



NOVEMBER 2020

# Vector-Based Gene Therapy and Gene-Editing Products On Going Clinical Trials

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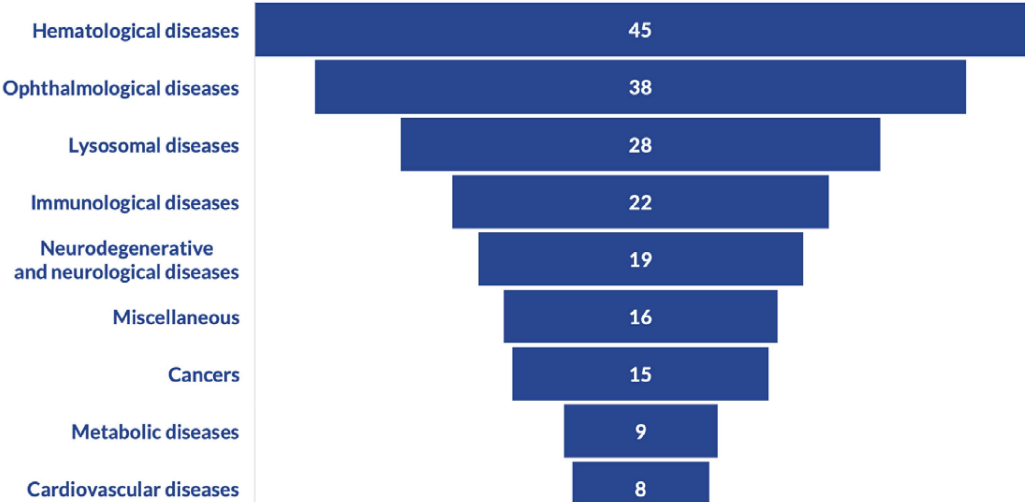
This study is based on data extracted from Clinicaltrials.gov. Selected trials have been registered and/or updated until 11/07/2020. Trials have been chosen on the basis of the following criteria:

- Interventional studies containing the term "gene therapy"
  - Recruitment status (Not yet recruiting – Recruiting - Enrolling by invitation - Active, not recruiting)
- CAR-T cells and oncolytic viruses have been excluded.

# 211 Ongoing Trials in More than 10 Therapeutic Areas

Hematological diseases, ophthalmological diseases and lysosomal diseases represent more than half of ongoing trials (52.6%).

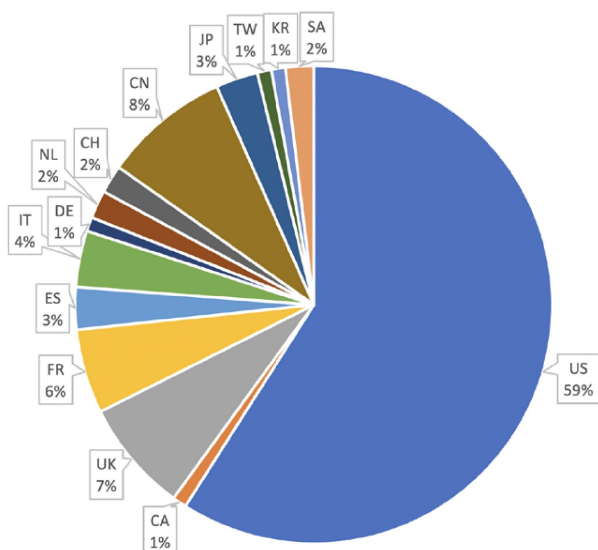
**Trials Distribution by Therapeutic Area**



# 106 Sponsors from 13 Countries Have Initiated These 211 Ongoing Trials

- 59% of trial sponsors come from the US
- 24.5% of trial sponsors come from 7 European countries. (France, Germany, Italy, Netherlands, Spain, Switzerland and United Kingdom)
- 15.1% of trial sponsors come from 4 Asian countries. (China, Japan, Republic of Korea, Saudi Arabia)

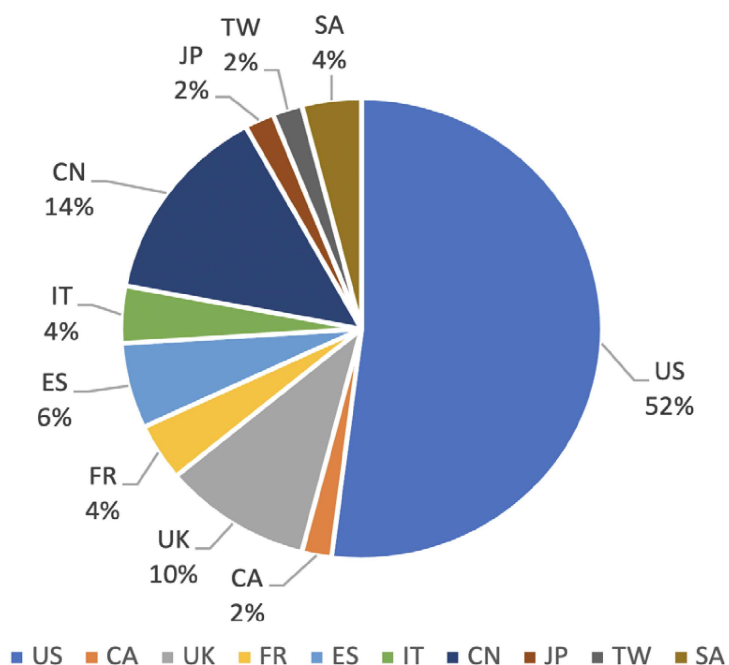
**Geographical Breakthrough of Trial Sponsors**



## 50 Academic Sponsors are managing 65 of these 211 trials (30.8% of trials)

- 52 % of these sponsors are located in the US
- 24% of these sponsors are located in 4 European countries (France, Italy, Spain, United Kingdom)
- 22% of these sponsors are located in 4 Asian countries (China, Japan, Saudi Arabia, Taiwan)

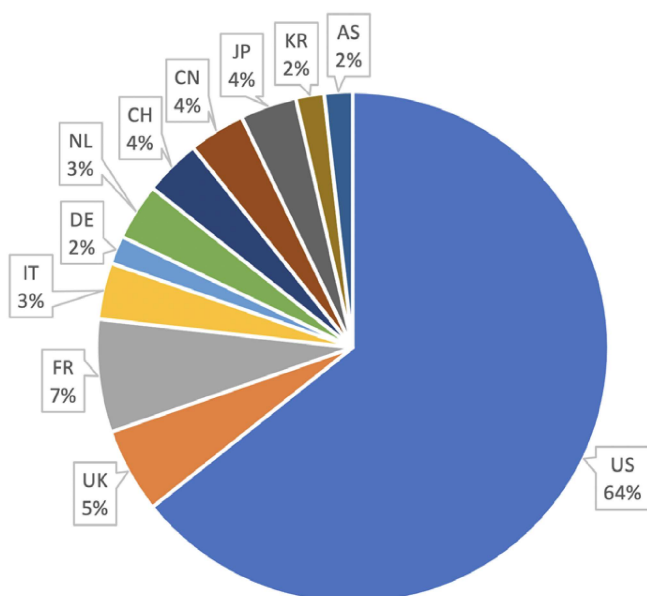
**Geographical Distribution of Academic Sponsors**



## 56 Industrial Sponsors are managing 146 of these 211 trials (69.2% of the trials)

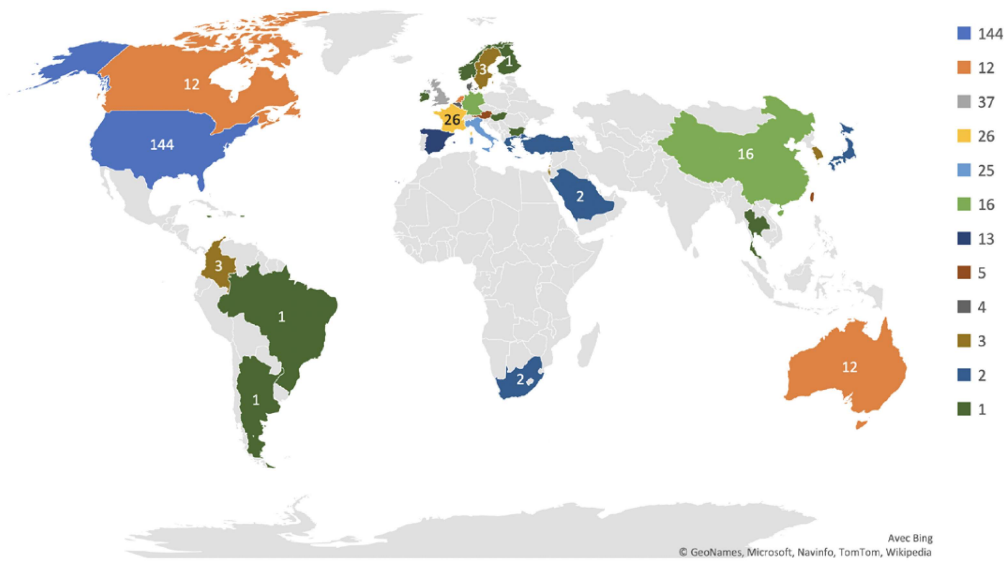
- 64 % of these sponsors are located in the US.
- 19.6% of these sponsors are located in 6 European countries (France, Germany, Italy, Netherlands, Switzerland, United Kingdom).
- 8.9% of these sponsors are located in 3 Asian countries (China, Japan, Republic of Korea).

### Geographical Distribution of Industrial Sponsors



# 68 % of On Going Trials with At Least One Location in the USA

## Breakthrough by country

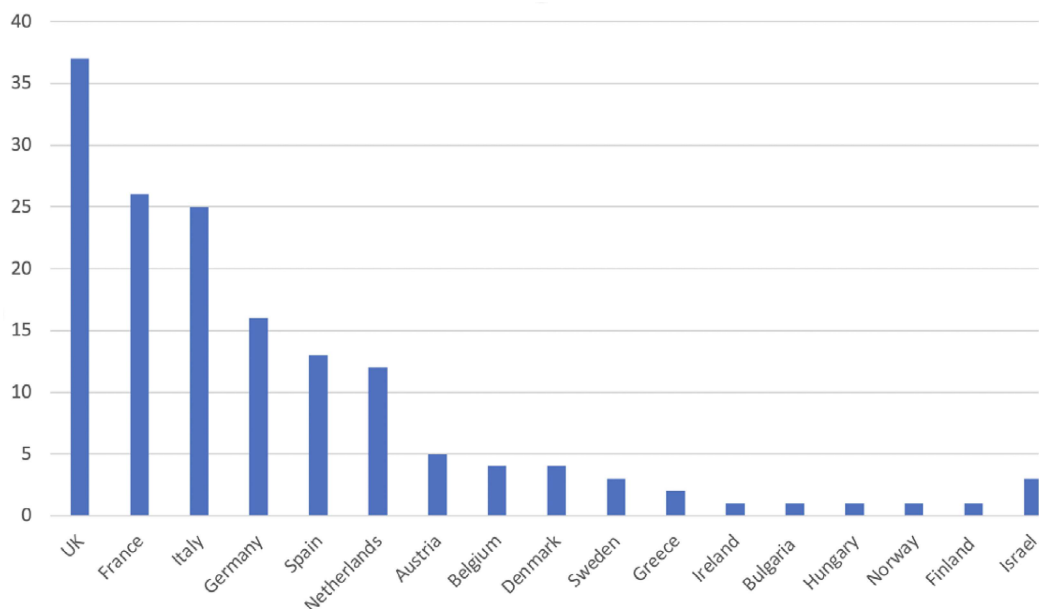


# 72% of On Going Trials with At Least One Location in Europe

Top 5 European countries are:

- UK (37 trials)
- France (26 trials)
- Italy (25 trials)
- Germany (16 trials)
- Spain (13 trials)

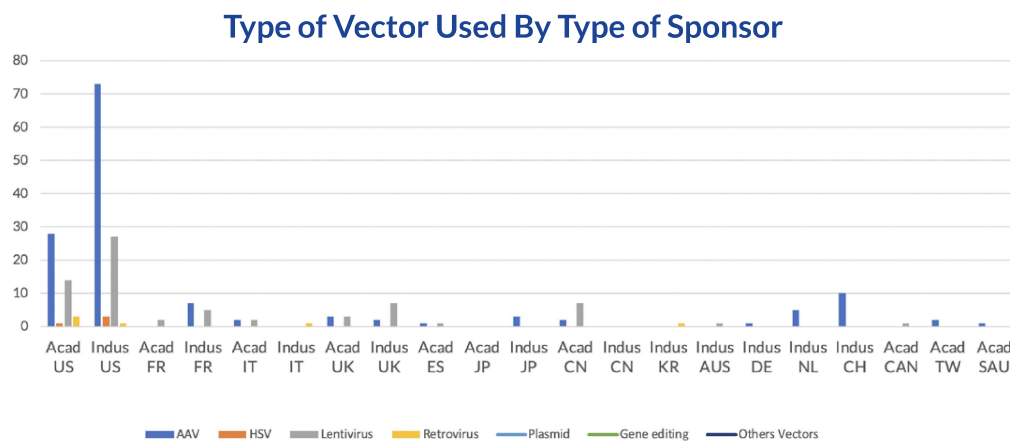
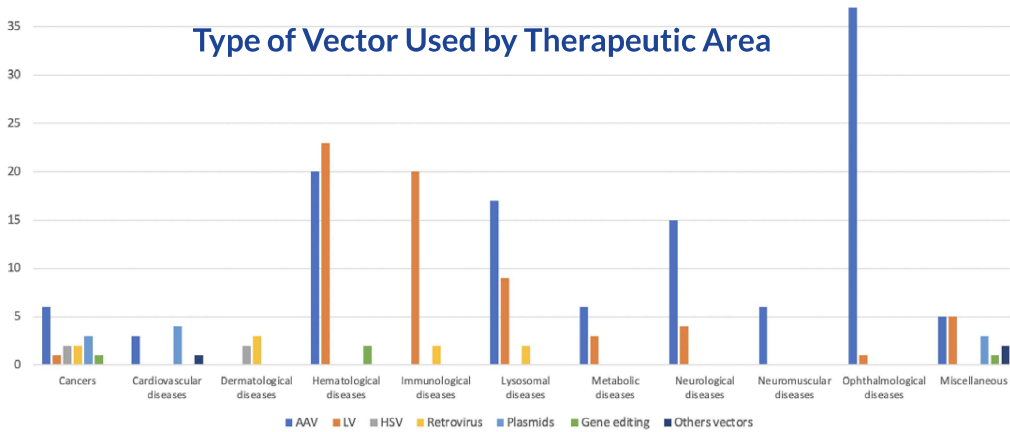
### Trials Distribution in European Countries





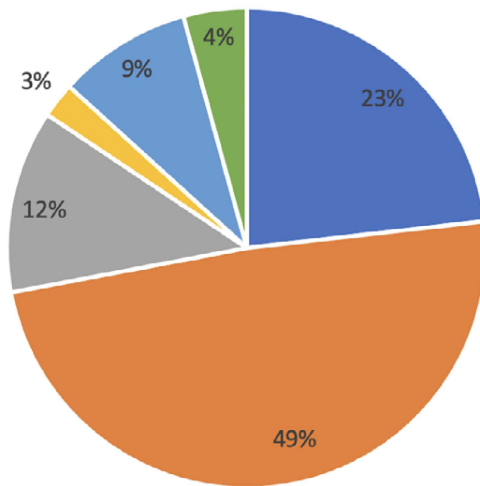
# AAV-Based Vectors and Lentiviral Vectors are The Most Widely Used Vectors

- 54.5% of the trials are using AAV-based vectors
- 31.3% of trials are using lentiviral vectors



# 72 % of Ongoing Trials are Early-Phase Trials (I or I-II)

Breakthrough by Phase



■ Phase I ■ Phase I-II ■ Phase II ■ Phase II-III ■ Phase III ■ Phase not applicable

# Hematological Diseases and Ophthalmological Diseases Are The Most Advanced Therapeutic Areas

Phase by Therapeutic Area

