PM SOCIETY

# Rare access, the varying pace of change

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Market Access

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The PM Society is a **Not-for-profit** organisation that believes excellent healthcare communications leads to better outcomes for patients.

- Established over 40 years ago
- Over 230 member companies
- Awards, Training, Events, Interest Groups

The PM Society has the following purpose:

✓ Supporting organization's and people in healthcare

✓ Recognising excellence and promoting best practice

✓ Providing education and development

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# Why join us?

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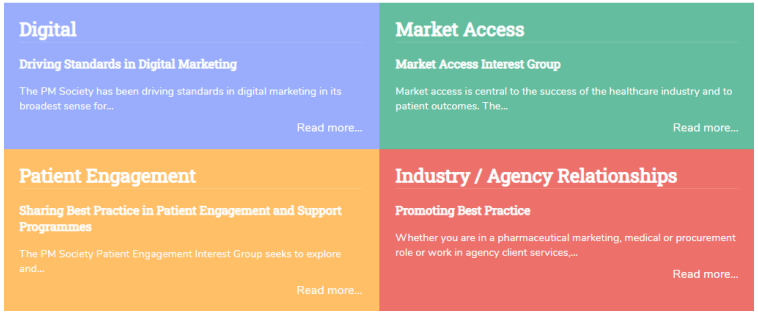


1

**Membership spans the industry:**
- A powerful industry voice
- Influence best practice and excellence

2

**Active interest groups:**
- Collaborate & learn with peers
- Deliver great events & education

3

**Knowledge and expertise:**
- Promoting best practice for over 40 years
- Access to people & resources

4

**Unique networking:**
- Leaders from right across the sector
- Awards & events

5

**Capability building:**
- Educational events and training
- Hear from industry leaders

6

**Instil Best Practice in your team:**
- Hear from award winners
- Broaden horizons

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# House keeping


- Microphones will be muted during the webinar
- Please place all questions in the Q&A section on the zoom call
- We will address questions at the end and follow-up as necessary
- The recording will be available on the portal afterwards

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# Webinar Moderators

**Andrew Mumford**
Principal Consultant

a.mumford@initiateconsultancy.com

**Bethany English**
Market Access Analyst

b.english@initiateconsultancy.com

**Craig Bradley**
Head of Marketing - Diabetes & Internal Medicine at Takeda UK

craig.bradley@takeda.com

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Unit 1 City Business Centre
6 Brighton Road
Horsham
West Sussex
RH13 5BB

Tel: 01403 264898



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# Initiate.

**The global market access strategy consultancy working with novel products designed to help people living with rare or life-limiting disorders**

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Synaptiq health

# Rare Access, the varying pace of change

*Andrew Mumford*

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# Agenda

| Time | Agenda |
|---|---|
| 4:05 pm<br>*15 min* | **Introduction and Survey**<br>*Andrew Mumford, Principal Consultant – Market Access, Initiate Consultancy* |
| 4:20 pm<br>*25 min* | **The pace of reimbursement in cell and gene therapies, how is the UK performing compared to Europe**<br>*Bethany English, Analyst – Market Access, Initiate Consultancy* |
| 4:45 pm<br>*25 min* | **Panel Discussion – Industry reflections on the varying pace of change**<br>*Led by Craig Bradley, Head of Marketing – Diabetes & Internal Medicine, Takeda* |
| 5:10 pm<br>*20 min* | **Q&A**<br>*Andrew Mumford, Principal Consultant – Market Access, Initiate Consultancy* |

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# Disclaimer

**Compliance**: We comply with all relevant codes of conduct including; ABPI, Data Protection Act, Market Research Society, European Pharmaceutical Market Research Association (EphMRA), British Healthcare Business Intelligence Association (BHBIA).

**Anonymised research:** Your comments from the questionnaire in this webinar will be anonymised and consolidated together with other respondents.

**Right to withdraw**: You have the right to withdraw from the questionnaire at any time or to decline to answer any particular questions you do not feel comfortable answering or if you feel the answer to the question would disclose confidential information.

**Session recording:** The session will be recorded and accessible in the PM Society web page.

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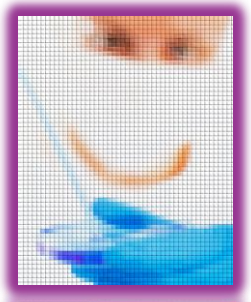
# Webinar Context: Advanced Therapy Medicinal Products

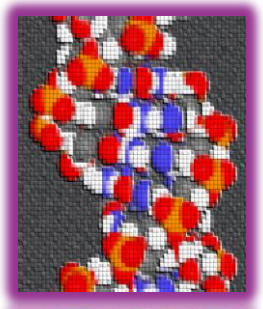
EMA Definition:

## Cell Therapies



Cells subject to substantial manipulation or not intended to be used for the same essential function(s) in the recipient and the donor used to treating, preventing or diagnosing a disease

## Gene Therapies



Contains recombinant nuclei acid, used to regulating, repairing, replacing, adding or deleting a genetic sequence

## Tissue Engineered Products

Contains engineered cells or tissues, used to regenerating, repairing or replacing a human tissue

## Combined products

Contains engineered cells or tissues with one or more medical device

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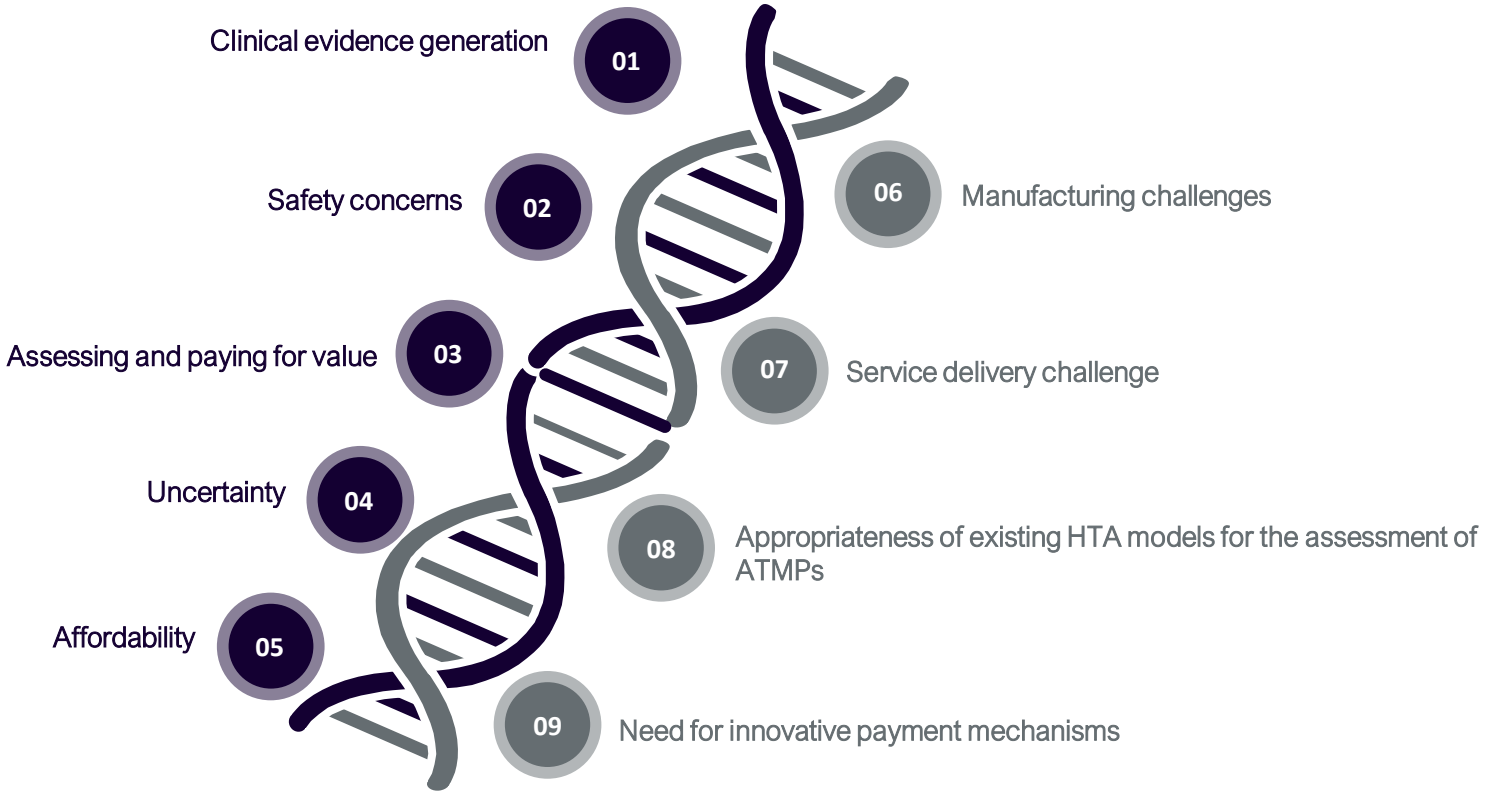
# ATMPs challenge to demonstrate effectiveness, cost-effectiveness and value within the HTA process

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# Key opportunities and challenges for gene therapies

- The development of gene therapies represents a new frontier in science with the potential to help many patients with serious or fatal conditions

- Evidence generation is problematic – Very small patient populations and the novel aspects of gene therapy make it difficult to generate robust clinical evidence needed by decision-makers

- Value assessment and budget impact difficulties – Uncertainty regarding clinical outcomes further complicates the challenges of assessing the value of potential “cures”

- Affordability concerns – Gene therapies heighten concerns about the affordability of emerging treatments under existing paradigms of pricing and payment



Reference: Marsden et al 2017

# Key considerations for gene therapy budgets

Fixed yearly budgets can impact payer's willingness to pay and lead to displayed therapies

Fixed cycle for yearly budget

Cost mitigation

Increasing premium and patient cost sharing is likely to be unsustainable in the long term

Therapy affordability

Cost assumption before benefits are delivered

Growth in the cell and gene therapies market will increase competition for a budget with limited growth

Gene Therapy Budget Impact factors

Budget impact can be lessened by linking payments to clinical outcomes from treated patients

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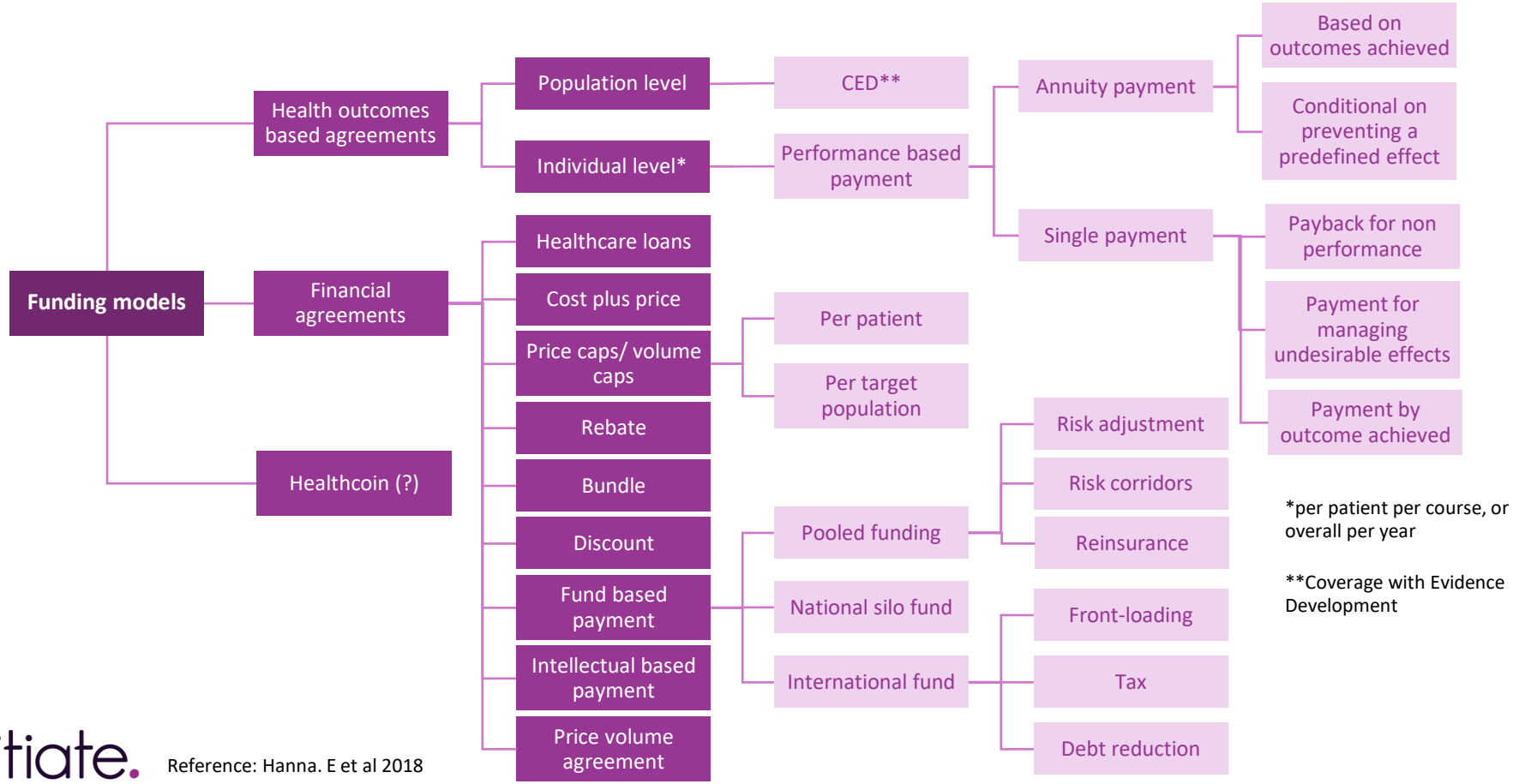
# Funding models for breakthrough therapies




*per patient per course, or overall per year

**Coverage with Evidence Development

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Reference: Hanna. E et al 2018

# Affordability of novel high budget impact therapies

Approaches and 'Tools' used across Europe to ensure affordability of novel budget impact therapies:

| Country | Affordability Threshold | Cap on Volume or Price Volume Agreement | Restriction on population | Special funding for expensive drugs | Limit in pharma expenditure increase / patient contributions | Informal Guidance to Prescription |
|---|---|---|---|---|---|---|
| Germany | | ✓ | | | | ✓ |
| France | | ✓ | ✓ | | ✓ | |
| England | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ |
| Italy | | ✓ | ✓ | ✓ | ✓ | |
| Spain | | ✓ | ✓ | | ✓ | ✓ |
| Sweden | | | ✓ | | | |
| Netherlands | | ✓ | ✓ | | | ✓ |

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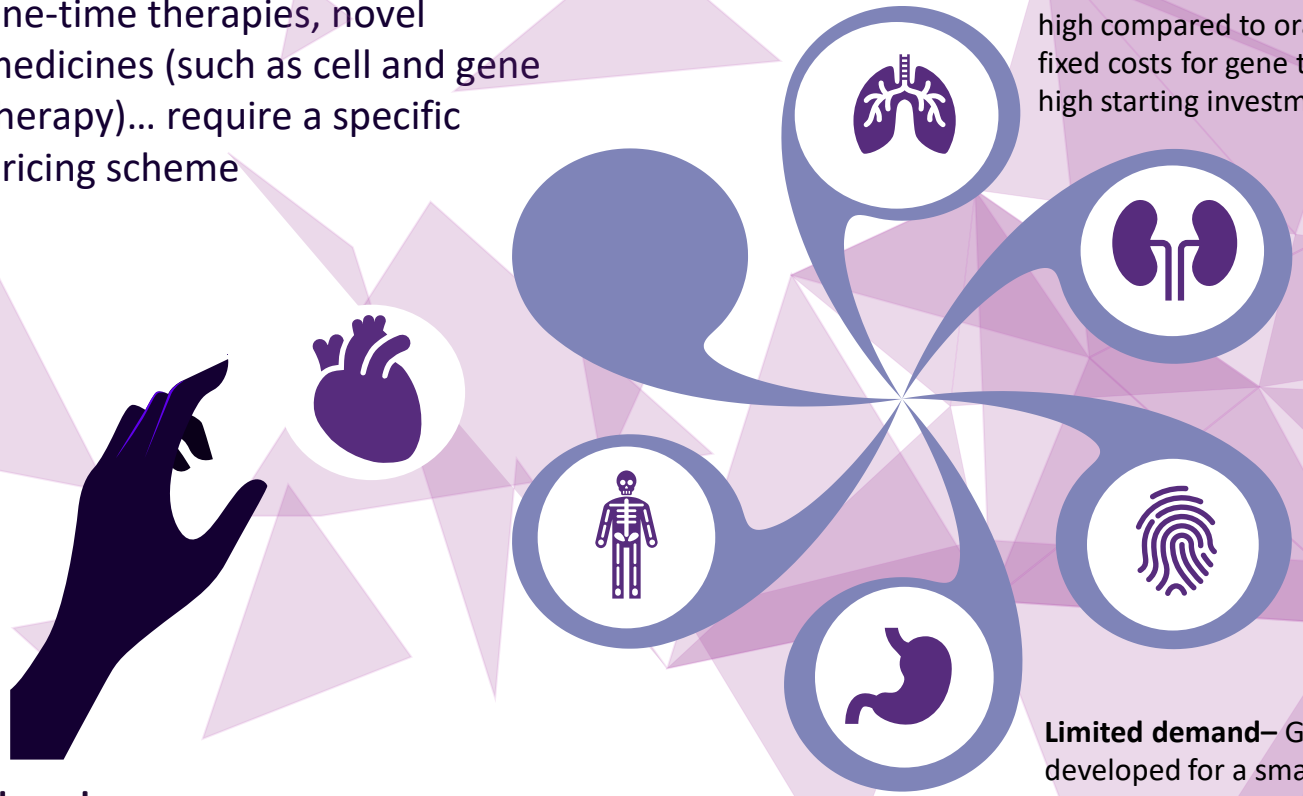
Reference: Flume et al 2018

# Gene therapy pricing constraints

Orphan drugs, rare disease and one-time therapies, novel medicines (such as cell and gene therapy)… require a specific pricing scheme



**Investment** – Gene Therapy manufacturing costs are high compared to oral solids or biologics. R&D and fixed costs for gene therapy development require a high starting investment

**High risk –**Novel therapies with failure risk during development and post-launch

**Regulatory and HTA Requirements** – advanced therapies with very complex production are not taken into account within the strict quality, safety and efficacy standards currently enforced by regulatory bodies

**Limited demand**– Gene therapies are often developed for a small target population, relying on high prices to recover the significant R&D and production investments

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# The pace of reimbursement in cell and gene therapies, how is the UK performing compared to Europe

*Bethany English*

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# The next decade in the UK will see gene therapies expanding from patients in clinical trials to embedment in the NHS


**~ 200 patients**
Mainly treated in clinical trials

**100 patients**
Receiving licensing products

**~ 5,000 patients**
Routine delivery of gene therapies

**~ 10,000 patients**
Delivery of gene therapies embedded in the healthcare system

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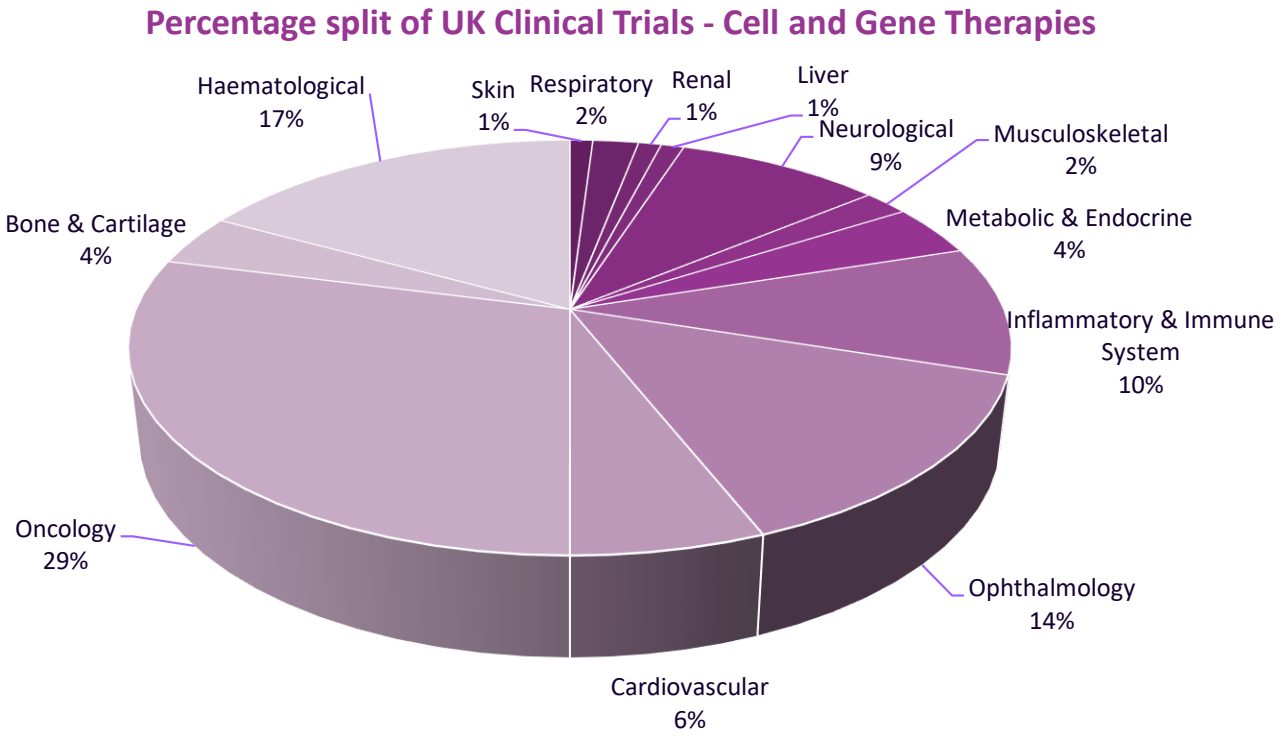
Reference: Catapult 2019

# The UK has currently 85 on-going cell & gene therapy (GT) trials

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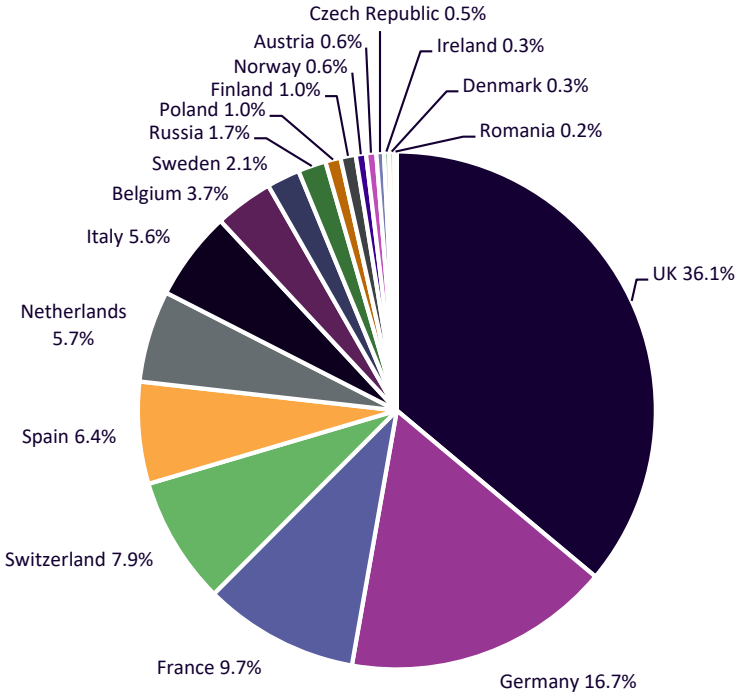
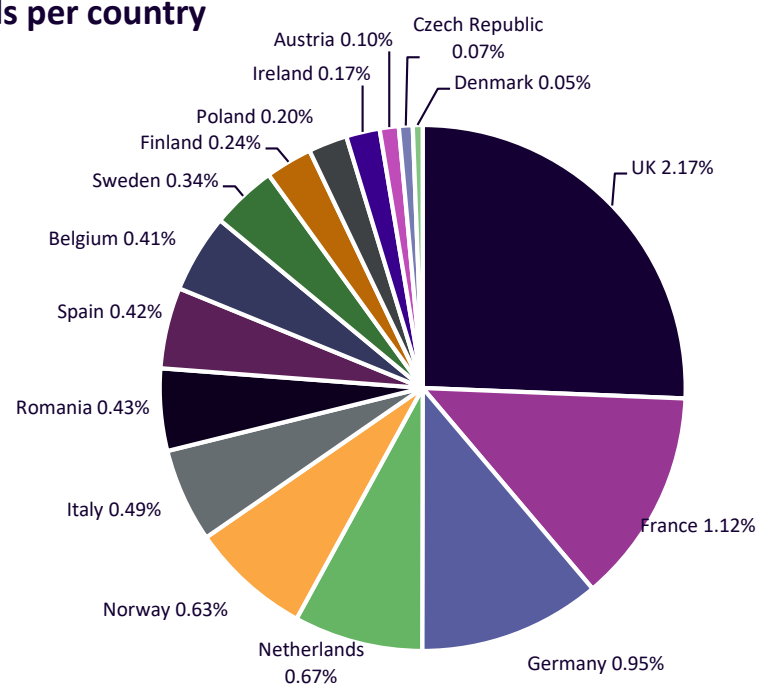
Reference: Catapult 2019

# Historically, the UK leads Europe both in total number of trials and proportion of gene therapy to other trials

**European countries with Gene Therapy Clinical Trials**




**% Gene Therapy Clinical Trials compared to Total Clinical Trials per country**

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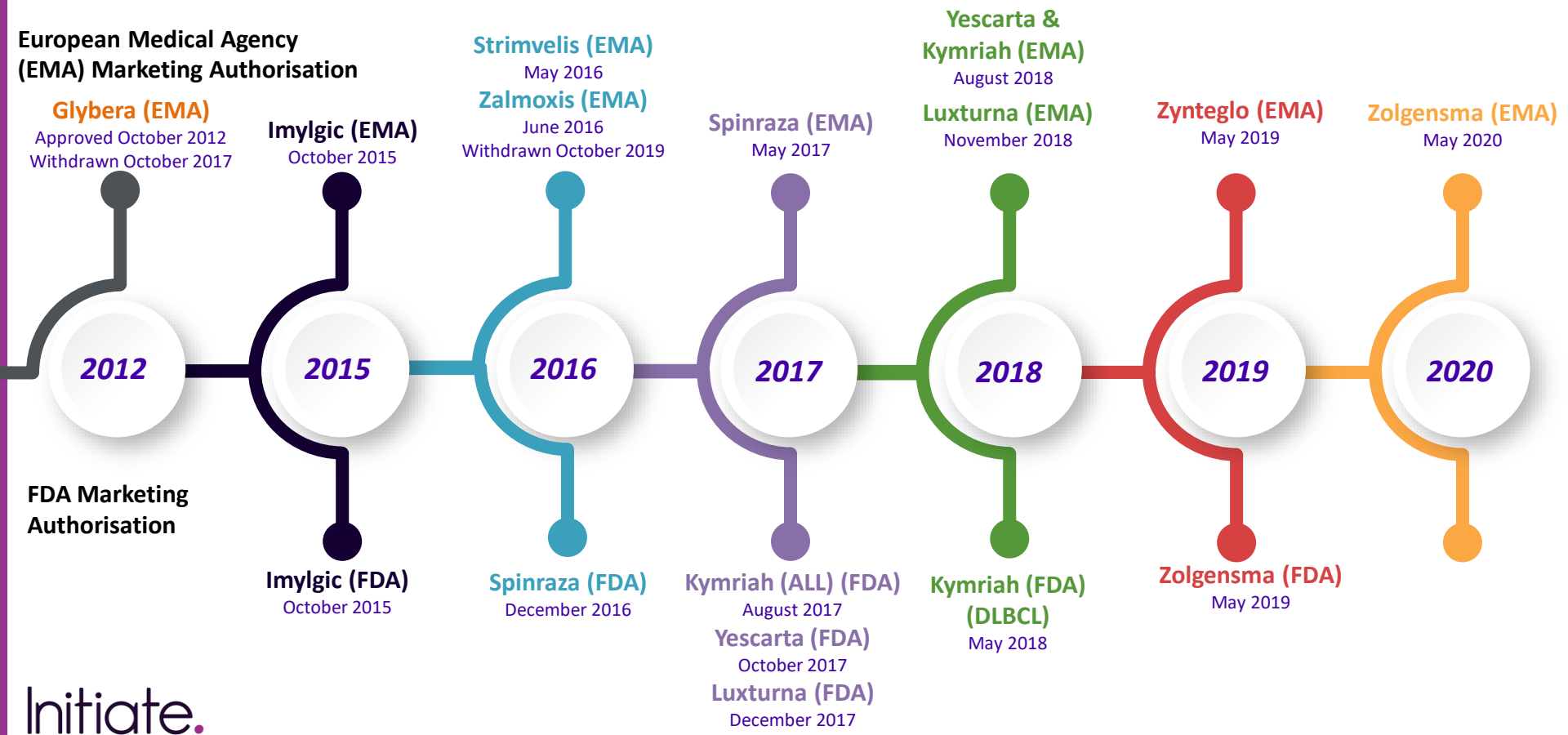
Reference: Gene Therapy Trials Worldwide. September 2019. The Journal of Gene Medicine. John Wiley & Sons.

# Gene therapies approval timeline (EMA and FDA)

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# Clinical evidence submitted for regulatory approval

| Treatments | Glybera | Imvigic | Strimvelis | Zalmoxis | Spinraza | Kymriah | Kymriah | Yescarta | Luxturna | Zolgensma | Zynteglo |
|---|---|---|---|---|---|---|---|---|---|---|---|
| Drug | alipogene tiparvovec | talimogene laherparepvec | autologous CD34+ enriched cell fraction | HSV-Tk | nusinersen | tisagenlecleucel | tisagenlecleucel | axicabtagene ciloleucel | voretigene neparvovec | onasemnogene abeparvovec-xioi | Autologous CD34+ cells encoding βA-T87Q-globin gene |
| Manufacturer | UniQure Biopharma / Chiesi | BioVex Limited | MolMed | MolMed | Biogen | Novartis Europharm Limited | Novartis Europharm Limited | Gilead Sciences, Inc | Spark Therapeutics | AveXis (Novartis) | Apceth Biopharma |
| Marketing Authorisation Holder | UniQure Biopharma / Chiesi | Amgen Europe B.V. | Orchard Therapeutics | MolMed SpA | Biogen Netherlands B.V. | Novartis Europharm Limited | Novartis Europharm Limited | Kite Pharma EU | Novartis Europharm Limited | Novarits | Bluebird Bio |
| Indication | Hereditary lipoprotein lipase deficiency (LPLD) | Unresectable melanoma regionally or distantly metastatic | Severe combined immunodeficiency (SCID) due to ADA deficiency | Hematopoietic Stem Cell Transplantation Graft vs Host Disease | Spinal Muscular Atrophy (SMA) | Relapsed or refractory B-cell acute lymphoblastic leukaemia <25 ys old | Relapsed or refractory diffuse large B-cell lymphoma (DLBCL) | Refractory DLBCL and PMBCL | Inherited retinal dystrophy | Spinal Muscular Atrophy (SMA), <2 years old | Transfusion-dependent beta-thalassemia |
| Clinical Trails (pivotal) | Three trials | Phase III trial | AD1116511, Phase II Study | TK008 Phase III Trial | ENDEAR Phase III (Type 1), CHERISH Phase III (Type 2,3) | ELIANA Phase II trial | JULIET Phase II Trial | ZUMA-1 Phase II trial | Study 301/302 phase III Study | STR1VE, Phase III Study | Northstar, Phase I/II study |
| Arms | Single-arm | Randomized | Single-arm | Randomized | Randomized | Single-arm | Single-arm | Single-arm | Randomized | Single-arm | Single-arm |
| Sample size | 35 (in total) | 295 treated | 18 | 170 | 121-126 | 75 treated | 111 treated | 101 treated | 21 (tx) 10 (control) | 20 | 22 |
| Clinical primary endpoints | Fasting median plasma triglyceride | Durable Response Rate | Overall survival | Disease-free survival | proportion of HINE motor milestone responders, change in HFMSE | Overall remission rate | Overall remission rate | Overall remission rate | Bilateral performance on mobility test | Independent sitting and event-free survival | Transfusion independence |

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# Challenges for gene therapy access across European countries

- **Great variability in approaching and handling affordability**
- **NICE is the only agency that publish a meaningful willingness to pay threshold**

- **No one solution used consistently across countries**
- **Number of tools used in combination in each country**

**Pharma companies need to move from ‘competitive intelligence’ to a broader ‘budget impact intelligence’ to account for future affordability issues**

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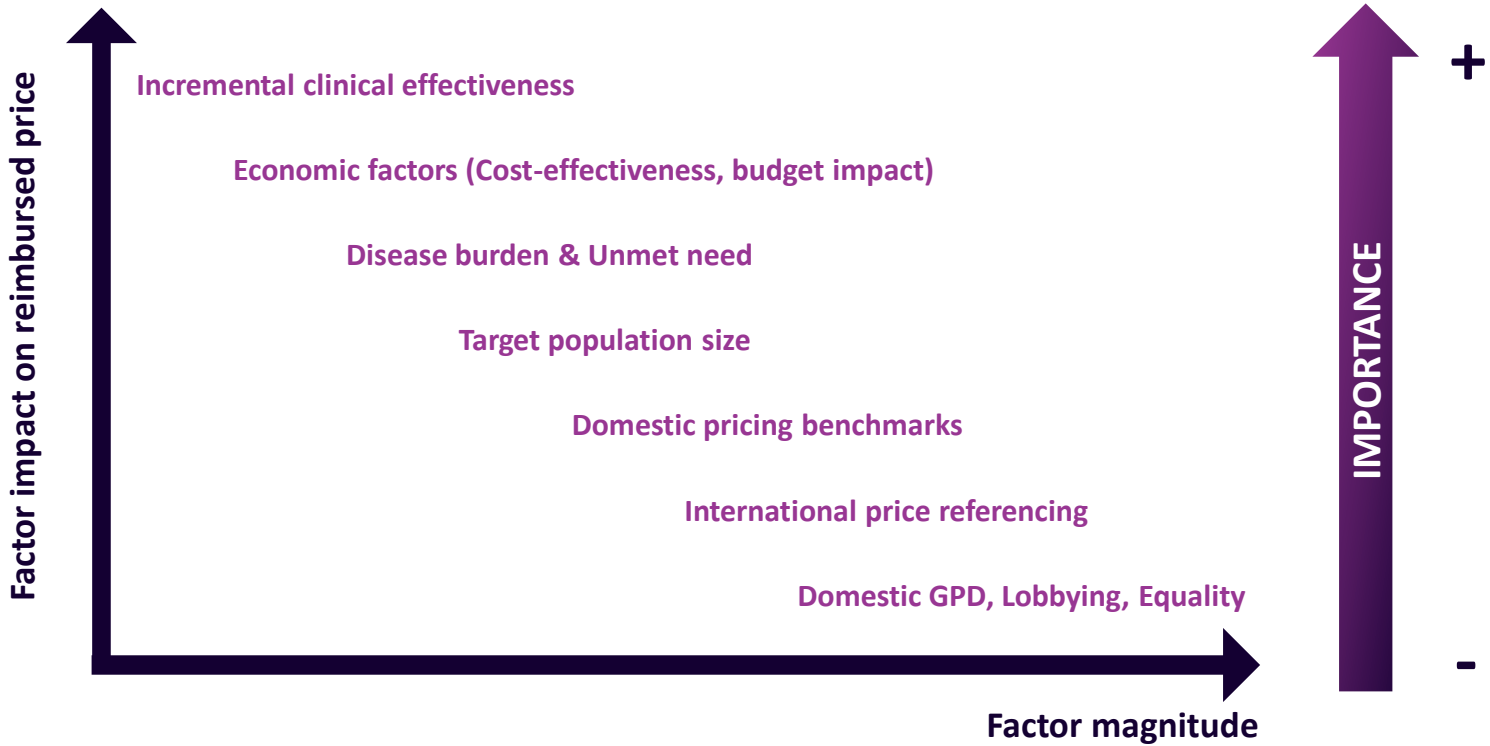
Reference: Flume et al 2018

# Factors impacting willingness to pay and reimbursed price potential

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Reference: Catapult 2019

# Determinants in reimbursement decision in the EU5

## 1st order determinants

Clinical effectiveness of the new therapy vs a relevant comparator in the given market

## 2nd order determinants

**Cost-utility**

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**Added benefit:**
- Budget impact
- Efficiency frontier
- International price referencing (EU 15)

**No added benefit:**
- Domestic comparator price

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**ASMR 1-3:**
International price referencing (EU 4)
+ Cost utility

**ASMR 4-5:**
- Domestic comparator price
- Price-volume agreements

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Budget impact
+ International price referencing
- Cost-utility (minor determinant)

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Reference: Catapult 2019

# Reimbursement in EU5

## Cell and Gene Therapies reimbursement status in EU5

| Name | UK (England & Wales) | Germany | France | Italy | Spain |
|---|---|---|---|---|---|
| **Glybera*** | Not commercialised | Non-quantifiable added benefit | Not recommended | Not commercialised | Not commercialised |
| **Imylgic** | Reimbursed- patient access schemes | No-added benefit (inappropriate comparator)- handled as procedure | Not evaluated | Not commercialised | Reimbursement authorisation denied |
| **Strimvelis** | Reimbursed- patient access schemes | Reimbursed for hospital use with managed entry agreements | Not evaluated | Reimbursed for hospital use with managed entry agreements (limited risk-share scheme with payback in case of treatment failure) | Not authorised; not commercialized |
| **Zalmoxis*** | Not reviewed yet | Reimbursed for hospital use with managed entry agreements | Not reviewed yet | Reimbursed for hospital use with managed entry agreements: flat price per patient | Reimbursed for hospital use with managed entry agreements: flat price per patient |
| **Spinraza** | Reimbursed access – restricted | Reimbursed access – all patients | Reimbursed access – all patients | Reimbursed access – all patients | Reimbursed access – all patients (subject to clinical criteria in type IIIb) |
| **Kymriah** | Reimbursed via cancer drugs fund | Reimbursed, pay-for-performance | Positive reimbursement decision- available through post ATU program | Reimbursed; payment by results (ALL); obligatory discount (DLBCL) | Reimbursed; payment by results |
| **Yescarta** | Reimbursed via cancer drugs fund | Reimbursed - G-BA assessed | Positive reimbursement decision- available through post ATU program | Reimbursed; payment by results (ALL); obligatory discount (DLBCL) | Reimbursed; payment by result |
| **Luxturna** | Reimbursed- patient access schemes | Reimbursed - under G-BA assessment | Positive reimbursement decision- available through post ATU program | P&R procedure not yet completed | Authorized, not commercialized yet |
| **Zynteglo** | Currently being appraised by NICE | Reimbursed- value-based payment agreement | Ongoing evaluation for reimbursement | Currently being appraised | Not authorised; not commercialized |
| **Zolgensma** | Pending decision- not defined as therapeutically critical | Reimbursed for a few patients by AKA | Temporary authorisation (ATU program) | Not commercialised until EMA approval | Not commercialised until EMA approval |

Not reimbursed

Pending decision

Reimbursed

*Discontinued commercialization

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# Reimbursement in EU5

## Cell and Gene Therapies reimbursement status in EU5**

| Name | UK (England & Wales) | Germany | France | Italy | Spain |
|---|---|---|---|---|---|
| Glybera* | - | €1 million per treatment | - | - | - |
| Imylgic | £ 1,670 per vial (max price £ 73,480) | - | - | - | - |
| Strimvelis | £ 504,900 | € 594,000 | - | € 594,000 | - |
| Zalmoxis* | - | € 130,000 per infusion | - | € 149,000 EUR per infusion (no VAT) | € 149,000 EUR per infusion (no VAT) |
| Spinraza | £ 450,000* first year, £290,561 annually thereafter | € 285,236 - 380,314 per year | € 210,000-280,000 per year | € 210,000-280,000 per year | € 210,000-420,000 per year |
| Kymriah | £ 282,000 | € 320,000 | € 297,666 | € 300,000 | € 307,200 |
| Yescarta | £ 280,451 | € 327,000 | € 327,000 | € 327,000 | € 313.920 |
| Luxturna | £613,000 | € 345,000 per eye | € 345,000 per eye | - | - |
| Zynteglo | - | €315,000 first year & for 4 following years if results | - | | - |
| Zolgensma | - | €1.9 million | €1.9 million- discounts applied retroactively | - | - |

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*Discontinued commercialization

**Prices are presented by patient and year unless specified. Prices displayed are mostly ex-factory published prices, they are subject to non-disclosed discounts with each NHS.

# Gene therapies reimbursed in the European market

## Cell and Gene Therapies available in European countries (1/2)

| Country | Glybera* | Imylgic | Strimvelis | Zalmoxis* | Spinraza | Kymriah | Yescarta | Luxturna | Zynteglo |
|---|---|---|---|---|---|---|---|---|---|
| Austria | × | - | × | × | ✓ | ✓ | - | ✓ | × |
| Belarus | × | - | × | × | × | × | - | × | × |
| Belgium | × | - | × | × | ✓ | ✓ | × | × | × |
| Bulgaria | × | - | × | × | × | × | - | × | × |
| Croatia | × | - | × | × | ✓ | - | - | - | × |
| Cyprus | × | - | × | × | ✓ | - | - | - | × |
| Czech Republic | × | - | × | × | ✓ | ✓ | ✓ | × | × |
| Denmark | × | - | × | × | ✓ | ✓ | × | × | × |
| England & Wales | × | ✓ | × | × | ✓ | ✓ | ✓ | ✓ | × |
| Estonia | × | - | × | × | × | - | - | - | × |
| Finland | × | - | × | × | ✓ | ✓ | ✓ | × | × |
| France | × | - | × | × | ✓ | ✓ | ✓ | ✓ | × |
| Germany | ✓ | × | × | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ |
| Greece | × | - | × | × | ✓ | × | × | × | × |
| Hungary | × | - | × | × | ✓ | × | × | × | × |
| Iceland | × | - | × | × | ✓ | - | - | - | × |
| Ireland | × | - | × | × | ✓ | × | × | In process | × |
| Italy | × | - | ✓ | ✓ | ✓ | ✓ | ✓ | In process | × |
| Latvia | × | - | × | × | ✓ | - | - | - | × |
| Lithuania | × | - | × | × | ✓ | - | - | - | × |
| Luxembourg | × | - | × | × | ✓ | - | - | - | × |
| Malta | × | - | × | × | ✓ | - | - | - | × |

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*Discontinued commercialization

Reference: Respective countries reimbursement agency drug registry

# Gene therapies reimbursed in the European market

## Cell and Gene Therapies available in European countries (2/2)

| Country | Glybera | Imylgic | Strimvelis | Zalmoxis | Spinraza[24] | Kymriah | Yescarta | Luxturna | Zynteglo |
|---|---|---|---|---|---|---|---|---|---|
| Netherlands | × | - | × | × | ✓ | ✓ | ✓ | ✓ | × |
| Northern Ireland | × | - | × | × | ✓ | - | - | - | × |
| North Macedonia | × | - | × | × | × | - | - | - | × |
| Norway | × | - | × | × | ✓ | ✓ | ✓ | - | × |
| Poland | × | - | × | × | ✓ | - | - | × | × |
| Portugal | × | - | × | × | ✓ | - | ✓ | - | × |
| Romania | × | - | × | × | ✓ | - | - | - | × |
| Russia | × | - | × | × | × | - | - | - | × |
| Scotland | × | - | × | × | ✓ | ✓ | × | In process | × |
| Serbia | × | - | × | × | ✓ | - | - | - | × |
| Slovakia | × | - | × | × | ✓ | - | - | - | × |
| Slovenia | × | - | × | × | ✓ | - | - | - | × |
| Spain | × | × | × | ✓ | ✓ | ✓ | ✓ | × | × |
| Sweden | × | - | × | × | ✓ | ✓ | × | ✓ | × |
| Switzerland | × | - | × | × | ✓ | ✓ | ✓ | ✓ | × |
| Ukraine | × | - | × | × | × | - | - | - | × |

*Discontinued commercialization

Reference: Respective countries reimbursement agency drug registry

# Reimbursement pathways and timelines for gene therapy

- **Early Access Programs (EAPs)** – allow commercialisation of therapies before marketing authorisation for specific patients and conditions with no approved treatment options

- **Health technology assessment (HTA)** or submission for application of reimbursement to the respective commissioning bodies

As Cell and Gene Therapies are often developed for life-threatening conditions or rare diseases with no alternative treatments, they are often subject to apply for EAPs

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# Early Access Programs allow patients to access gene therapies within 6 months of submission

Early Access Programs (EAPs) in Europe provide pre-launch access for drugs in advance of their Marketing Authorization (MA) for patients with life-threatening conditions and no approved treatment options

- EAPs are country-specific, and products entering these programs are generally not reimbursed, except in France where dedicated financing is offered, or within named-patient schemes
- There are two types of EAPs in Europe, distinguishing access for a cohort of patients or for individual patients
  - **Compassionate Use Programs** are initiated by pharmaceutical companies for a group of patients in a selected clinic or hospital, and are not reimbursed by the public payer
  - **Named-Patient Programs** are granted in response to requests by physicians on behalf of specific or "named" patients and are reimbursed

| Country | Scheme | Setup timeline |
|---|---|---|
| Germany | Named patient | 3-6 months |
| UK | EAMS | 6 months |
| France | ATU | 6 months |
| Italy | Named patient | 3-6 months |
| Spain | Named patient | 3-6 months |

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Reference: European Medicines Agency, Committee for Medical Products for Human Use, Guideline on the Compassionate Use of Medicinal Products, Pursuant to Article 83 of Regulation (EC) No 726/2004

# Early Access to Medicines Scheme in the UK

| | | |
|---|---|---|
| **Type** | Individual | Groups / cohorts of patients |
| **Responsible Agencies** | Medicines and Healthcare products Regulatory Agency (MHRA) is responsible for the benefit/risk scientific opinion; NICE | |
| **Initiator** | Prescribing Physician | Manufacturer / license holder |
| **Duration** | Ends with product license | 12-18 months with 3 monthly review |
| **Pricing and Reimbursement** | Product supplied free of charge | |
| **Restrictions and Requirements** | • The product is likely to offer benefit significant advantage over and above existing options<br>• Potential adverse events are likely to be outweighed by benefits | |
| | • Although the company will record what they supply, there is often no central registrar for the patients that are being treated | • The applicant is able to supply the product in each region in the UK |

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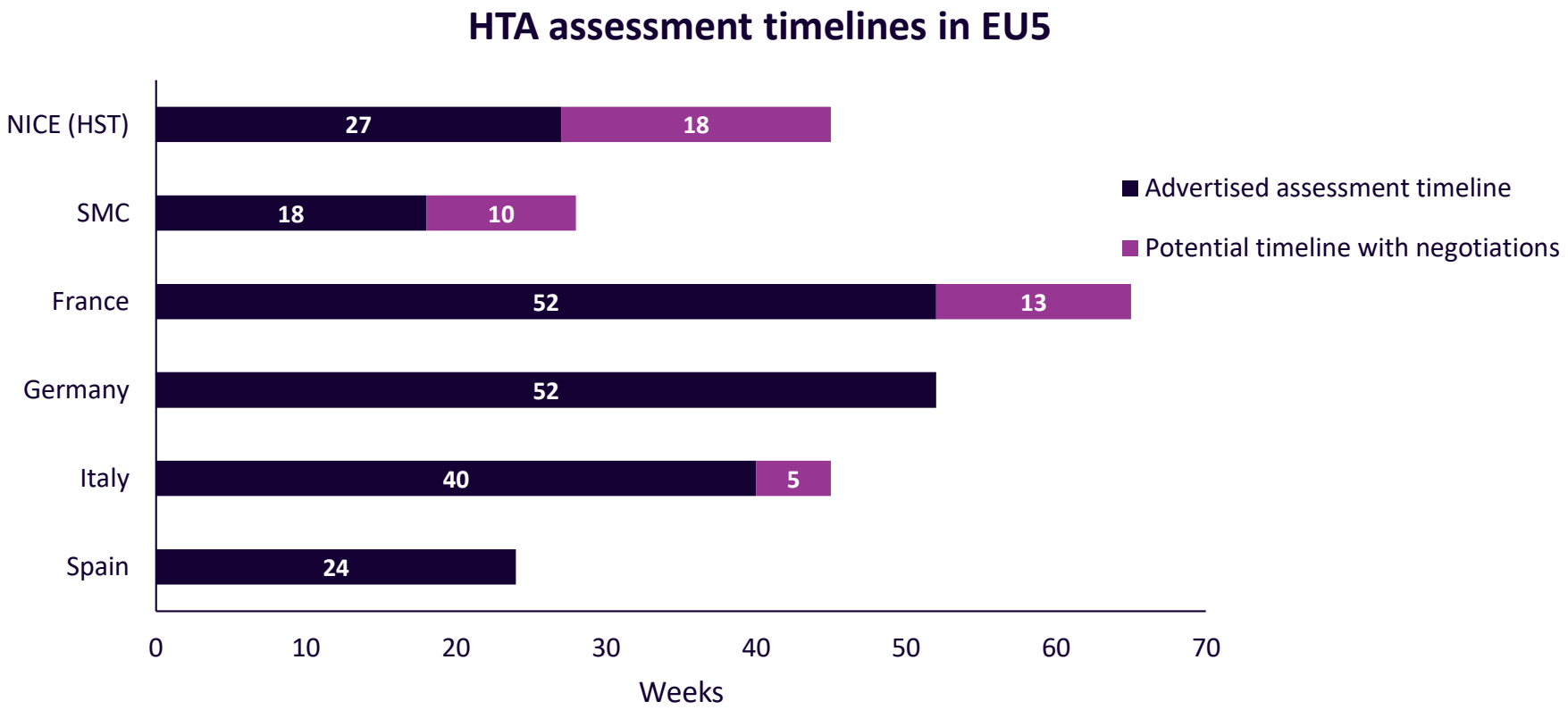
Reference: Gov.uk, Office for Life Sciences, Early Access to Medicines Scheme Guidance

# Minimal time to reimbursement for gene therapies in EU5

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Reference: Walzer et al 2019.

Negotiation estimates based upon experience in market – lack of published data. Germany negotiation timelines are included in the advertised assessment timeline, Spanish negotiation timelines are very variable (depending on political cycles and regional vs centralised decision making)

# Actions to secure market access for gene therapies: planning for reimbursement

## Shaping Early Development

- Early Health Economic (HE) analysis:
  - Identification of value drivers (clinical & HE)
  - Room for innovation
  - Prioritisation of the indication & therapeutic position
- Identify benefit and cost thresholds
- Target Product Profile (TPP) definition, mapping of evidence generation to substantiate
- Establish “go/no go” criteria for the “Stage-Gate” process

## Early P&R strategy development

- Engagement with key market access stakeholders to explore:
  - Key value drivers
  - Likely positioning, pricing & reimbursement
  - Supporting data requirements

## Reimbursement Optimization

- Identify price corridor:
  - Revenue maximising price per market
  - International price referencing
  - Launch sequence
- Contingency planning and risk-sharing schemes
- Planning for post-launch evidence generation

## Value Story Development

- Develop Value Story
  - Test reliability and impact of messages
- Address evidence gap between clinical trial data and value proposition
  - Model data
- Finalise HE models
- Develop Value Dossier

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Reference: Catapult 2019

# Innovative reimbursement contracting schemes

Value-based risk-share agreements are an **innovative payment model** that brings together two key stakeholders—**health care payers** and **biopharmaceutical manufacturers**—to deliver therapies to patients:

## Financial-Based Agreements

Price level or nature of reimbursement is based on financial considerations, not related to clinical performance

- Price-volume agreements
- Total cost cap
- Non-price discounts/ free goods

## Outcomes or Performance-Based Agreements

Price or reimbursement is tied to future metrics ultimately related to patient performance, outcomes, efficacy, tolerability, dosing, benefit, outcomes, quality of life, or clinical usage

- Outcomes guarantee
  - Duration of treatment
  - Need for reintervention
  - Achievement of clinical milestones
- Compliance monitoring
- Pattern or process of care

## Coverage with Evidence Development (CED)

Reimbursement decision in which approval is conditional on the collection of additional population level studies after launch (with provisional reimbursement) to support coverage or pricing

Mostly used by insurance companies in managed entry agreements

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# Performance-Based Agreements (PBA) are the preferred contracting model for gene therapies in Europe

Most recently authorised gene therapies use different types of PBA for reimbursement:

- **Kymriah and Yescarta:** relatively uniform list prices across the EU5, reimbursed according to their MA
  - France and UK: reimbursement on the condition of collecting additional data (at the cohort level) and subject to future reassessments
  - Germany: price rebates
  - Italy and Spain: staged payments linked to individual patient outcomes (RWD)
- **Zolgensma:** "Day One" access program, that offers customizable options including:
  - Retroactive rebates and outcomes-based rebates ensuring early access costs are aligned with negotiated prices following assessment processes
  - Deferred payments and instalment options, allowing reimbursement bodies to manage budget impact during the early access phase
- **Zynteglo**: outcomes-based pricing throughout Europe:
  - Yearly payments of 20% of the list price linked to outcomes
  - Therefore putting 80% of the base price at risk

PBA target 2 key challenges for gene therapy reimbursement:

- High upfront financial risk
- Absence of long-term outcomes data

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# Panel Discussion – Industry reflections on the varying pace of change

*Craig Bradley*


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# Panel Discussion

**Panel Chair:**
**Craig Bradley**

Head of Marketing - Diabetes &
Internal Medicine
Takeda UK

**Jerome Penn**

Senior Public Affairs Manager
Takeda UK

**Andrew Mumford**

Principal Consultant
Initiate Consultancy

**Bethany English**

Market Access Analyst

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PM SOCIETY
Market Access

Multiple-choice poll

Survey (1/9)

**Are you (or have you) worked on market access for orphan (or Ultra orphan) treatments?**


slido

Multiple-choice poll

Survey (2/9)

**Does your company have rare disease treatments in their pipeline?**

Multiple-choice poll

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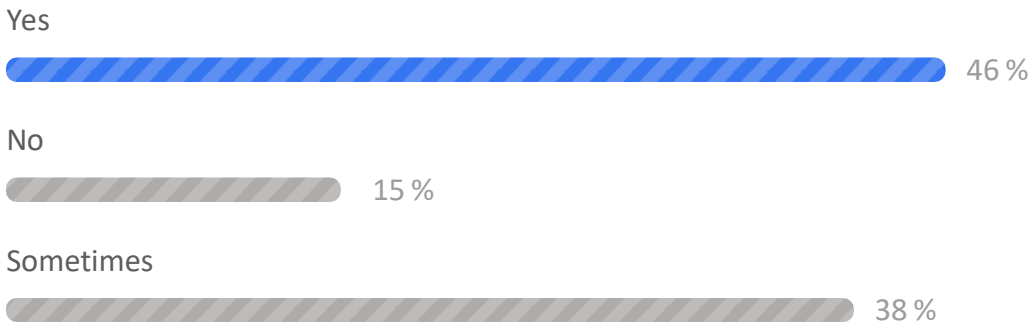
Survey (3/9)

0 1 3

**Do you feel that the UK lags behind other markets in Europe in granting access to innovative treatments in rare diseases?**



Multiple-choice poll

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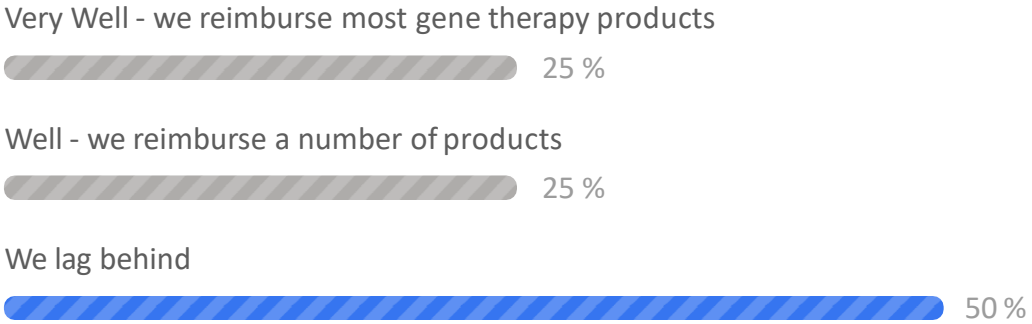
Survey (4/9)

0 1 2

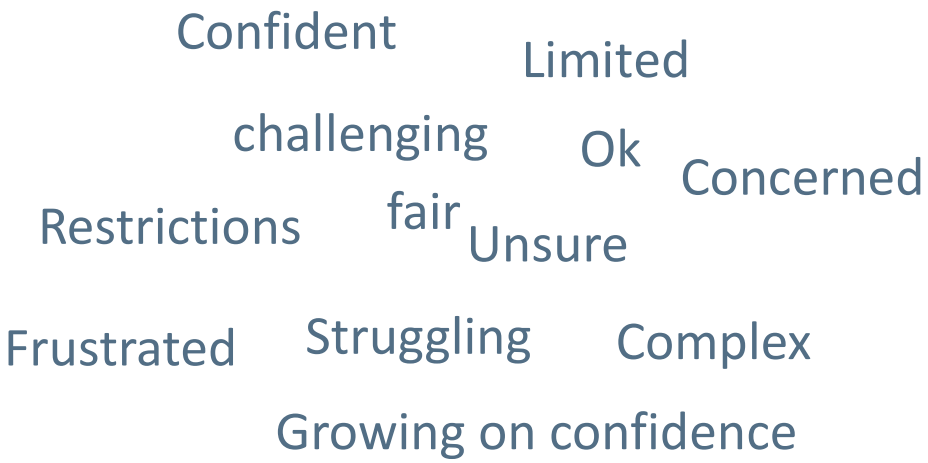
**How do you feel the UK performs when compared to other EU5 markets in reimbursing gene therapy?**



Wordcloud poll

Survey (5/9)

**How do you feel about the ability of the NHS to fund innovative treatments in rare diseases?**

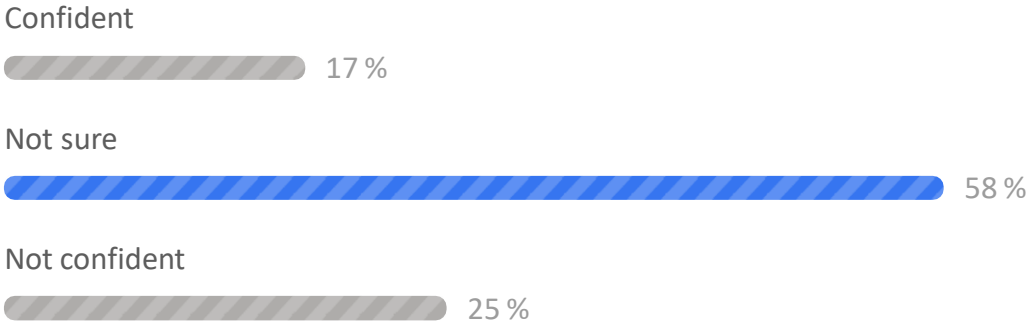



slido

Multiple-choice poll

Survey (6/9)

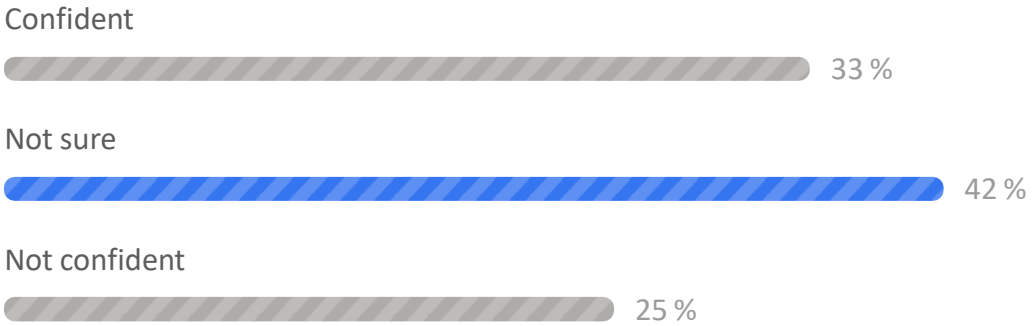
**How confident are you in the NICE HST process to deliver access for rare diseases?**



Multiple-choice poll

Survey (7/9)

**How confident are you in the SMC Ultra Orphan pathway to allow access in rare diseases?**



Wordcloud poll

Survey (8/9)

**What do you feel are the biggest influences in the HTA process in rare diseases?**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Survey (9/9)

**What changes would you like to see in rare disease access?**

No managed access agreements and acceptance of OBS or annuity schemes

hta

Accelerate process

affordability

clarity

bespoke

More bespoke HTA process

Increased options / routes to access

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Andrew Mumford
Principal

M: +44 7932691250
E: a.mumford@initiateconsultancy.com
www.initiateconsultancy.com

Initiate Consultancy Ltd.
The Potting Shed
Pury Hill Business Park
Northants NN12 7LS
UK

Initiate Consultancy Ltd.
Holden House
57 Rathbone Place
London W1T1JU
UK


Synaptiq health

NEXT WEBINAR: Wednesday 5th August:

Responding to patient need: managing in a time of crisis

16:00 **Introduction**
Jon Hoggard, Patient Engagement Director, Nucleus Global

16:10 **Patient insights on healthcare during COVID-19**
Claire Murray, Director, Aurora Communications

16:40 **How pharmacy services are adapting to the new normal**
Graham Thoms, CEO, Pharmadoctor

17:10 **How CCGs are adapting**
Pam Green, COO, NHS North East Essex CCG

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# Papyrus

https://www.justgiving.com/fundraising/pm-society

- The PM Society is proud to be supporting PAPYRUS this year as it's chosen charity
- A donation of just **£5** pays to service one call, text or email to HOPELINEUK, which can help a young person stay safe from suicide. Just one call really could save a life.

PAPYRUS is the leading national charity dedicated to the prevention of young suicide. Founded in 1997 by a group of parents who had all tragically lost a child to suicide, PAPYRUS exists to reduce the number of young people who take their own lives by shattering the stigma around suicide. They support and equip young people and their communities with the skills to recognise and respond appropriately to suicidal behaviour.

PAPYRUS provides confidential support and advice to young people struggling with thoughts of suicide, as well as anyone worried about a young person through their helpline – HOPELINEUK and engages with communities and volunteers in suicide prevention projects, delivering training and awareness-raising programmes to individuals and community groups. They also aim to shatter the stigma that remains around suicide and shape national social policy making significant contributions to local and regional implementation of suicide prevention strategies.