

# Principles for Ethical Application of Gene Editing in the Clinic

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## A Perspective from Industry

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**57** Therapeutic Developers  
Active in Gene Editing

## Companies include:

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

35

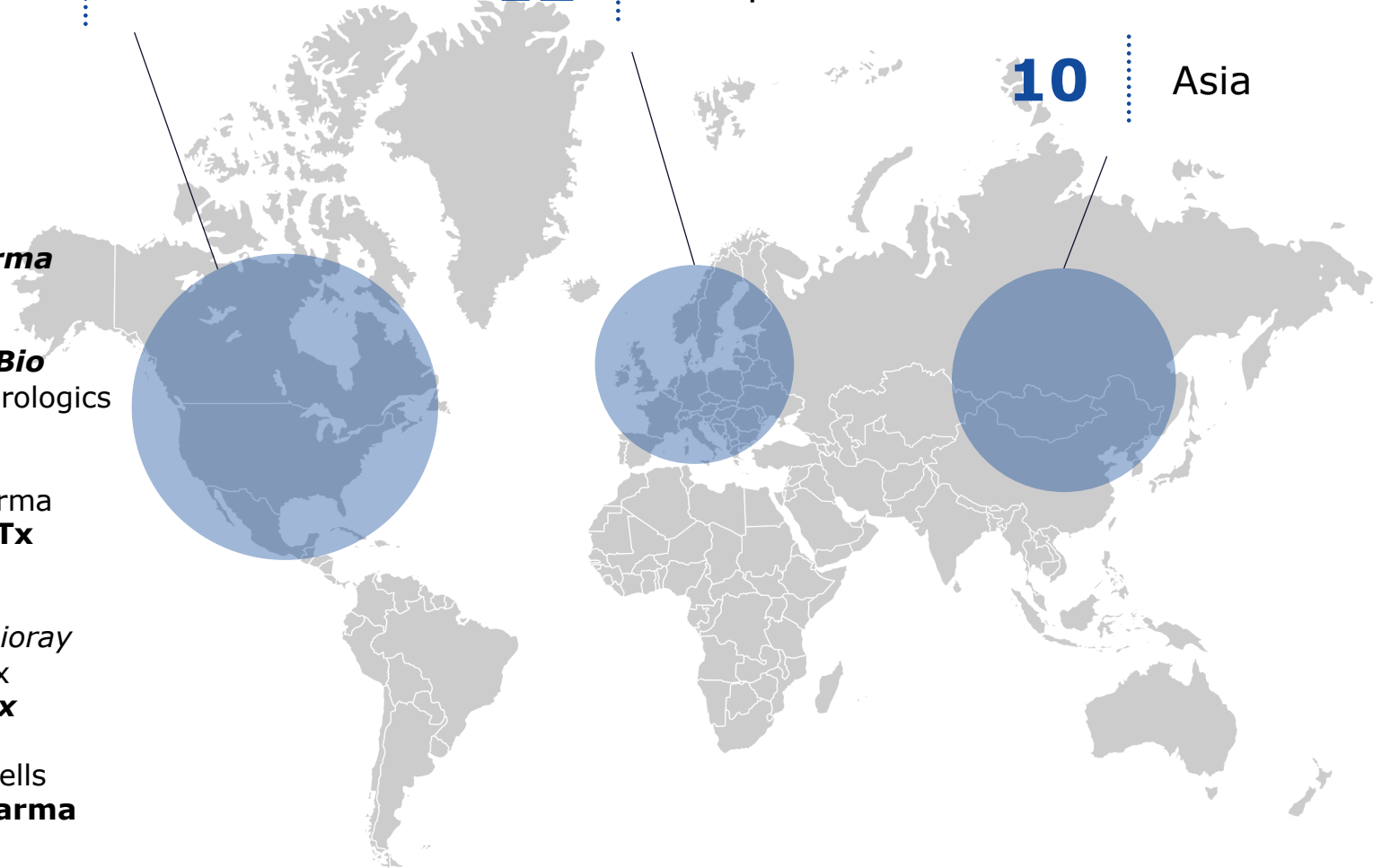
North America

12

Europe

10

Asia



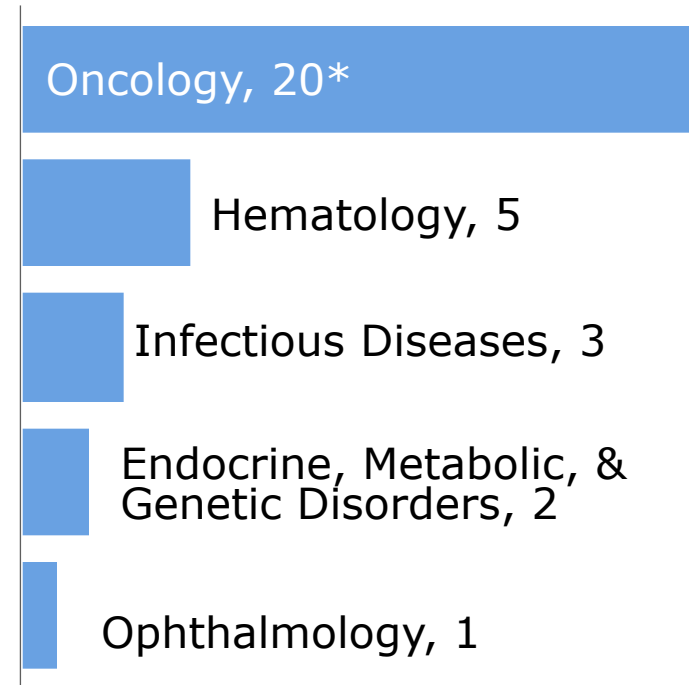
\*Companies with clinical stage genome editing programs in **bold**; companies primarily utilizing gene editing technologies to modify cell therapies for applications in oncology are *italicized*.

# Clinical Pipeline for Gene Edited Therapies

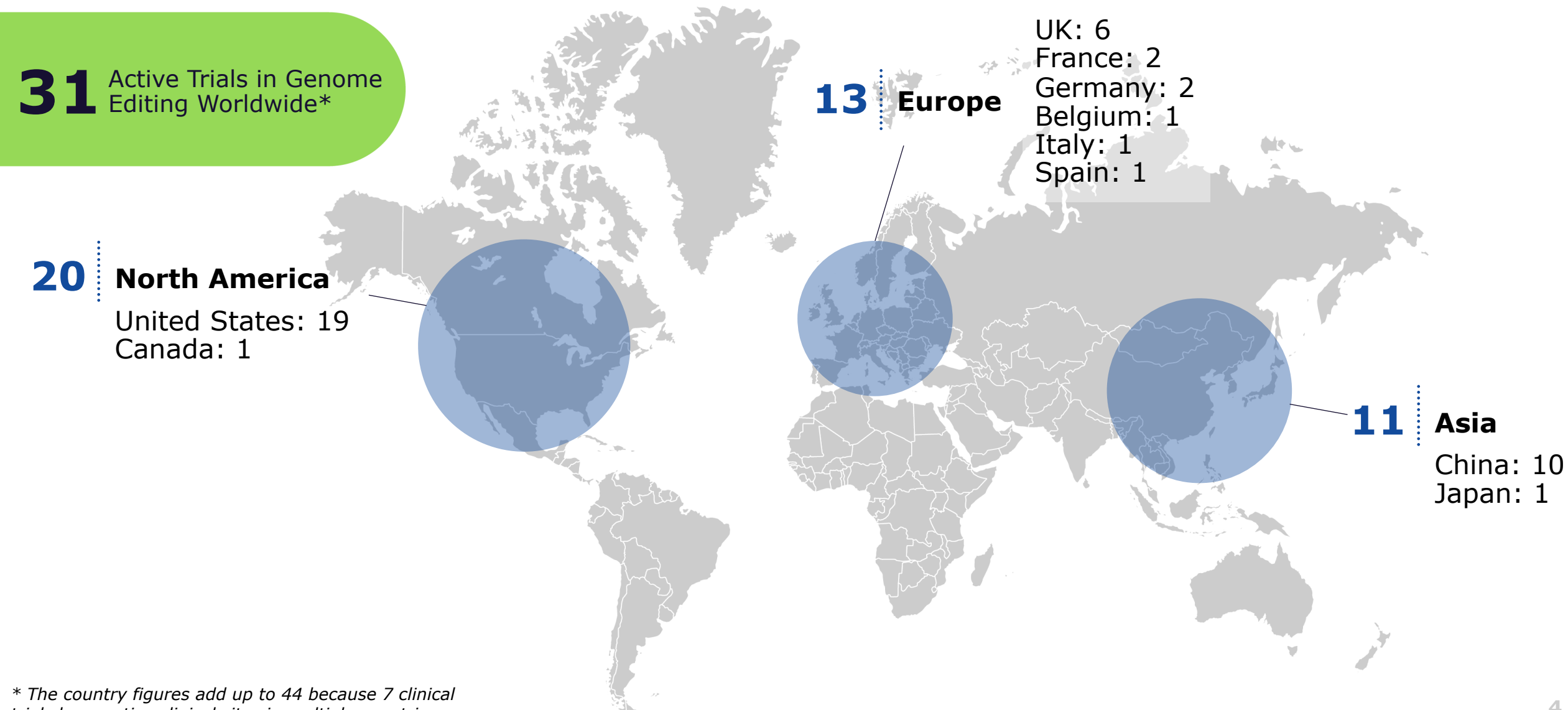
## There are currently 31 active, ongoing clinical trials involving gene editing

- 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



# Genome Editing Clinical Trials by Country



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