# Gene Therapy Development Programs are Both Growing and Evolving in the Face of Commercial Headwinds


## Authors

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## Objectives

- Gene therapies (GTx) are transformative, offering potential cures for both genetic and acquired diseases.
- As some GTx struggle in the marketplace and manufacturers recalibrate development programs, understanding recent development trends is critical for health economics, market access, and policy.
- We evaluate the GTx pipeline from 2020–2025, focusing on clinical stage progression and therapeutic areas.

## Conclusions

- Clinical-stage development from 2020–2025 reflects sustained growth.
- However, there is a marked pullback in numbers of therapies in preclinical development.
- While Oncology and Rare Diseases remain central, growth in more common conditions signals broadening patient populations and potential market expansion, along with continued concerns about affordability.
- There will be ever greater pressure on public and private initiatives to solve the regulatory, value assessment, and reimbursement challenges facing GTx.

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## Methods

Data from American Society of Gene & Cell Therapy's (www.asgct.org) quarterly reports were abstracted. Measures included the number of gene therapy (GTx) programs across preclinical and clinical development phases, as well as therapeutic area. Descriptive analysis was performed to assess trends from 2020-2025.

## Results

Figure 1 shows that the overall GTx pipeline expanded rapidly between 2020 and 2022 (16-30% annually), followed by slow, positive growth in clinical development through 2025 (2-3.5% annually).

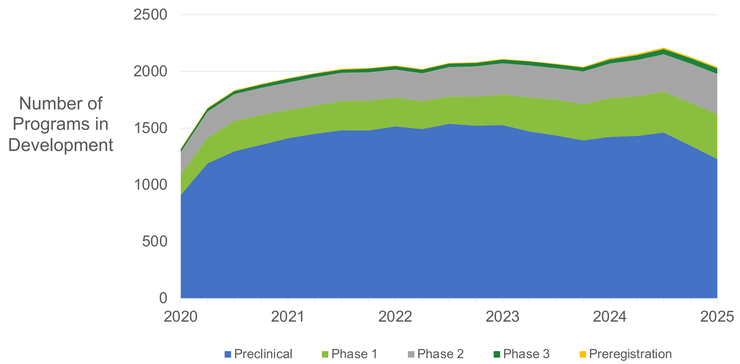


**Figure 1. Number of gene therapies in preclinical development through FDA review**

As shown in Figure 1, the number of programs in preclinical development has dropped recently: a 20% decrease since 2023. However, the number of clinical-stage programs has grown over 35% over the same period. Figure 2 depicts the resulting trend. Programs in Phases 1–3 rose from 35% of the pipeline in 2022/2023 to over 60% by 2025, indicating accelerated maturation of candidates and a slowing of preclinical development.

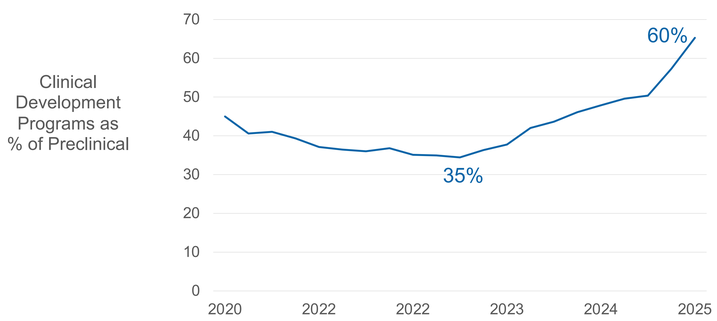


**Figure 2. In-clinic GTx programs as % of preclinical programs**

Clinical stage programs in most therapeutic areas (TAs) have grown since 2021 (Fig. 3). Oncology and Rare Diseases are dominant, with 74% of programs in 2025. Programs targeting more prevalent acute and chronic conditions showed momentum, with an 87% increase since 2020 compared to a 31% rise in Rare and Oncology programs. The only TA without significant growth is Anti-Infectives, which saw substantial expansion of mRNA and RNAi modalities.

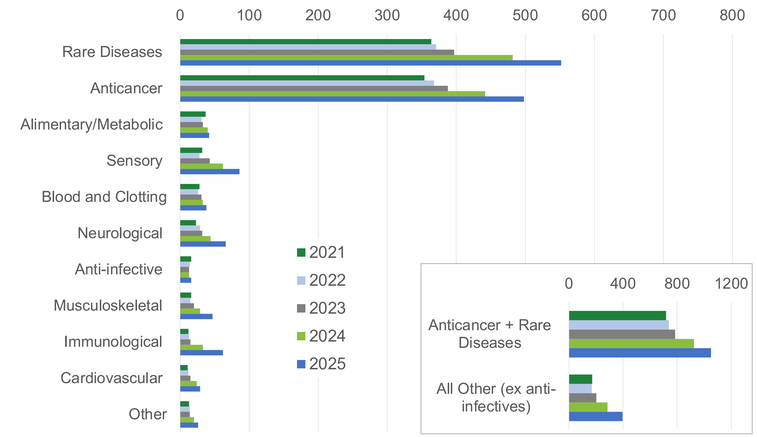


**Figure 3. Number of clinical development programs by TA**

Reflecting overall preclinical development trends, the distribution of preclinical programs by TA shows largely downward trends. Rare Disease and Oncology programs have dropped nearly 25% in recent years. Programs targeting Immunology and Cardiovascular show counter-trends, rising over 5% recently.

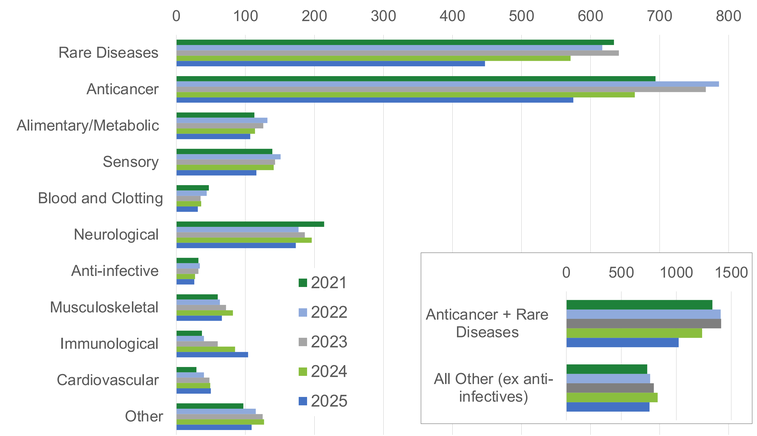


**Figure 4. Number of preclinical development programs by TA**