lymphoproliferative syndrome, Thyroid dyshormonogenesis and many others

Working with commissioning colleagues in NHS England, NICE, MHRA and others to ensure treatment and interventions are available

First site live late in 2023

Working with sites across England to operationalise study over a number of phases and begin recruitment of patients

Driving the use of precision treatments and optimising the use of medicines

Gene therapies	• Use cutting-edge technology to deliver tailor-made genetic material into a patient's cells to treat disease
New targeted treatments	• Based on an increased understanding of the genomic basis for disease & diagnosis
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Repurposing	• New indications for existing medicines
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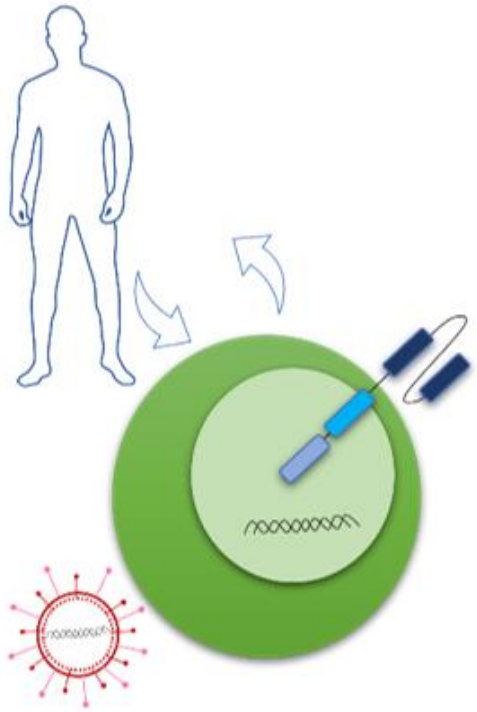
ATMPs and Gene Therapies

What we will cover:

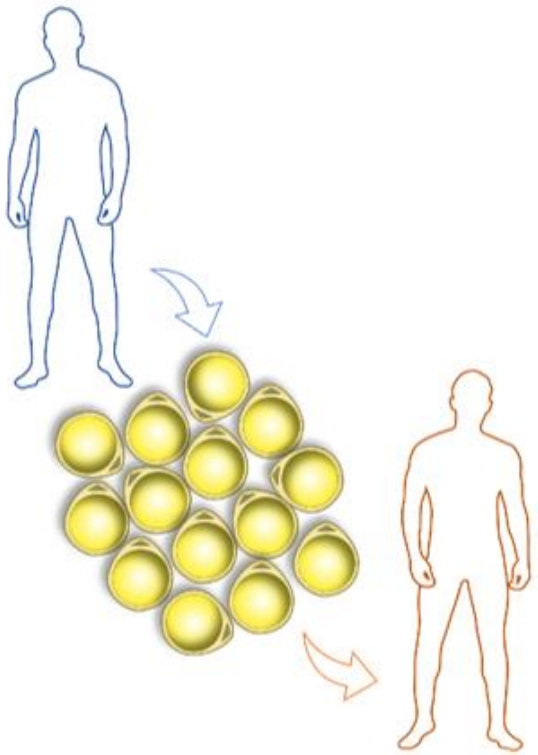
- Introduction to Advanced Therapy Medicinal Products ATMPs and gene therapies
- Gene therapies already in use by the NHS
- Commissioning of ATMPS
- Horizon scanning
- Our workplan

Different types of ATMPs



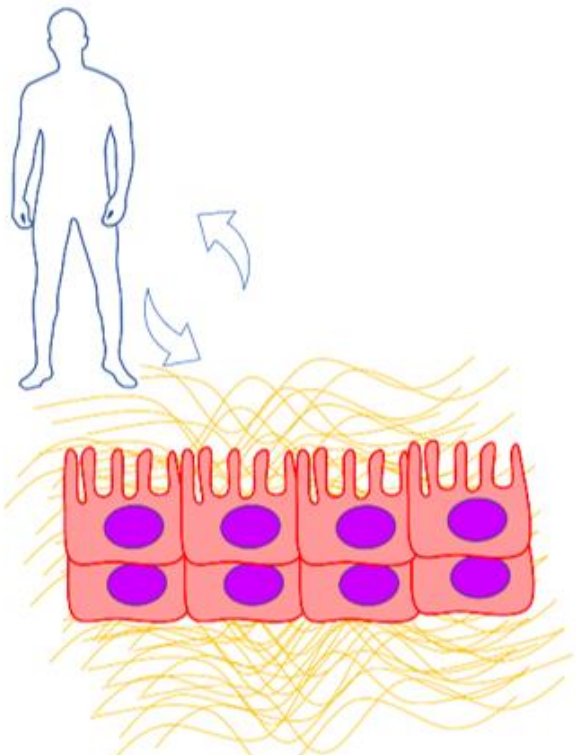
Gene Therapy Medicinal Product (GTMP)

E.g. genetically modified T cells



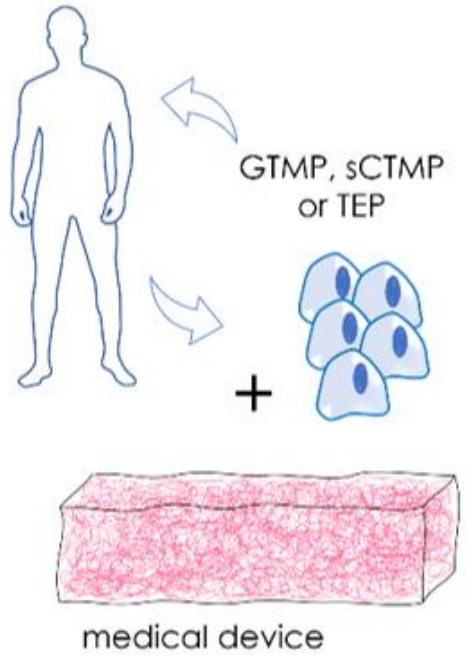
Somatic Cell Therapy Product (sCTMP)

E.g. *ex vivo* expanded adipose stem cells



Tissue-Engineered Product (TEP)

E.g. *ex vivo* expanded corneal epithelial cells attached to a fibrin support



Combined ATMP

E.g. porcine collagen scaffold seeded with autologous chondrocytes

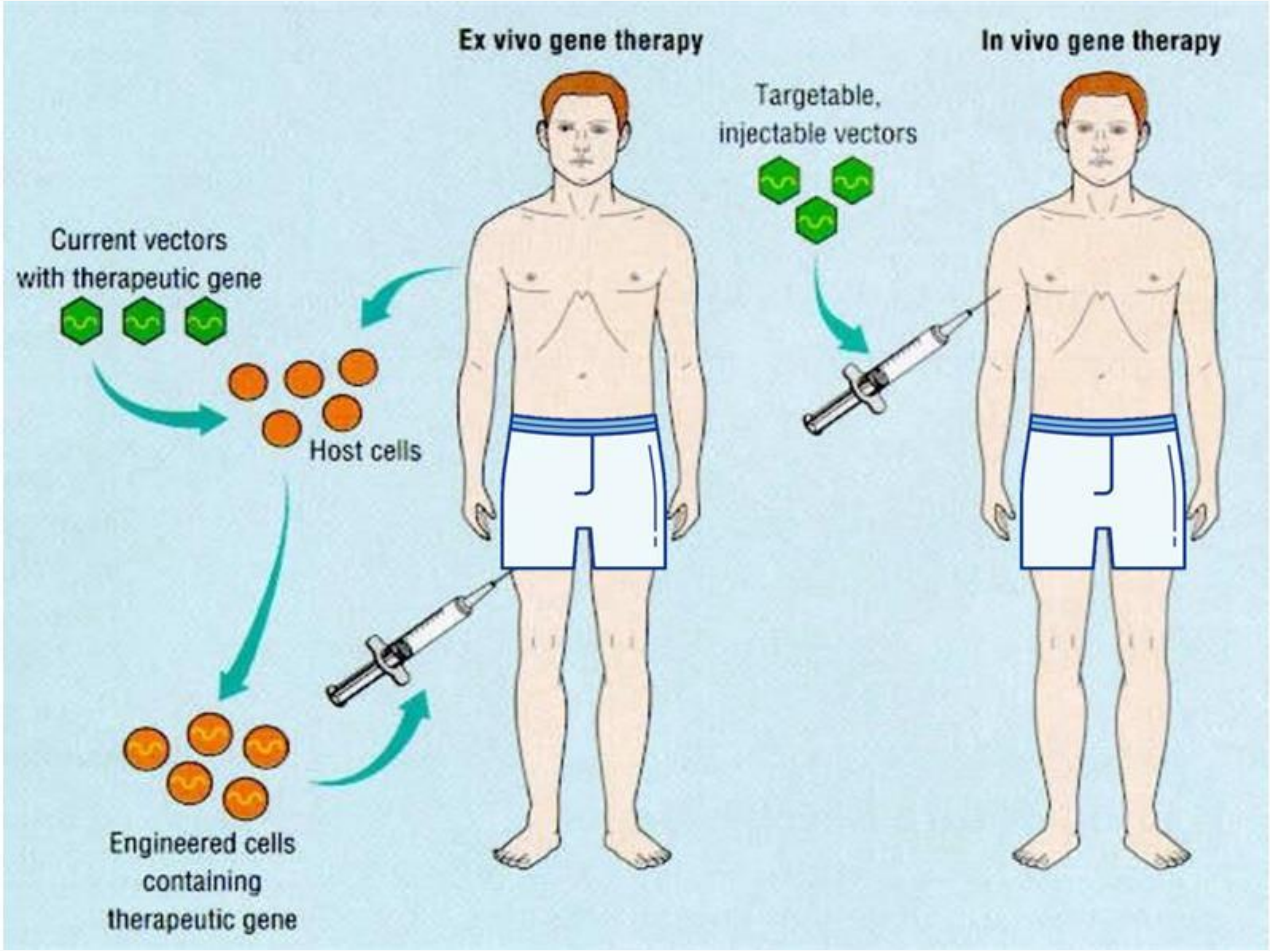
Gene Therapies

Gene therapy medicinal product means a biological medicinal product which has the following characteristics:

a. **It contains an active substance which contains or consists of a recombinant nucleic acid used in or administered to human beings with a view to regulating, repairing, replacing, adding or deleting a genetic sequence;**

b. **It's therapeutic, prophylactic or diagnostic effect relates directly to the recombinant nucleic acid sequence it contains, or to the product of genetic expression of this sequence.**

Directive 2009/120/EC amending Directive 2001/83/EC



ATMPs in England – some facts and figures

Commissioned since 2016

12 ATMPs commissioned across 14 indications

20 hospitals delivering ATMPs

36 ATMPs in 34 indications within next three years.*

700+ patients treated per year / 3000+ treated in total so far

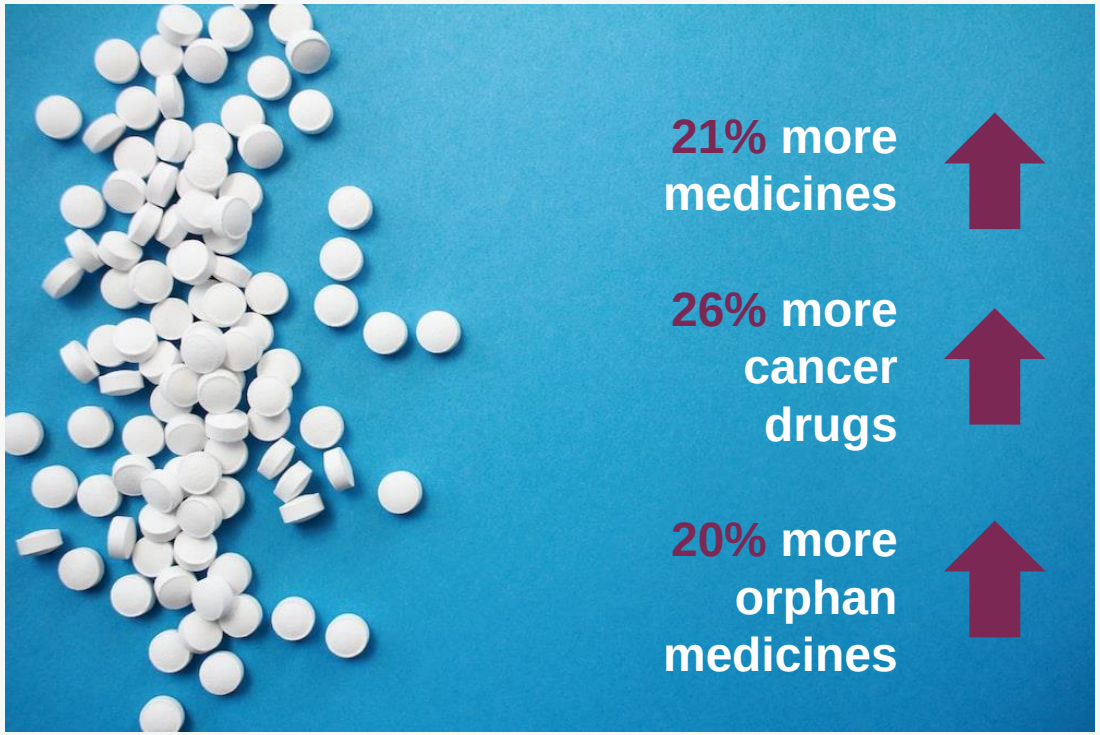
CAR-T treats the most patients (500+ per year in 2023/24)

*Considerable uncertainty due to high attrition rates for ATMPs and value proposition

Access to new medicines in England is significantly better than the European average

Reviewing 168 licensed medicines across 37 European countries, between 2018 – 2021:

- For every four treatments available in Europe, there is an **additional medicine available in England**
- **One quarter more cancer drugs** are available
- **One fifth more orphan medicines** are available



Available in England compared to the European average

Figures from EFPIA Patients W.A.I.T Indicator 2022 Survey, published April 2023. IQVIA.

Supporting rapid access to ATMPs

- The NHS in England has a track-record of using commercial capabilities to secure cell & gene therapies for NHS patients.
- Recent agreements enabling patient access have include:
  - **atidarsagene autotemcel (Libmeldy®)** – a gene therapy that offers the prospect of a normal life for children with metachromatic leukodystrophy.
  - **onasemnogene abeparvovec (Zolgensma®)** – a one-off gene therapy that can enable mobility in babies and young children with spinal muscular atrophy. One of three SMA treatments that has transformed paediatric outcomes [right]
  - **Axicabtagene ciloleucel (Yescarta®)** and **brexucabtagene autoleucel (Tecartus®)** – CAR T therapies for adults with advanced diffuse large B-cell lymphoma and B-cell acute lymphoblastic leukaemia that has returned

World's most expensive drug slashes rare disease death rate for children

Spinal muscular atrophy type 1 was deemed a death sentence before 2019 but new cutting-edge treatments are saving lives

By Michael Searles, HEALTH CORRESPONDENT
7 August 2023 • 7:00am

type 1 as an infant when her mum noticed that she appeared to have delayed development

'Gene therapy is a game changer for our son'

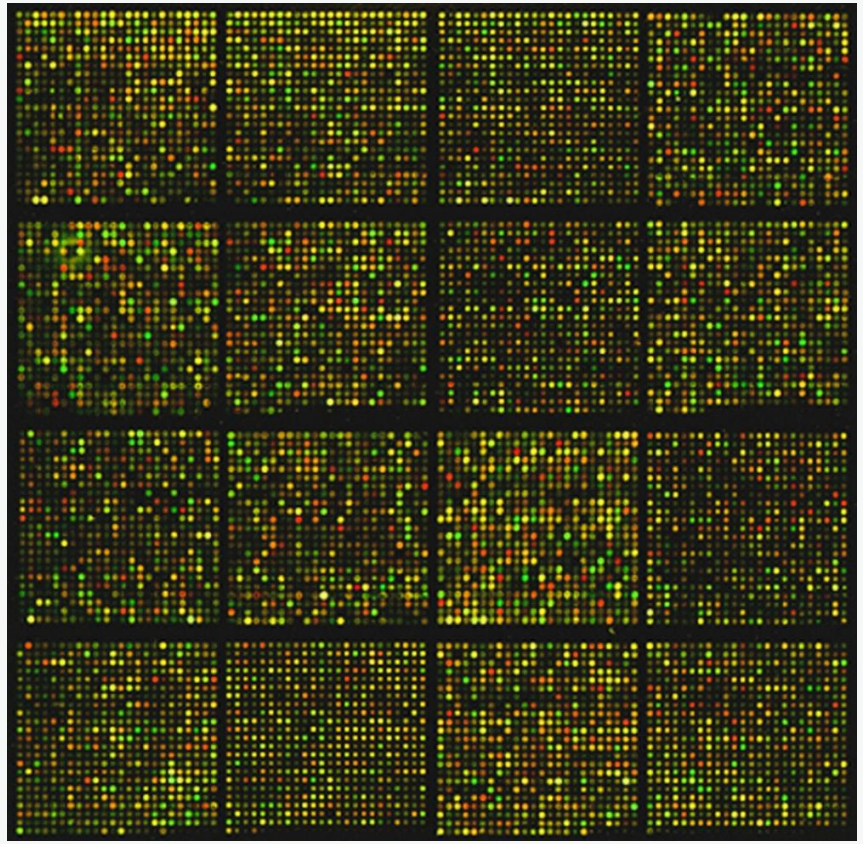
By Fergus Walsh
Medical editor

1 June

Arthur's dad says "If this treatment didn't happen, he wouldn't be around for very long"

Strategic approach to ATMPs

- Horizon scanning
- Intelligence gathering
- Engagement with NHS providers
- Pan UK Pharmacy Working Group
- Commissioned services ready as close to a **NICE decision** as possible
- Provider selection approach that takes account of the **broader** future pipeline (not just ATMPs)
- Providers may be commissioned in waves (similar approach to CAR-T) to **gradually build expertise**
- **NHS Commercial Framework provides for commercial flexibilities** to support access routes e.g. Cancer Drugs Fund and Innovative Medicines Fund
- Service costs for ATMPs if needed

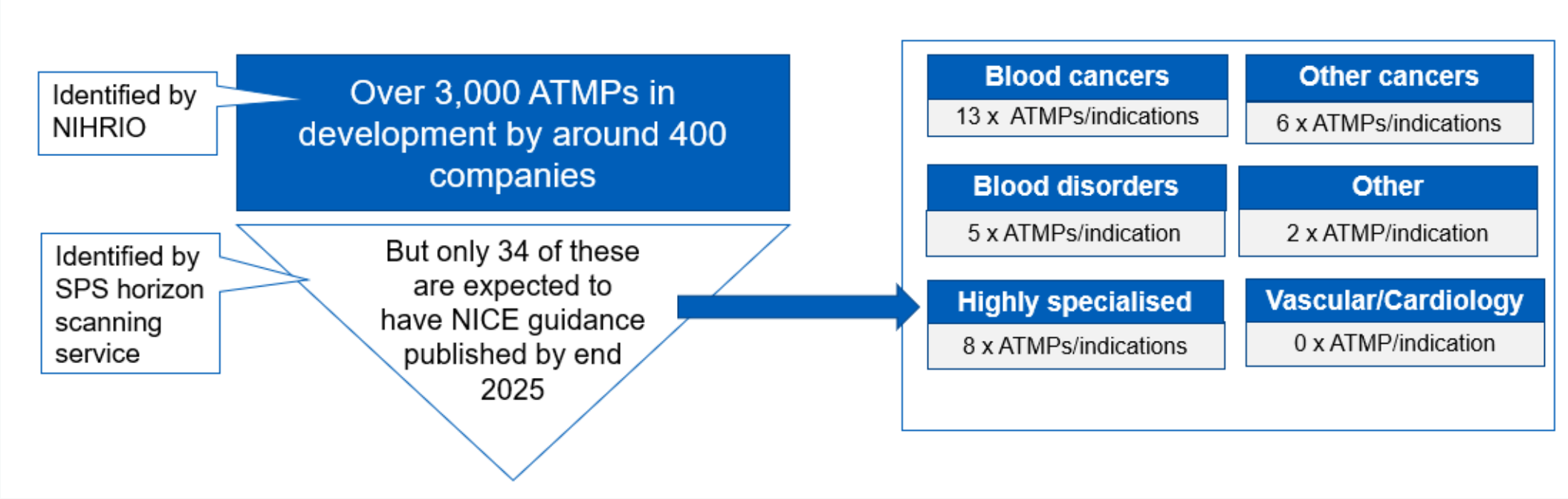


Horizon scanning

The number of ATMPs being assessed by NICE is likely to increase in the coming years.

The USA Food and Drug Administration (FDA) estimates that by 2025 they will begin to approve 10 - 20 ATMPs per year. This is consistent with the numbers seen in NICE's topic selection pipeline.

We identified 31 ATMPs in 39 indications (in April 2023) that may go through NICE assessment over the next 3 years to end of 2025.



Horizon scanning

Etranacogene dezaparvovec *(Hemgenix)*, CSL Behring
Estimated NHS availability: 2023

Blood disorders

Last reviewed: 18 January 2023, updated 6 April 2023

Regulatory status		NICE status		Commissioning
Trial status	Phase III	**NICE ID**	3812	**Commissioner** NHSE
EU orphan status	Yes	**Route**	STA	**Relevant clinical programme** Blood & Infection NPoC (F02. Specialised Blood Disorders)
Regulatory approval	**MHRA** March 2023 (conditional) **EMA** February 2023 (conditional) **FDA** November 2022	**Publication date**	20/9/23	**Type of ATMP**
Special status	**EU** PRIME, Accelerated assessment	**US** Breakthrough therapy		Gene therapy (*in-vivo*)

Indication and trial data

Indication	Description of therapy & clinical trial result
Treatment of severe and moderately severe haemophilia B (congenital Factor IX deficiency) in adults without a history of Factor IX (FIX) inhibitors See SmPC	**Mode of action** Haemophilia B is a rare inherited condition that affects the blood's ability to clot, due to faulty or low levels (<50%) of clotting FIX which leads to prolonged bleeding. Patients with severe haemophilia have <1% of normal clotting factor and those with moderate haemophilia have between 1% and 5%. Etranacogene dezaparvovec uses AAV5 vector to deliver the highly functional Padua variant of the FIX gene to liver cells, where it stimulates production of FIX that is 8 times more active than normal. **Outcomes** Number of bleeding episodes, use of FIX therapy (prophylaxis and treatment of bleeds), joint pain/impairment (haemophilic arthropathy), FIX levels, presence of FIX inhibitors, adverse effects (AEs), quality of life. **Trial data** [UK trial sites: University Hospitals Bristol, Cambridge Haemophilia and Thrombophilia Centre, The Royal London, University Hospital Southampton] **Efficacy** In the pivotal PIII open-label, single-arm HOPE-B study (n=54 males with severe or moderately severe haemophilia B), the adjusted annualised bleeding rate (ABR) decreased by 64% (p=0.0002) for all bleeds and by 73% for all FIX-treated bleeds (3.64 to 0.99; p<0.0001) over months 7 to 24. FIX activity increased from ≤2% at baseline to a mean of 39 IU/dL at 6 months and 36.7 IU/dL at 24 months. In addition, 96.3% treated with etranacogene discontinued FIX with mean annual FIX consumption reduced from 257,338.8 to 9,751 IU/year/patient. Etranacogene was also reported effective in patients with pre-existing neutralising antibodies. In another open-label single-arm PIIb trial (n=3), mean FIX activity increased from ≤1% to mean 30.6% at 6 weeks and mean 36.9% at 3 years with sustained reduction in bleeding or need for FIX replacement. Complete elimination of bleeds occurred in 2/3 participants. **Safety** In the PIII HOPE-B study, etranacogene \zand included elevation of transaminase levels managed successfully with a course of steroids (17%), infusion-related reactions (13%), headache (13%) and influenza-like symptoms (13%).

Current treatment pathway and other recently launched or new treatments

There is currently no cure. Lifelong FIX replacement (injected 1-2 times a week) prevents bleeds and allows the person to grow up with normal joints. Its also used to treat bleeds. Concizumab, fitusiran, marstacimab and another gene therapy fidanacogene elaparvovec are in development (all due ≥2023).

Etranacogene dezaparvovec *(Hemgenix)*, CSL Behring
Estimated NHS availability: 2023

Blood disorders

Product administration, delivery, handling and service implications

Starting material Insect cell culture and baculovirus expression vector system	**Delivery mode** Intravenous (IV) infusion at a constant infusion rate of 500mL/hour. Prior to infusion, dilute vials in sodium chloride 0.9% (500mL for patients weighing <120kg, and 2 x 500mL for patients weighing ≥120kg).	**Dose and duration of therapy** 2×10^{13} genome copies/kg as a single dose
Presentation Concentrate for solution for IV infusion in vials containing an extractable volume of not less than 10mL at a concentration of $\geq1\times10^{13}$ genome copies/ml *Manufacturing site:* USA. After manufacturing product will be packed in Marberg, Germany and shipped to UK sites.	**Pre- treatment medication** Single dose of short-acting FIX (40 IU/kg) to provide sufficient FIX coverage for 2 to 3 days post treatment with etranacogene + additional doses if required at clinician's discretion in first weeks post-infusion	**Handling and storage (including shelf life)** Storage and shelf life: Store in a refrigerator in original packaging to protect from light. Once diluted, can be stored at 15-25°C in infusion bag (protected from light) for up to 24 hours. *Shelf life:* 20 months *Genetically modified microorganism (GMM) class* = Biosafety level 1
Infrastructure requirements Facilities for storage and temperature monitoring. Possible that critical care facilities may be needed, as well as links with hepatology. Pharmacy laboratory aseptic facilities (isolator) required for dose preparation. **Service implications** There may be additional costs for staff training, patient counselling and with preparation, storage and disposal and co-ordination of care.		**Patient monitoring and follow up** Likely monitored in hospital for 24 hours after dosing. Weekly FIX and LFTs for first 12 weeks (with results processed at same laboratory). Course of prednisolone if alanine transaminase (ALT) level ≥ normal limits or ≥2-fold increase over baseline with weekly ALTs during prednisolone tapering phase. Regular alpha-fetoprotein level testing and annual abdominal ultrasound in patient's with pre-existing risk factors for hepatocellular carcinoma.

Proposed population in England (see assumptions)

Prevalent population ~ 210 to 215 adult men (with FIX<1 IU/dL or FIX 1-5)	**Incident population** ≤1/year	**Estimated population to be treated** ≤1/ year (from incident population) and ~55 (from prevalent population) if treating 25% of eligible patients

Uncertainties Of the adult men with FIX levels 1-5 IU/dL, the exact number with FIX1-2 IU/dL is not available. The estimate of the prevalent population is largely based on the number with FIX<1 IU/dL (n=211 in UK in 2021/22). Uptake is estimated at 25% based on opinions of clinicians who manage patients with haemophilia B. This will depend largely on patient choice.

Patient pathway

Standard of care at relevant stage of pathway Prophylactic FIX replacement therapy. Brands include Haemonine, Replenine and BeneFIX [active patent not identified for these]	**Providers** Comprehensive care centres (haemophilia)
	Potential number of providers 17 (maximum, likely fewer)

Diagnostics / Genomic testing
Clinical history and assessment followed by blood coagulation tests (aPTT, specific factor IX activity level) are carried out. Once an individual is diagnosed with haemophilia B, the specific mutation in the F9 gene responsible for causing haemophilia may be identified – this may assist in determining an individual's risk of developing an inhibitor and identify carriers within families. Genetic testing is required for diagnosis. Regular inhibitor screens are also necessary.

Workplan 2023 – Live Topics

Topic	Indication	POC	Expected launch
Eladocagene	Aromatic L-amino acid decarboxylase deficiency	Highly specialised	Launched
Etranacogene dezaparvovec (CSL Behring)	Prevention of bleeding in adult men with severe to moderately severe haemophilia B who are currently using Factor IX (FIX) prophylaxis therapy or who have had life threatening or serious recurrent bleeding episodes	Blood and Infection	Early 2024
Exagamglogene autotemcel	Beta thalassaemia	Blood and Infection	Early 2024
Exagamglogene autotemcel	Sickle cell disease	Blood and Infection	Early 2024
Beremagene geperpavec	Treatment of wounds due to recessive or dominant dystrophic epidermolysis bullosa (RDEB/DDEB) in adults and children aged ≥6 months	Highly specialised	2024

Workplan 2024- Expected launch 2025 & beyond

Topic	Indication	POC	Comment
Fordadistrogene movaparvovec	Duchenne muscular dystrophy	Women and children	Expected availability 2025
Lifileucel	Malignant melanoma	Cancer	Expected availability 2025
Delandistrogene moxeparvovec	Duchenne muscular dystrophy	Women and children	
Botaretigene sparoparvovec	X-linked retinitis pigmentosa	Trauma	
Afamitresgene autoleucel	Sarcoma	Cancer	
Autologous human chondrocytes in vitro expanded (Novocart Inject)	Traumatic cartilage defects of the knee	Trauma (orthopaedics)	
Nadofaragene firadenovec	Bladder cancer	Cancer	
Tabelecleucel / tab-cel (Ebvallo)	Patients with Epstein-Barr Virus positive post-transplant lymphoproliferative disease (EBV+ PTLD) who have received at least one prior therapy.	Cancer	Delayed from 2022 launch.
Murcidencel (DCVax-L)	Newly diagnosed glioblastoma following standard of care (surgical resection, external beam radiation therapy and initiation of temozolomide)	Cancer	Delayed from 2023 launch.
Lenadogene nolparvovec	Leber hereditary optic neuropathy	Trauma (Ophthalmology)	Suspended
VX880	Type 1 diabetes with impaired hypoglycaemic awareness and severe hypoglycaemia	Internal Medicine (ICBs)	

The NHS is ready to engage on new treatments

Come and speak to us to learn more about new product commissioning in the NHS.

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