Principles for Ethical Application of Gene Editing in the Clinic

A Perspective from Industry

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Global Overview

57 Therapeutic Developers Active in Gene Editing

Companies include:
- Adaptimmune
- Agenovir
- Allergan
- Allogene Tx
- Astellas Pharma
- Baxalta/Shire
- Beam Tx
- Biogen
- bluebird bio
- BlueRock Tx
- Caribou Bio
- Casebia Tx
- Cellectis
- CRISPR Tx
- EdiGENE Corp
- EdiGene Inc
- Editas Medicine
- Eliigo Bio
- Elixirgen
- Exonics Tx
- Fate Tx
- Feldan Tx
- Healios
- Homology Medicines
- Intellia Tx
- Intrexon
- Juno Tx / Celgene
- Kedgene Bio
- Kite / Gilead
- Les Laboratoires
- LogicBio Tx
- MaxCyte
- MedGenCell
- Merck
- Mustang Bio
- Nanjing Bioheng Bio
- Nanjing KAEDI Bio
- Novartis
- PACT Pharma
- Pfizer
- Poseida Tx
- Precision Bio
- Precision Virologics
- Regeneron
- Roche
- Rocket Pharma
- Sangamo Tx
- Sanofi
- Senti Bio
- Shanghai Bioray
- Spotlight Tx
- Tmunity Tx
- ToolGen
- Universal Cells
- Vertex Pharma
- Verve Tx
- ViaCyte

*Companies with clinical stage genome editing programs in **bold**; companies primarily utilizing gene editing technologies to modify cell therapies for applications in oncology are **italicized**.
There are currently 31 active, ongoing clinical trials involving gene editing

- 45% are Phase 1 and 55% are Phase 1/2.
- 85% are *ex vivo* and 15% are *in vivo*.
- The technologies used in these trials include:
  - CRISPR/cas9 (52%)
  - ZFN (29%)
  - TALEN (16%)
  - Other (6%)

**Clinical Trials by Indication**

- **Oncology, 20***
  - Hematology, 5
  - Infectious Diseases, 3
  - Endocrine, Metabolic, & Genetic Disorders, 2
  - Ophthalmology, 1

*All clinical trials active in oncology are in gene-edited cell therapies (CAR-Ts, TCRs)*
Active Trials in Genome Editing Worldwide*

31 Active Trials in Genome Editing Worldwide*

North America
United States: 19
Canada: 1

Europe
UK: 6
France: 2
Germany: 2
Belgium: 1
Italy: 1
Spain: 1

Asia
China: 10
Japan: 1

The country figures add up to 44 because 7 clinical trials have active clinical sites in multiple countries.