



# Cell and Gene Therapy Catapult

## UK ATMP Clinical Trials Report 2022

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## 1. Executive Summary

The number of advanced therapy medicinal product<sup>1</sup> (ATMP) clinical trials in the UK continues to increase year-on-year, with 178 trials reported as ongoing in 2022 up from 168 last year. While a modest increase compared to previous year's growth, the steady state in the UK contrasts with the global 13% decline in ongoing ATMP clinical trials observed worldwide. This includes a 15% reduction in both North America and Asia Pacific, and similarly a 14% decrease in ongoing ATMP clinical trial activity in Europe<sup>2</sup>. Commercially sponsored trials accounted for about 80% of all UK ATMP clinical trials (increasing from 131 trials in 2021 to 145 in 2022), demonstrating the attractiveness the UK is to commercial sponsors.

The UK's influence on the global stage also continues to grow and is now represented in 14% of global ongoing ATMP commercial trials in all Phases I - III<sup>2</sup>, and 8% of all trials in total. Oncology remains the dominant therapeutic area under investigation in ATMP clinical trials, followed by haematological indications, metabolic disorders, and ophthalmology. The majority of ATMP trials are investigating gene therapies (77%), with an even distribution between *in vivo* and *ex vivo* gene therapy trials.

Phase II/III trials have increased, from three trials in 2021 to seven trials in 2022, demonstrating the ongoing successful clinical progression of therapies.

It is important to note that throughout 2021 and into early 2022, COVID-19 greatly impacted the routine operations of society, business, and healthcare. Since then, the MHRA has not needed to be notified of interruptions to trials due to COVID-19, therefore the true impact of the pandemic on all stages of clinical trials is not yet completely understood. Fewer trials were reported as completed in 2022 compared to 2021, and this is likely due to the ongoing COVID-19 pandemic, which has resulted in trial delays.

1 As defined by EC ATMP regulation 1394/2007  
2 Alliance for Regenerative Medicine State of the Industry Briefing September 2022



# 1.1 Introduction to the 2022 ATMP clinical trials database

The UK 2022 ATMP clinical trials database covers advanced therapy medicinal product (ATMP) clinical trial activities that the Cell and Gene Therapy Catapult (CGT Catapult) understands have been ongoing in the UK between January and December 2022. It supersedes the 2021 database, and both are available at [ct.catapult.org.uk/resources/clinical-trials-database](https://ct.catapult.org.uk/resources/clinical-trials-database)

The 2022 database has been compiled and verified by the CGT Catapult team, and includes:

- all ATMP trials (see definition of trials included in Section 5.2 Methodology), including non-interventional long-term follow-up (LTFU) trials
- academic (which includes non-profit research) and commercial trials
- ongoing trials in the UK regardless of the geographic location of the sponsor's headquarters



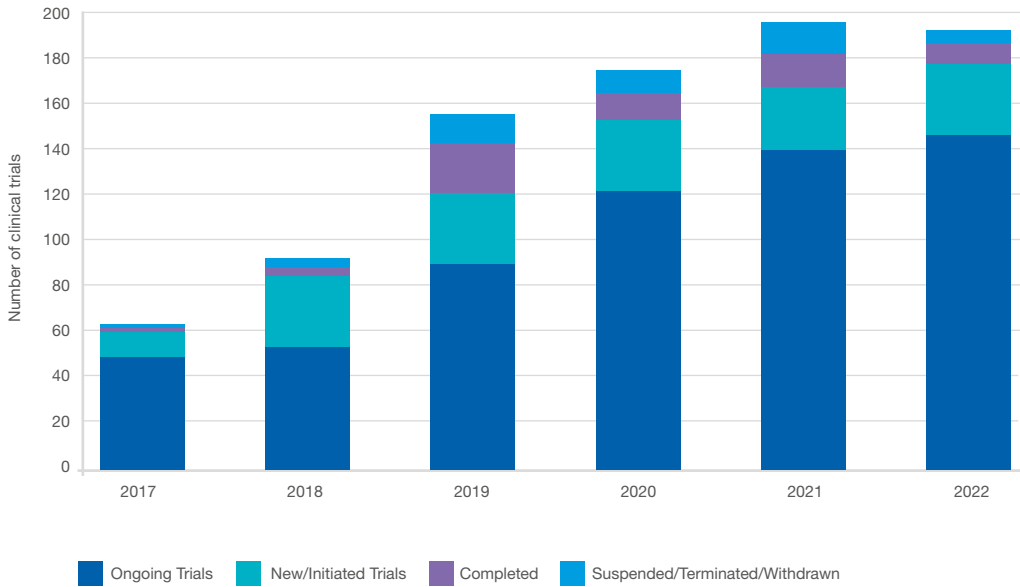
# 2. Commentary on key findings 2022



## 2.1 The UK's portfolio of ATMP clinical trials continues to grow

The 2022 UK ATMP clinical trials database continues to see a year-on-year increase of ongoing ATMP clinical trials, although the rate of growth has slowed from previous years. There are 178 trials ongoing in the UK in 2022, compared to the 168 trials reported in 2021, with the UK now represented in 8% of all global trials as described in the 2022 ARM report, and 14% of Phase I-III ongoing commercial global trials. As shown in Figure 1, there has been a slight increase in trials initiated during 2022 (31) compared to 2021 (29), with fewer suspended, terminated, or withdrawn. This is likely due to the persisting legacy of the COVID-19 pandemic, which previously resulted in delays to trial initiation.

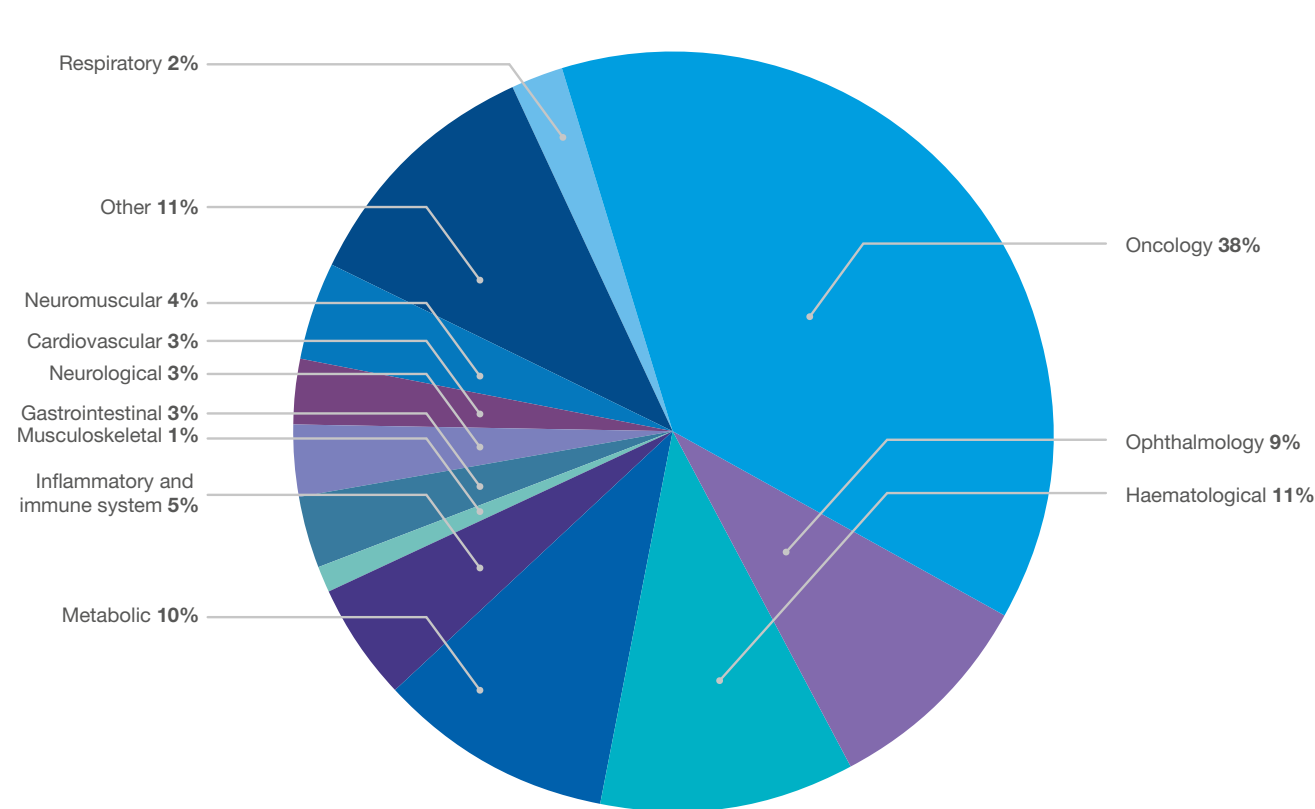
Figure 1. Number of ATMP clinical trials in the UK per year



## 2.2 Oncology remains the largest therapeutic area

The division of therapeutic indications among the ongoing ATMP trials remains largely unchanged from previous years. Oncology, which includes haematological malignancies and solid tumours, remains the dominant therapeutic area accounting for 38% of ATMP clinical trials, followed by haematological (11%), metabolic (10%), and ophthalmology (9%), Figure 2.

**Figure 2.** Distribution of UK ATMP clinical trials by therapeutic area



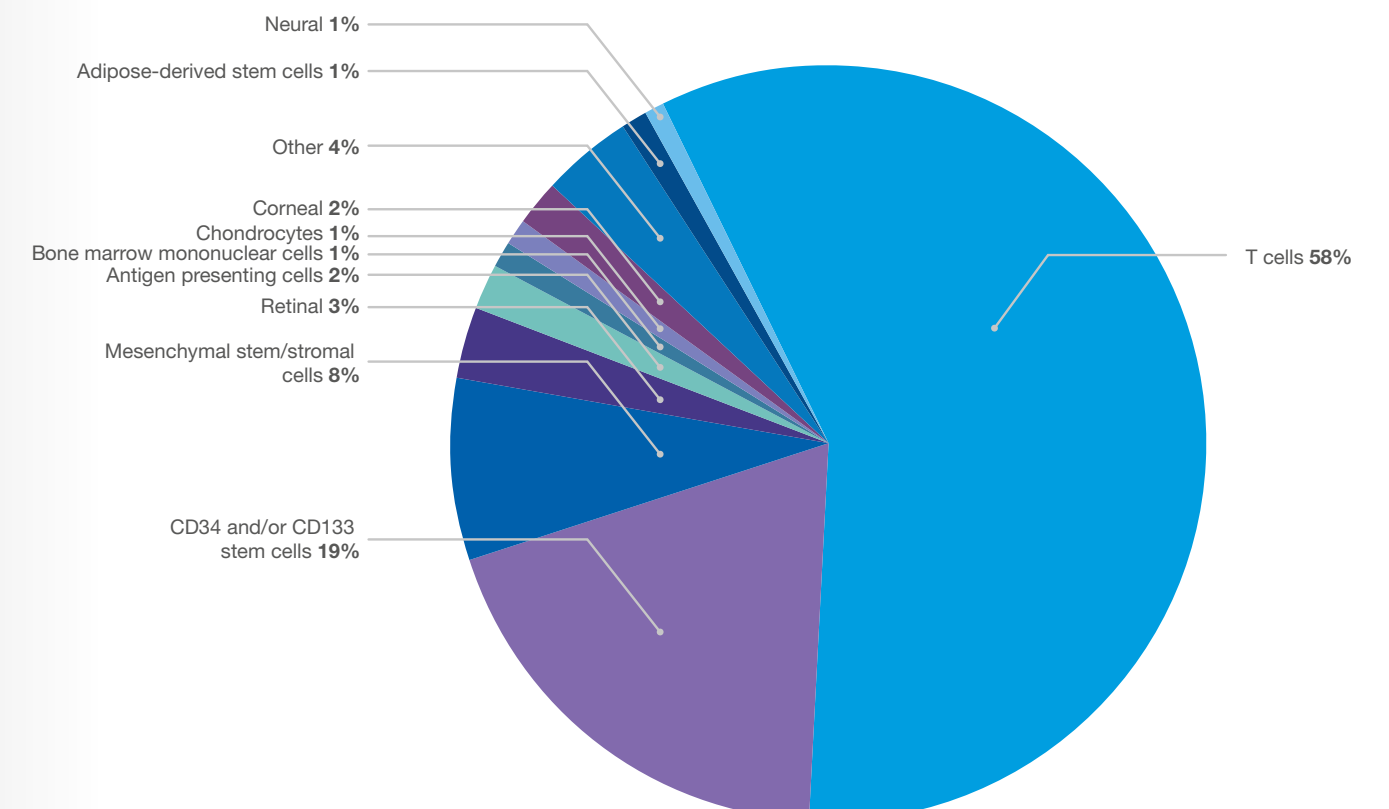
### 2.2.1 Breakdown of trials initiated by therapeutic area

In addition to being the largest therapeutic area investigated overall, oncology remained the largest therapeutic area of trials initiated in 2022, accounting for 29% of the clinical trials initiated this year. Genetic disorders were the second largest therapeutic area targeted by trials initiated in 2022 (19%), followed by trials targeting infectious diseases (13%) and then haematology (10%); data not shown.

## 2.3 T cells are the dominant cell type investigated

The cell types investigated in ATMP clinical trials remain largely unchanged from 2021; T cells continue to be the most commonly developed cell type, accounting for 58% of UK ATMP clinical trials, Figure 3. This is as expected since research into oncology, the largest therapeutic area, is largely T cell focused and this is consistent with data from previous years.

**Figure 3.** Breakdown of ongoing UK ATMP clinical trials by cell type





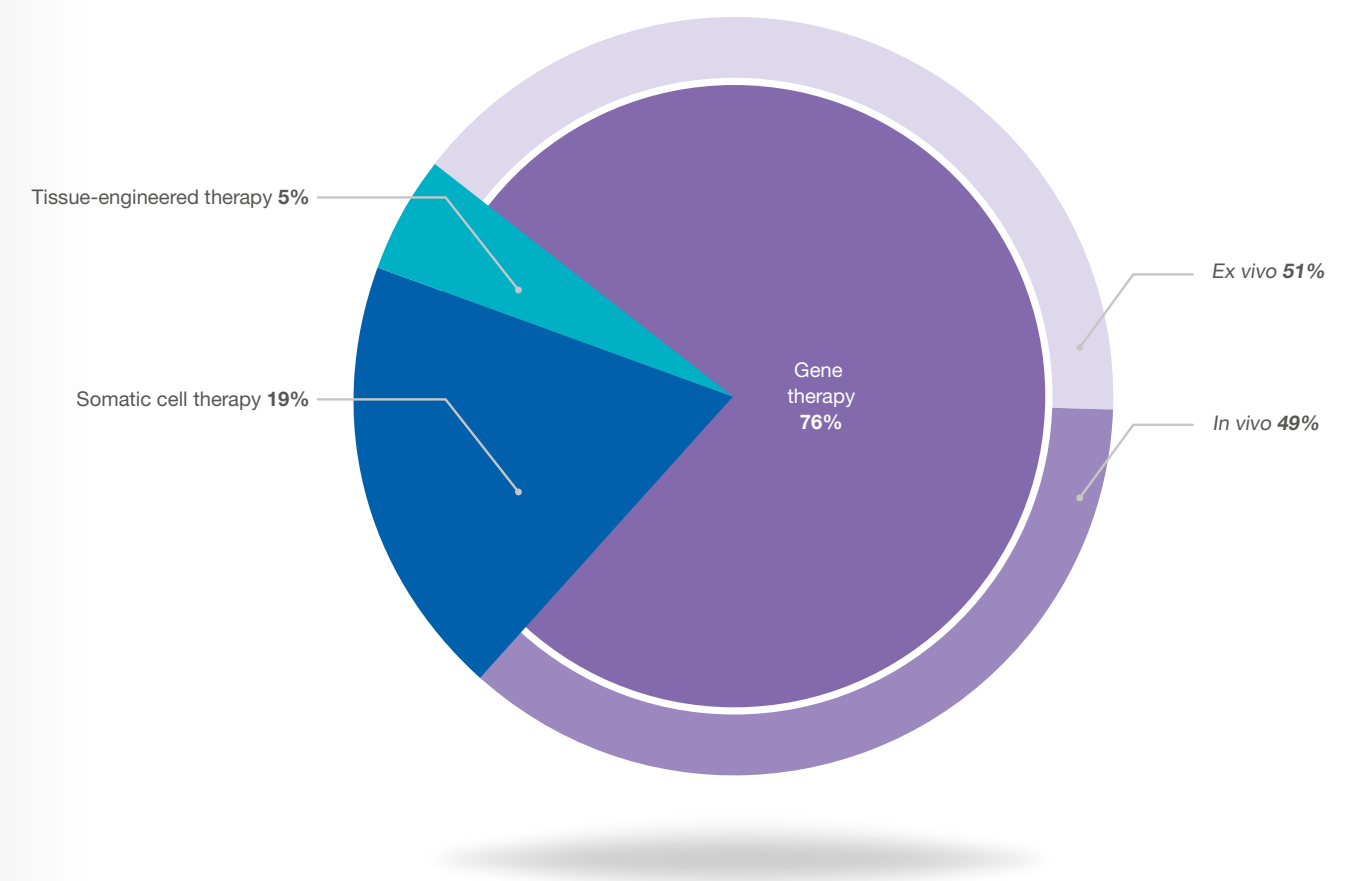


## 2.4 Majority of therapies in the database are genetically modified

The majority of the 178 ATMP clinical trials ongoing in the UK in 2022 were gene therapies (76%) followed by somatic-cell therapies (19%). Tissue-engineered therapies accounted for approximately 5% of the ongoing clinical trials, Figure 4.

Of the ongoing gene therapy clinical trials, similar numbers of *ex vivo* (51%), which includes CAR-T, and *in vivo* (49%) genetic modifications were investigated. These gene therapy trials are further discussed in the Gene delivery technologies Section 2.8.

**Figure 4.** Breakdown of UK ATMP clinical trials by ATMP type



# 2.5 Breakdown of UK ATMP clinical trials by Phase

Most of the UK ATMP clinical trials observed in 2022 are in Phase I/II (37%), Figure 5a. The distribution by phases, broken down into gene therapy, somatic cell, and tissue-engineering trials, is further shown in Figures 5b-d.

Figure 5a. ATMP trials by phase for 2022

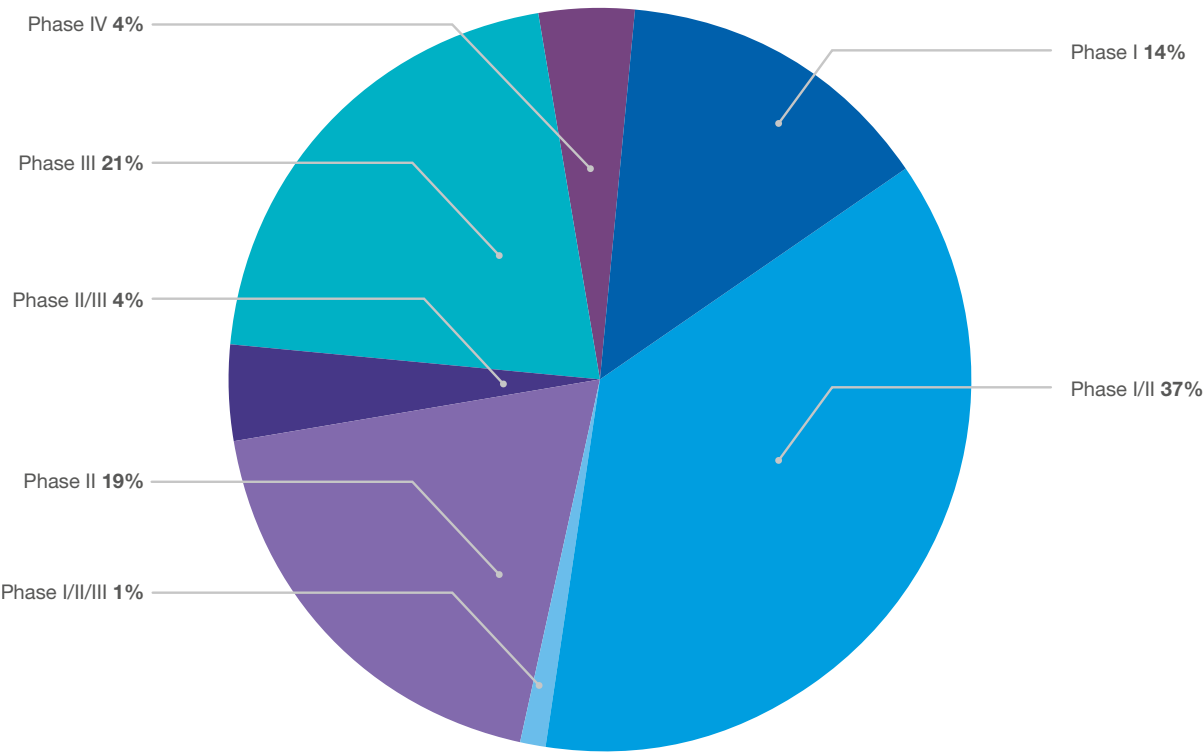


Figure 5b. Gene therapy

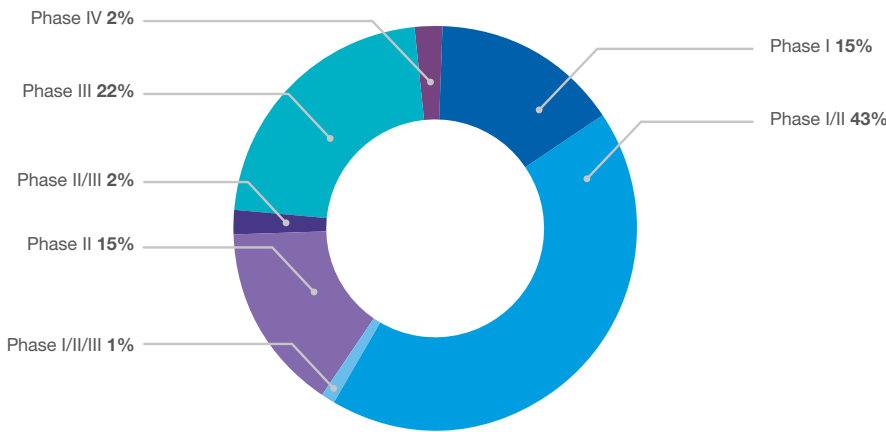


Figure 5c. Somatic-cell therapy

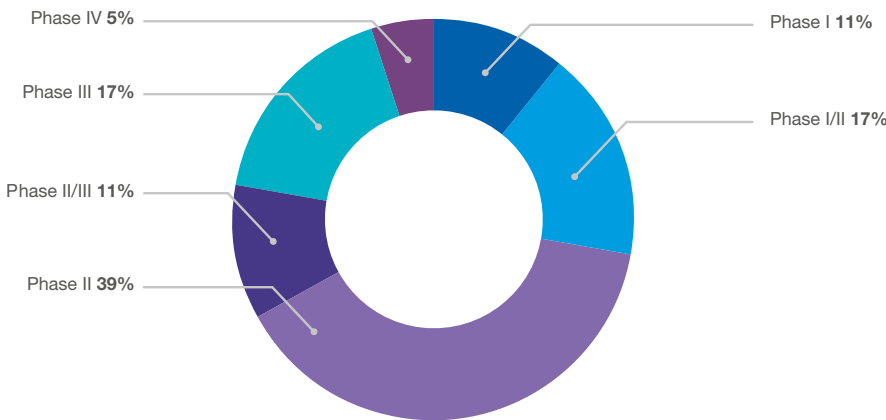
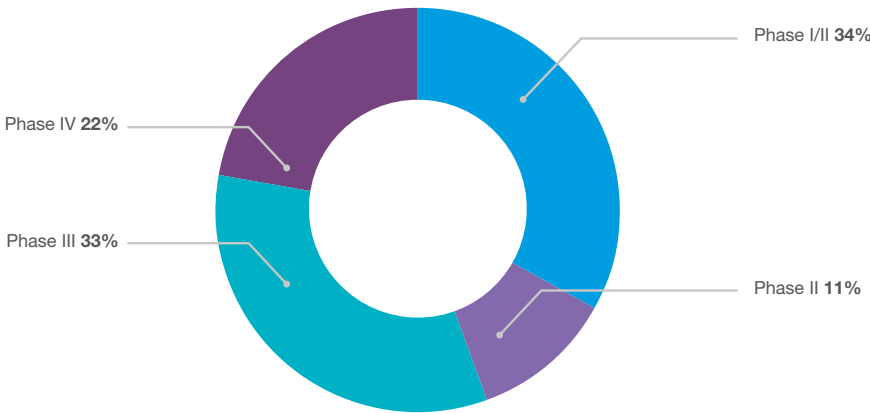


Figure 5d. Tissue-engineered product



## 2.6 Breakdown of 2021 UK ATMP trials by status

Overall, most of the UK ATMP clinical trials that were active in 2022 are currently recruiting (51%) or in follow-up (34%), Figure 6a. A breakdown of each ATMP type showed a similar trial status distribution (Figures 6b-d). For tissue-engineered products, trials recruiting account for 33% of all trials, whilst trials in follow-up account for 22%, Figure 6d.

Figure 6a. Distribution of UK ATMP clinical trials

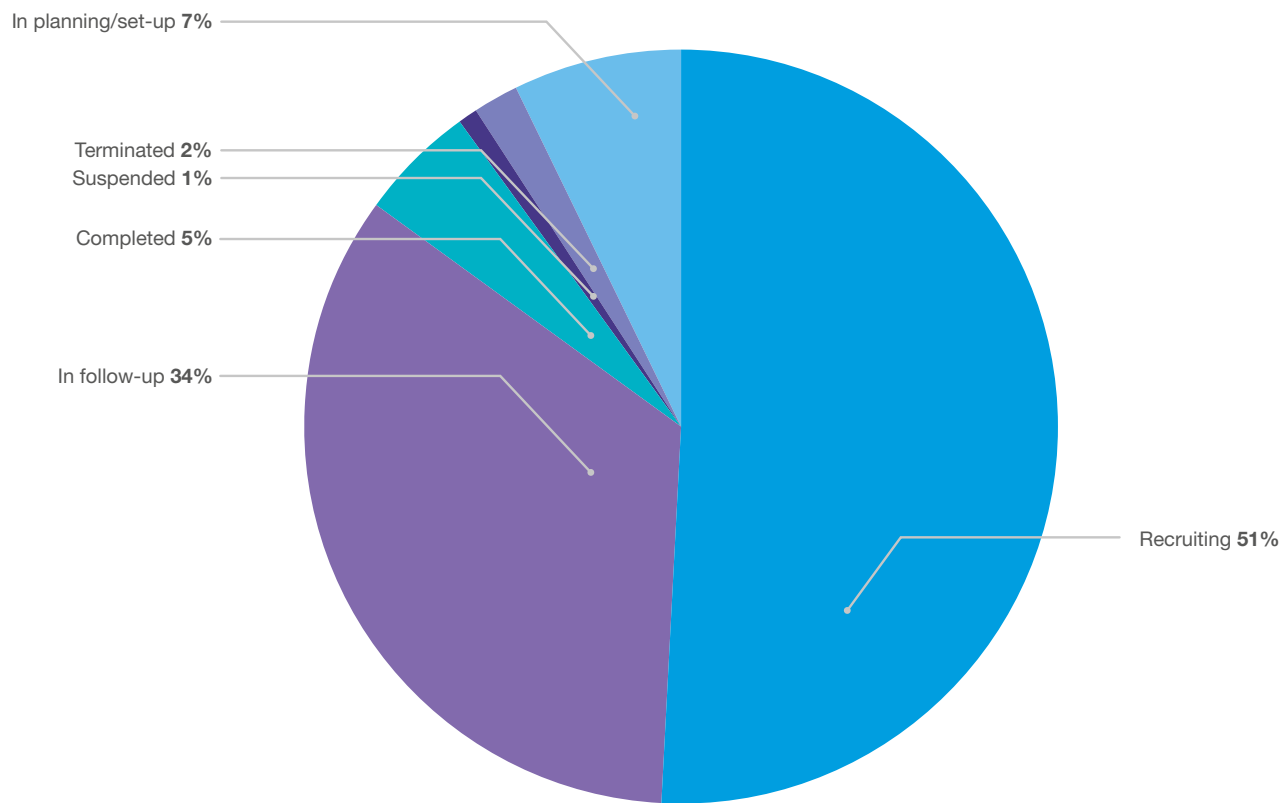


Figure 6b. Gene therapy

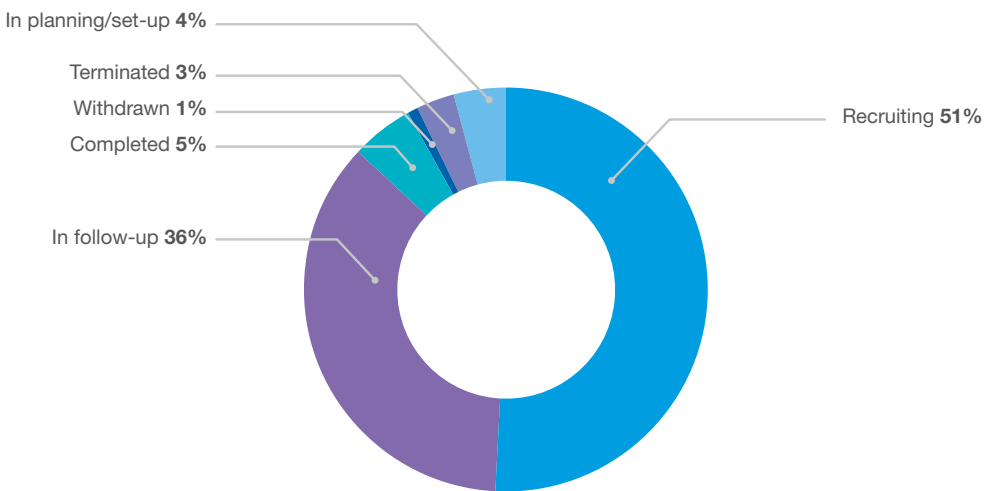


Figure 6c. Somatic-cell therapy

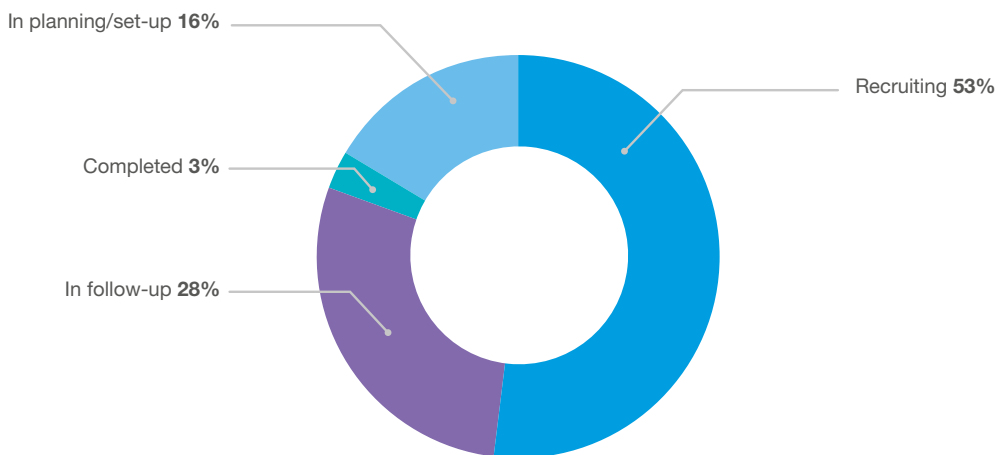
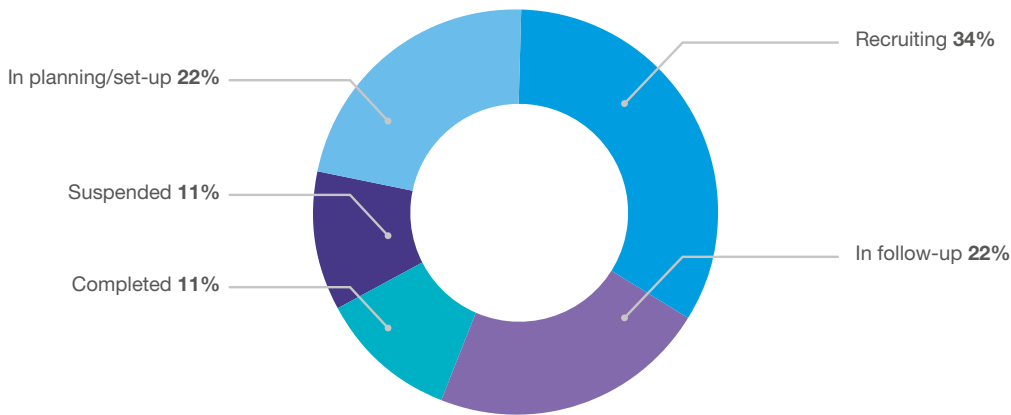


Figure 6d. Tissue-engineered product

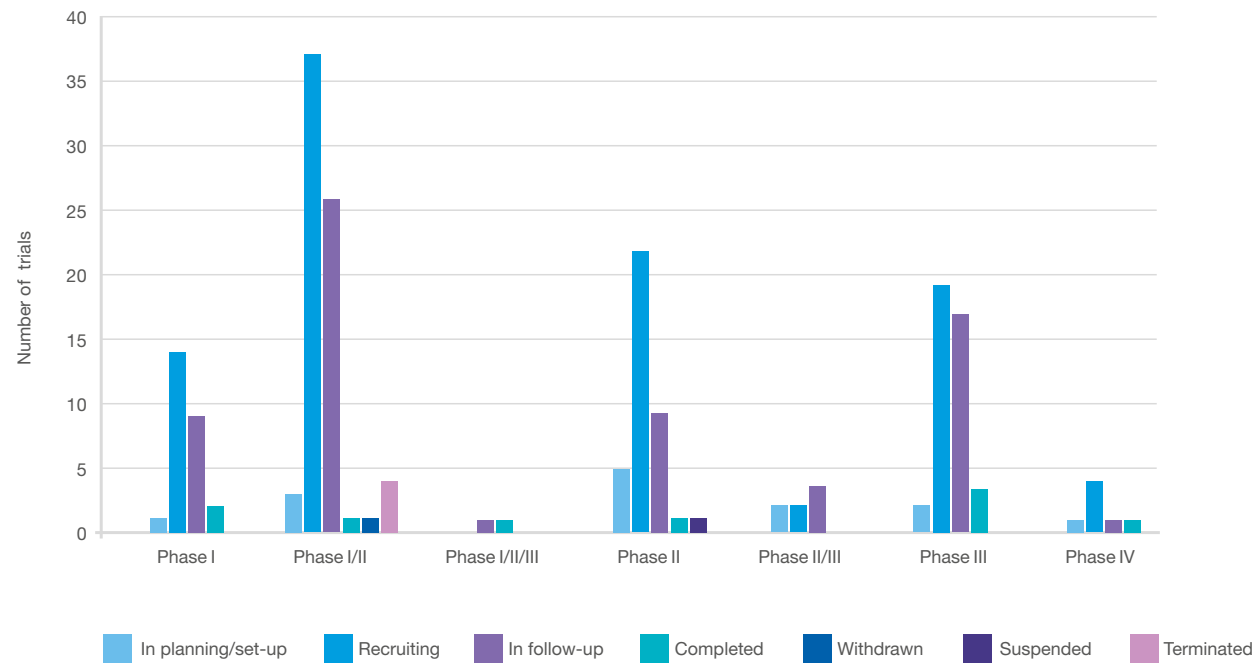




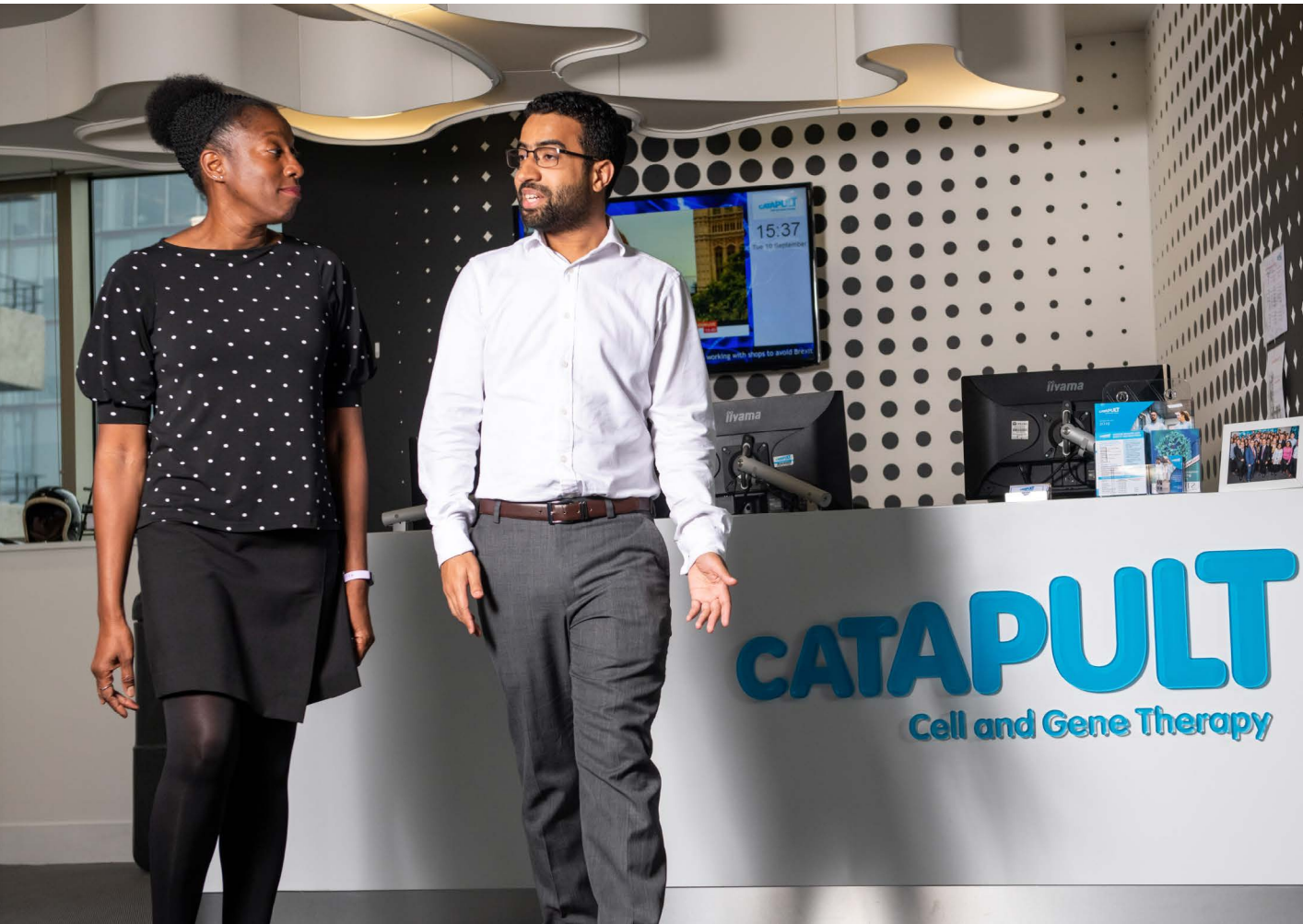
# 2.7 Distribution of UK ATMP trials by Phase and status

Of the total UK ATMP clinical trials currently recruiting, the majority (37) are Phase I/II, followed by Phase II (21), and Phase III (19), Figure 7. We observed four Phase IV clinical trials in recruitment.

Figure 7. Number of ATMP clinical trials in the UK in 2022



Most of the trials initiated in 2022 are currently recruiting (56%), followed by in planning/set-up (34%). The majority of these newly initiated trials are Phase III and Phase I/II, with eight and eleven trials respectively (data not shown).

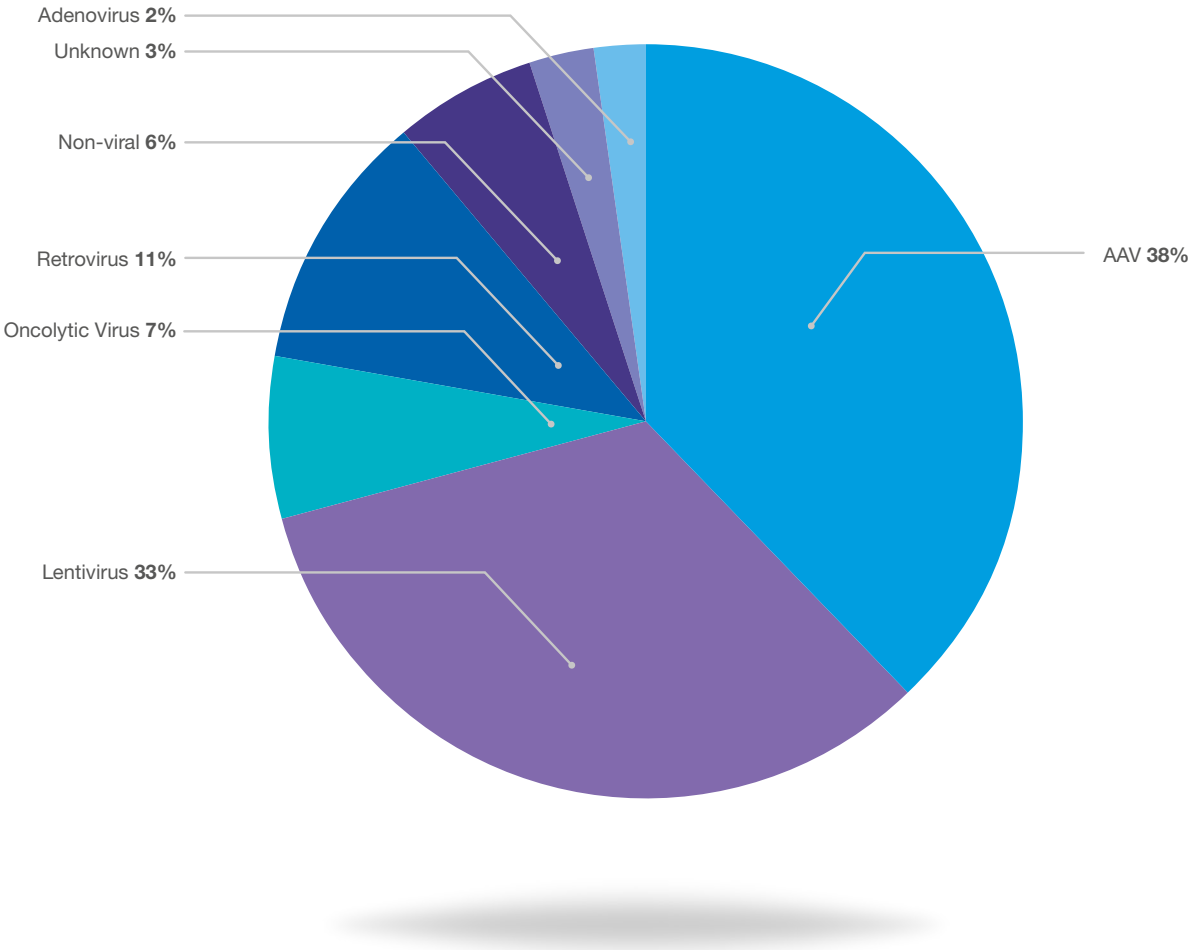




## 2.8 Gene delivery technologies

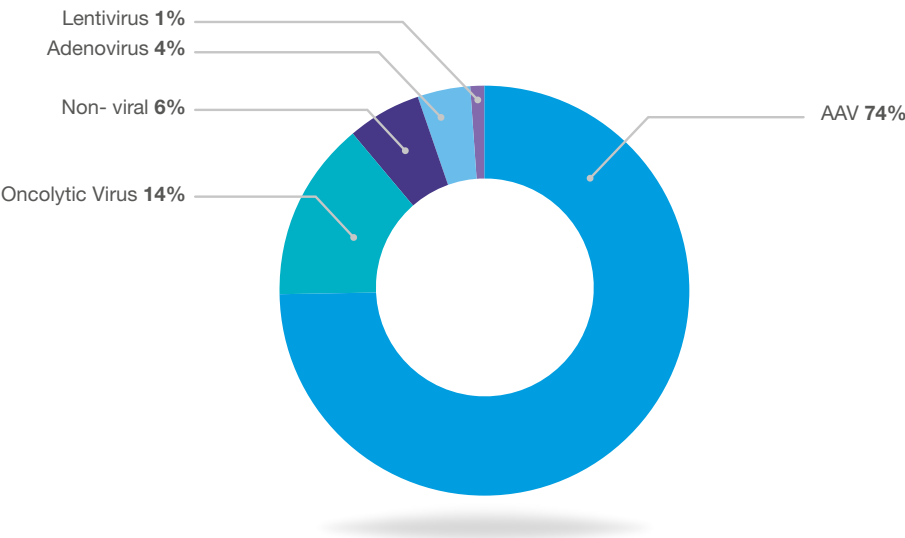
AAV and lentiviral vectors remain the most commonly used gene delivery vectors, Figure 8A. For *in vivo* gene therapy clinical trials, AAV based vectors remain the main vector of choice (74%), followed by oncolytic viruses (14%). A small proportion of trials are investigating therapies using non-viral vectors (6%), adenovirus (4%), or lentivirus (1%), Figure 8b. For *ex vivo* gene therapy clinical trials, the use of lentiviral vector delivery dominates (66%), followed by retroviral vectors (21%), Figure 8c. Non-viral *ex vivo* based gene therapies, accounted for 7% of ATMP trials observed.

**Figure 8a.** Distribution of gene transfer vectors used in 2022 ATMP clinical trials

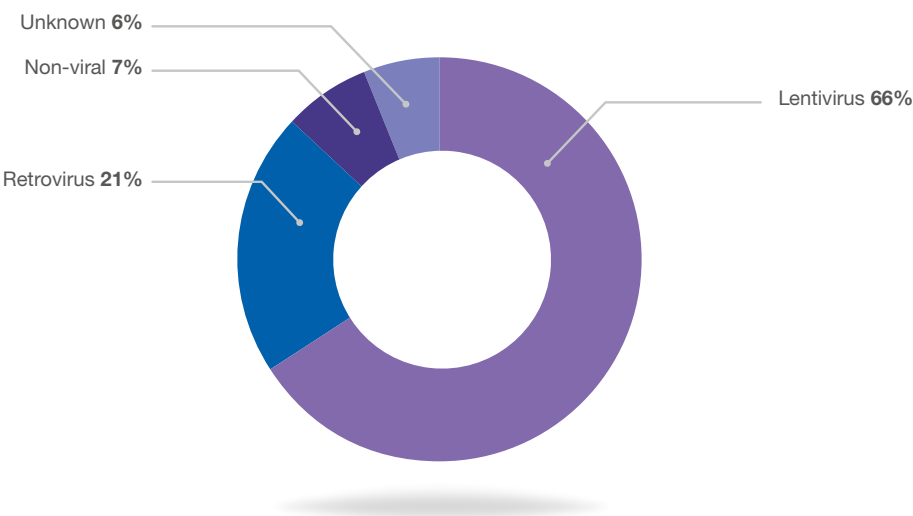


*Ex vivo* vectors are predominantly used for CAR-T based therapies (CAR-T and CAR-T + gene editing) which account for 43% of *ex vivo* clinical trials. This is followed by "Gene corrections/insertion" therapies which account for 33%, Figure 8D. The *ex vivo* CAR-T and TCR based clinical trials almost exclusively focus on oncology, whilst therapies involving gene corrections target multiple disease types (not shown).

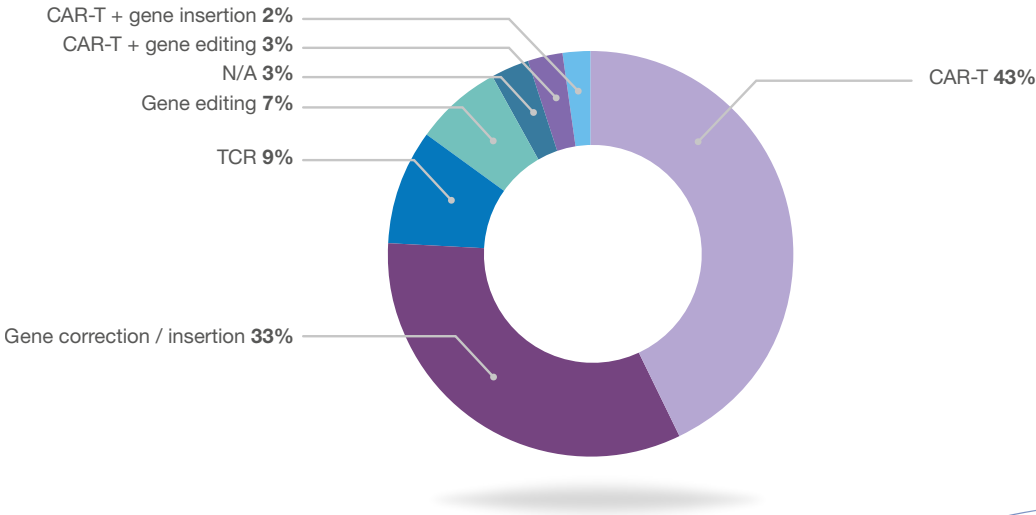
**Figure 8b.** Distribution of *in vivo* gene transfer vectors used in 2022 ATMP clinical trials



**Figure 8c.** Distribution of *ex vivo* gene transfer vectors used in 2022 ATMP clinical trials



**Figure 8d.** Distribution of the type of *ex vivo* gene therapy in ongoing clinical studies



# 2.9 Long-term follow-up (LTFU) trials

The LTFU trials discussed in this section are distinct trials classified as LTFU, however there also continues to be earlier phase clinical trials which include LTFU as part of their study design, which are not discussed here.

Of the 30 LTFU studies identified, 19 are currently recruiting, 6 are in follow-up, 3 are in planning/set-up, and 2 were completed in 2022 (data not shown). Oncology (32%) is the largest therapeutic area for which long-term follow-up trials are ongoing, followed by ophthalmology (21%) and haematology (18%), Figure 9a. Together, these account for over 70% of LTFU clinical trials. The majority of these LTFU trials are either Phase II or Phase III trials, Figure 9b, and 64% of the LTFU clinical trials are interventional, Figure 9c.

Figure 9a. Distribution of long-term follow-up studies by therapeutic area

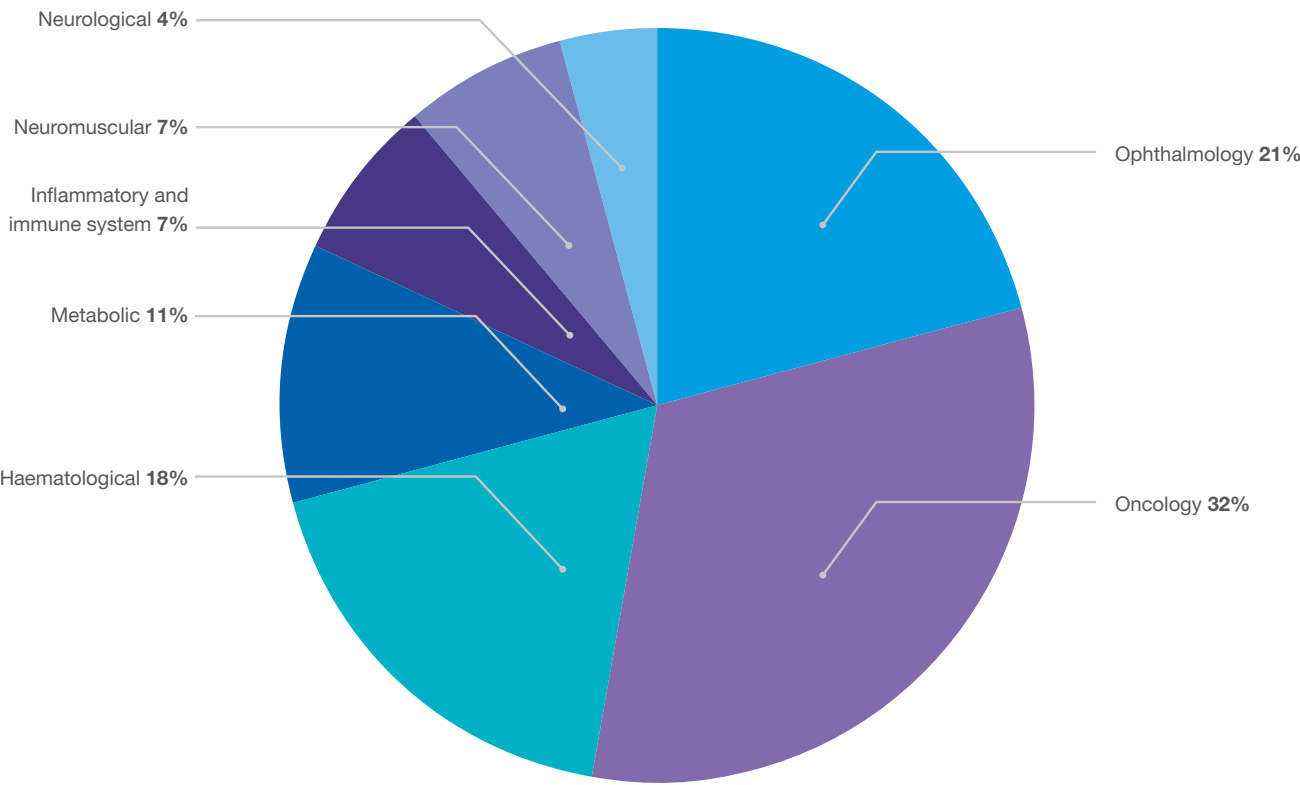


Figure 9b. Distribution of long-term follow-up studies by Phase

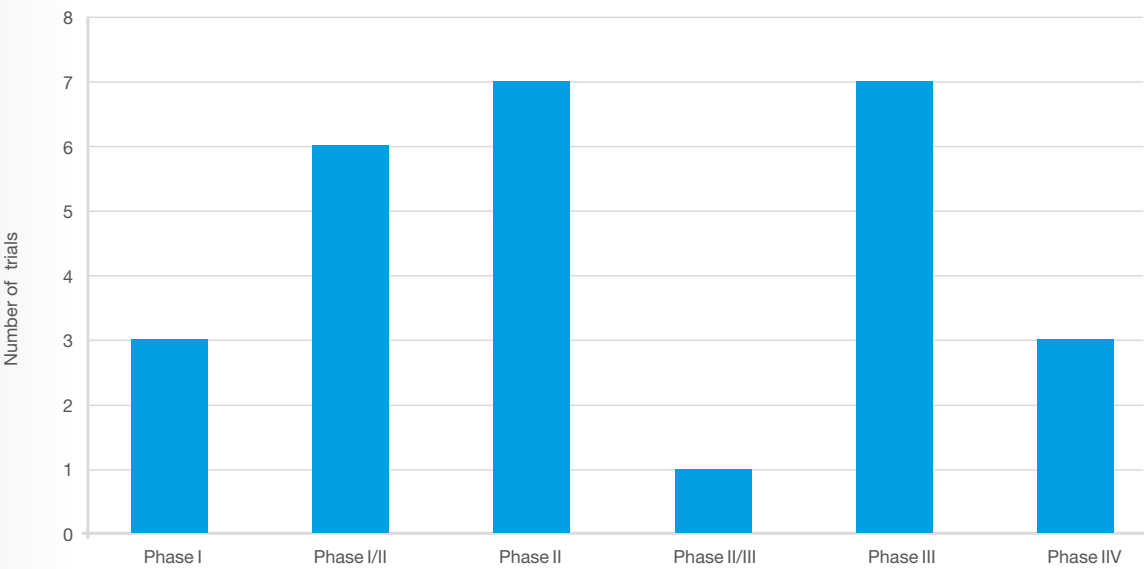
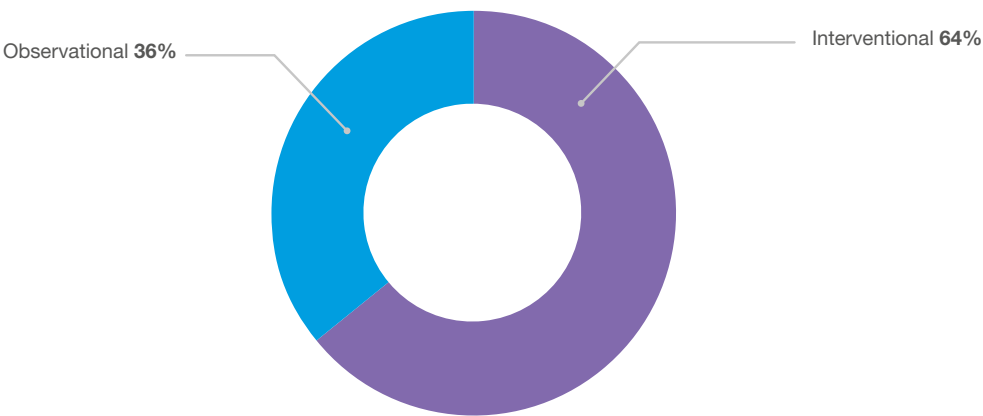


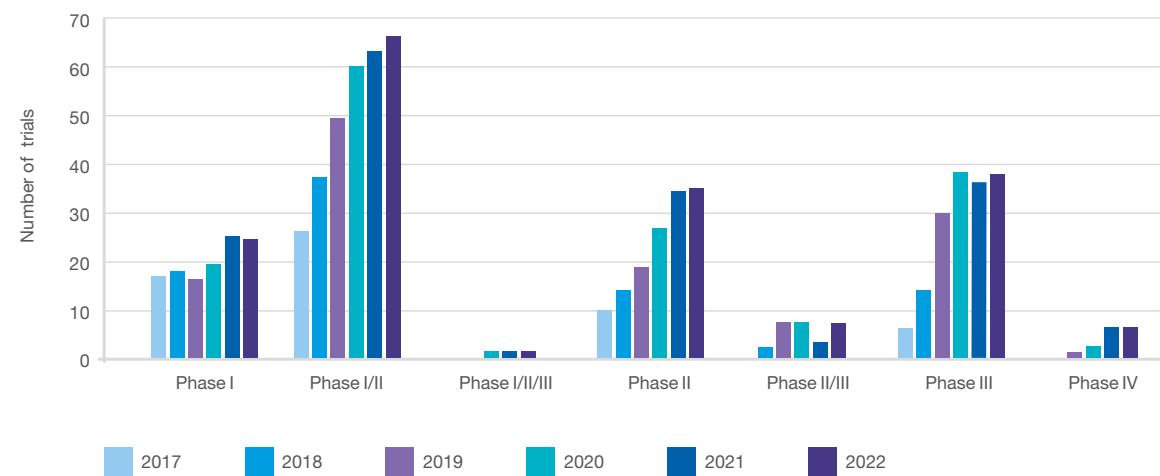
Figure 9c. Distribution of long-term follow-up studies



## 2.10 Growth continues in early phase trials

There has been an increase from 2021 observed in the number of clinical trials across all Phases, with the exception of Phase I clinical trials which showed a minor decrease, Figure 10. The growth seen in 2022 of early phase trials is indicative of the ATMP space growing and successful translation of therapies from non-clinical programs into First-in-Human clinical trials. Additionally, many new umbrella trials have started, which group together long-term safety follow-ups on various trials where subjects have been exposed to the same therapy. In the coming years, it is likely that the greatest impact on trial status will be these trials having longer follow-up periods.

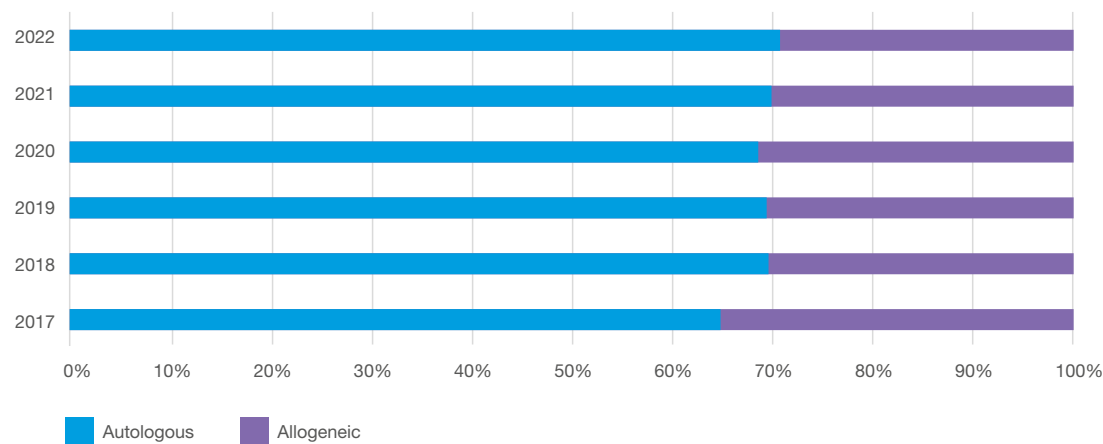
Figure 10. Ongoing ATMP trials in the UK by clinical Phase year-on-year (2017-2022)



## 2.11 Consistent split between autologous and allogeneic cell therapy trials

There has been an increase in the overall number of ATMP clinical trials (for both autologous and allogeneic therapies) observed as ongoing each year since 2017, however, the proportion of autologous to allogeneic products has remained consistent, with approximately 70% being autologous and 30% allogeneic, Figure 11.

Figure 11. Distribution of ongoing autologous and allogeneic cell therapies clinical trials in the UK from 2017-2022



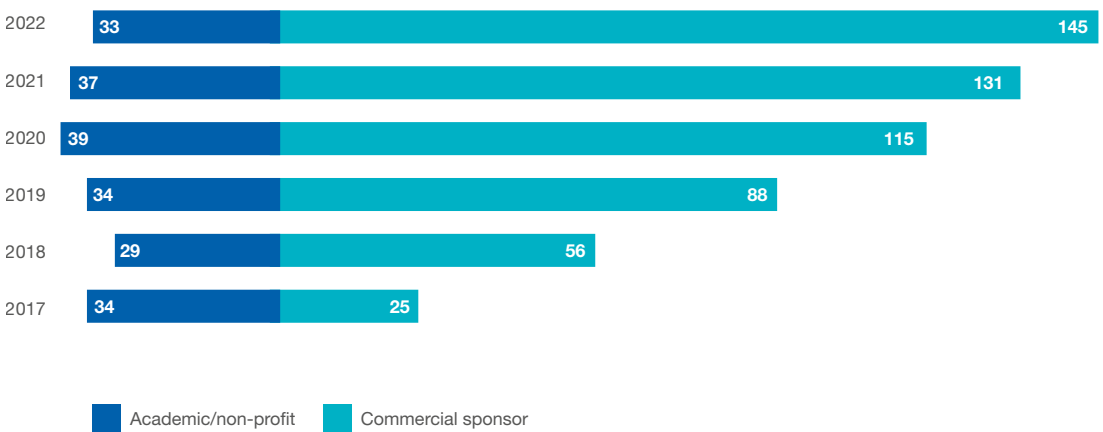
## 2.12 Growing number of commercially sponsored trials in the UK

From 2017 to 2018, most UK clinical trials were supported by academic/non-profit organisations. More recently, we have seen the emergence of commercial clinical trial sponsorship building from this base.

Commercially sponsored trials accounted for just over 80% of all UK ATMP clinical trials in 2022, demonstrating the attractiveness of the UK ecosystem, in particular the Advanced Therapies Treatment Centres (ATTC) network, to commercial sponsors.

This year we once more saw an increase in the number of commercially sponsored clinical trials from 131 to 145, Figure 12. Whereas the number of academic/non-profit clinical trials decreased slightly from 37 to 33.

Figure 12. Numbers of academic/non-profit and commercial of ongoing ATMP clinical trial from 2017-2022







### 3. Favourable ecosystem for ATMP trials in the UK

The Advanced Therapy Treatment Centres network (ATTC; <https://www.theattnetwork.co.uk>), coordinated by the CGT Catapult, continues to grow and provide more developers of advanced therapies with a clear route to adoption and commercialisation within the UK. This is essential for the potential of these therapies to be realised and for the UK to achieve the ambition of building a world leading advanced therapy industry and maximising patient access to these therapies.

As of February 2022, the ATTC network consists of 114 organisations, including 25 NHS Trusts and 59 ATMP industry partners. The proportion of ongoing clinical trials run a dedicated ATTC network site has increased consistently both in the UK (from 39% to 62%) and representation in global trials (from 2% to 5%) over the last 5 years.

### 4. CGT Catapult UK ATMP clinical trials database – 2022 conclusions

The 2022 CGT Catapult UK ATMP clinical trials database reveals an industry that continues to grow and mature with an increase in the number of trials ongoing in the UK, even following the COVID-19 pandemic and Brexit. The 2022 data continues to demonstrate a year-on-year increase in clinical trials in the UK with 178 trials currently ongoing. This equates to representation in 8% in all global trials and 14% of Phase I-III commercial trials, as described in the 2022 ARM report. The steady state of ATMP clinical trial numbers in the UK contrasts with the global landscape, with the Alliance for Regenerative Medicine (ARM) observing an overall 13% decline in ongoing ATMP clinical trials worldwide.

There has been a further increase in commercially sponsored trials, now accounting for approximately 80% of all ATMP trials in the UK. This increase reflects the favourable landscape for ATMP trials in the UK, supported by investments and partnerships including the Advanced Therapy Treatment Centres (ATTC) network and other UK government led initiatives.

# 5. Appendix

## 5.1 The purpose of the CGT Catapult UK ATMP clinical trials database

The CGT Catapult is an independent innovation and technology organisation committed to the advancement of cell and gene therapies with a vision of a thriving industry delivering life changing advanced therapies to the world. Its aim is to create powerful collaborations which overcome challenges to the advancement of the sector. With over 400 experts covering all aspects of advanced therapies, it applies its unique capabilities and assets, collaborates with academia, industry and healthcare providers to develop new technology and innovation.

The UK ATMP clinical trials database aims to assess the progress and state of the UK ATMP clinical development landscape. The database is updated annually and provides, what we believe to be, a comprehensive and accurate review of the UK ATMP clinical trial landscape. The input of the cell and gene therapy community is important however to help us maintain its relevance, and we welcome updates, additions and corrections, which can be sent to us at [clinicaldatabase@ct.catapult.org.uk](mailto:clinicaldatabase@ct.catapult.org.uk).

Several countries and organisations now produce reports detailing the current state of clinical trials in the field of “regenerative medicine”, of which ATMPs is a specialised subset. These activity reports, however, utilise different methods to identify and analyse trial activity and encompass more than just ATMPs, making global comparisons increasingly difficult. It is important to note that CGT Catapult data may differ from other databases which often include cell therapies which do not fulfil the criteria for ATMPs or may include the same trial in more than one category. To generate our data, we used consistent and systematic methods to interrogate and refine automatically generated data using the comprehensive GlobalData Database. This method identified and removed trials investigating products/therapies not complying with European ATMP definitions (e.g., non homologous use/minimally manipulated products), and ensured each trial was only counted once in the analysis. It is also important to note that ARM data is significantly different this year compared with previous years with substantially more Asia-Pacific, investigator-led and non-commercial trials included than previous years.

## 5.2 Methodology

ATMP clinical trials conducted in the UK were identified through a targeted search of GlobalData’s clinical trial database<sup>3</sup>. Using these data, trials that did not have a publicly available ID (such as a Clinical Trials or Eudra CT number), and which were unable to be verified outside of GlobalData, were then excluded.

The products used in the remaining clinical trials were then assessed for whether they fulfilled the category of ATMPs, as outlined in Directive 2001/83/EC and amended by the ATMP Regulation 1394/2007.

To ensure each trial identified was only counted once in the analysis, products that could fall under the definition of more than one ATMP type were assigned to a single type. For example, a product (such as a CAR-T) that was reported as both a gene and cell therapy in the GlobalData database, was counted once as an *ex vivo* gene therapy in this report. Clinical trials investigating multiple indications were also only assigned to a single therapeutic area.

Key trial information was verified using at least one of the following methods:

- Confirmation by Sponsor or other key clinical trial stakeholders (such as participating sites)
- Confirmation in the public domain such as by way of:
  - EU Clinical Trials Register
  - ClinicalTrials.gov
  - ISRCTN Registry
  - NIHR CRN Public Dashboard
  - WHO International Clinical Trials Registry Platform

UK ATMP trials for which key trial information was unable to be verified outside of GlobalData, using one of the methods above, were excluded from the analysis.

<sup>3</sup> <https://www.globaldata.com>



## View the Cell and Gene Therapy Catapult Clinical Trials Database 2022

The UK ATMP clinical trials included in this report are provided in our Clinical Trial Database 2022. The database provides more information about each of the UK clinical trials including sponsor, project title, clinical database numbers, site, status, phase and year started. The database also states relevant cell types used, gene modification/gene therapy, vector type, autologous/allogeneic, disease area and indication.

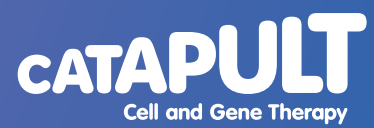


Scan the QR code to view the full Cell and Gene  
Therapy Catapult ATMP Clinical Trials Database 2022.

<https://ct.catapult.org.uk/resources/clinical-trials-database>







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