

LIGHT OF HOPE

What Makes Rare Disease Clinical Trials Successful?

 Patient
Enrollment

 Funding
Support

 Unmet
Medical Need

 Future
Trends





Rare Diseases

Have The Most Clinical Trials



Clinical Trials Shed Light On Leukemia

Incidence rate
2011-2015:

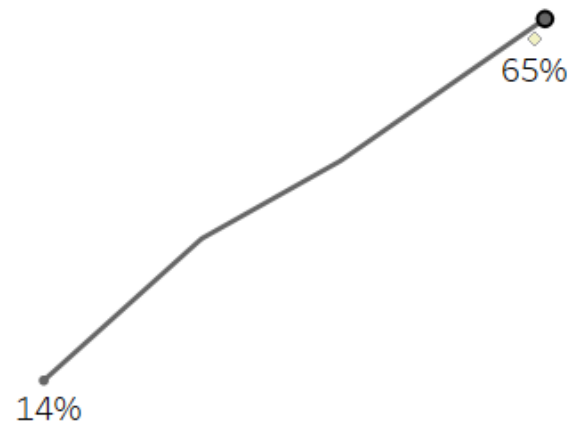
13.8 in
100,000
in US



The 5-year survival
rate for Leukemia
has increased

+ 51% 

From 1960 to 2014





Optimization Of Sampling

For Scientific Success On Clinical Trials:

Overall Survival Rate 



All Gender



Adult, Older Adult



Multi-location

For scientific acknowledgment



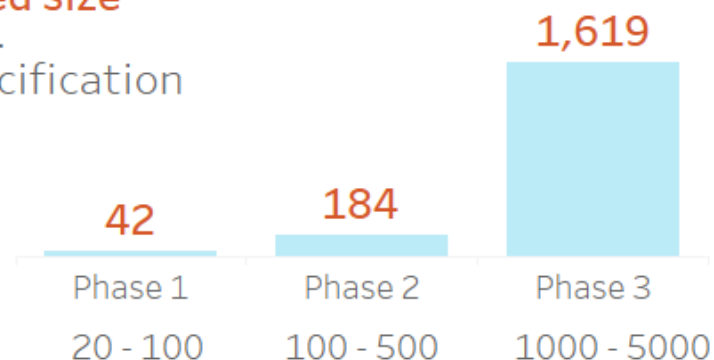
Enrollment Size

Depends on trial design

42% clinical trials are completed with multi-location for:

- * Data Reproductivity
- * Manipulation Reproductivity
- * Genetic Background Variation

Optimized size
vs.
FDA Specification





Monetization Success On Clinical Trial:

Cost Efficiency and Complete Rate ▲

Collaboration With Institutes To Save Cost And Time

1

Research Institute Cooperation



Acquire compounds for clinical trials to lower preclinical cost

2

Enrollment Management



80% of trials miss deadlines, resulting in **\$1.3M/day** loss

3

Patient Monitoring



30% average dropout rate

Years

5

6.5

8

13

15

%
of total R&D
investment
per new drug
(\$1.5-2B)

30%

Drug Discovery

Preclinical Phase

20%

Clinical Trials

Phase 1

Phase 2

30%

Phase 3

20%

FDA Review

Market



Single Funding Yields More Successful Trials

Industry Collaboration Adds On Integrated Infrastructure

Funding Contributors

77%

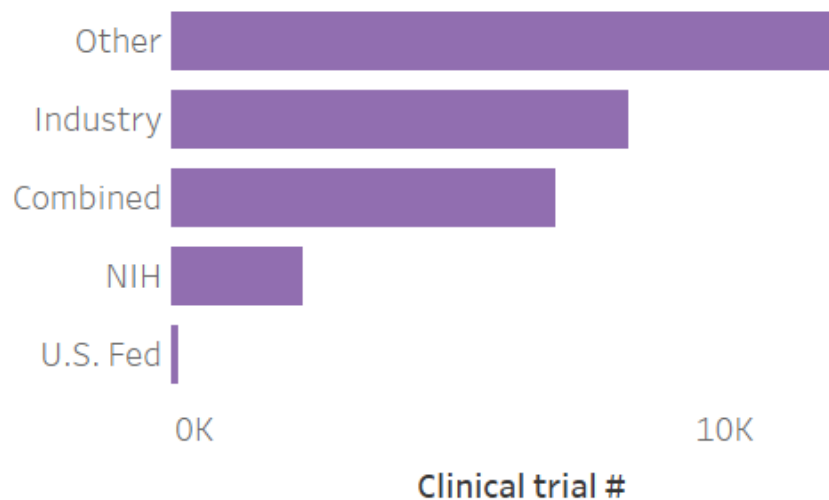
completed trials funded by other source, including private foundations, local research grants, and etc.

VS.

75%

fundings come from Industry in the US

Funding Sources



Industry Funding Advantages



statistics



Management



Cost-effectiveness

Public Funding Benefits



Scientific Success



Fundamental Research



Future Trends In Clinical Trials

20~30% ▼

cost from

Digitalization &
Patient Centricity



1

AI Enrollment Optimization

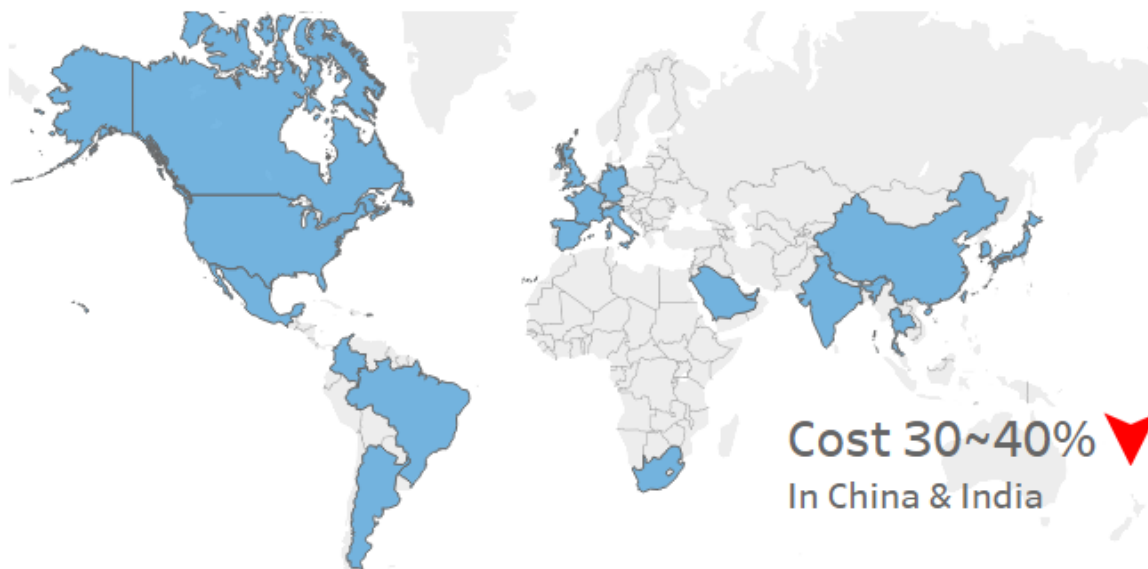
AI match trial and patient with Electronic Health Records and lab images

2

Medication Adherence and Control

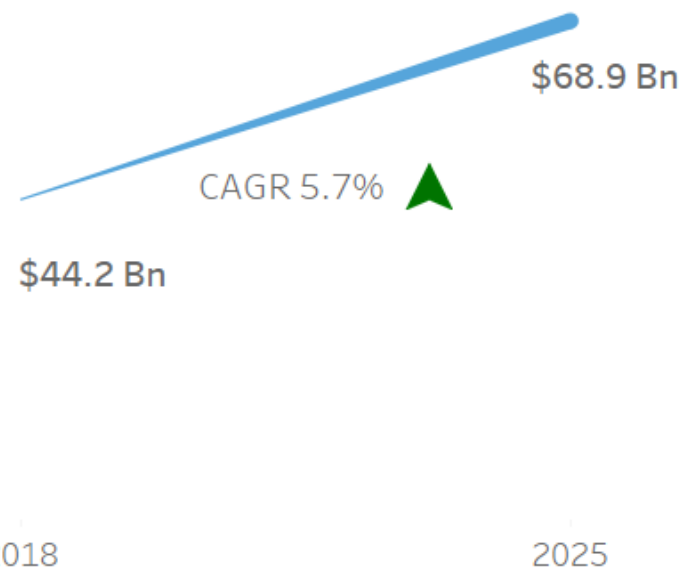
Global Trends

Move from US or Europe to other countries for:
Lower cost and easier patient recruitment



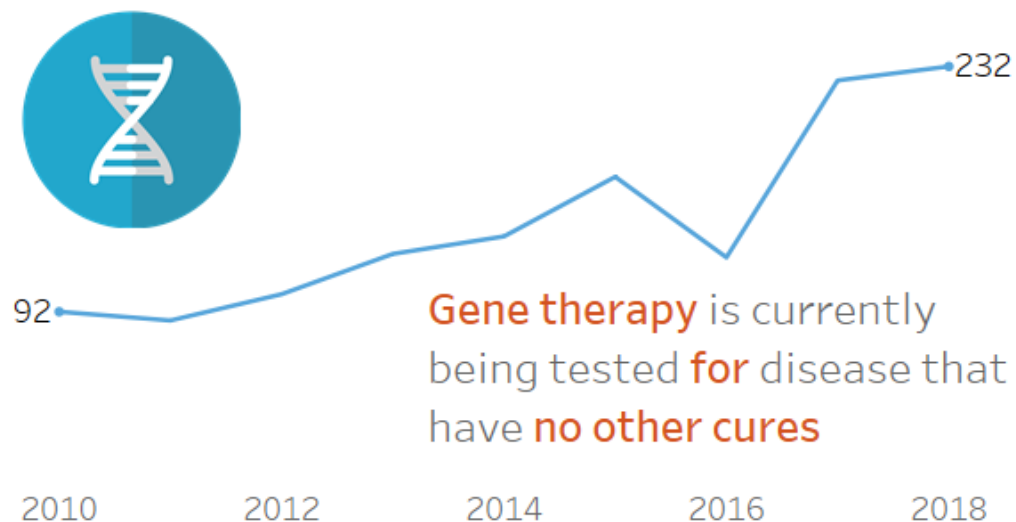
Global Spending

Industry continues to invest

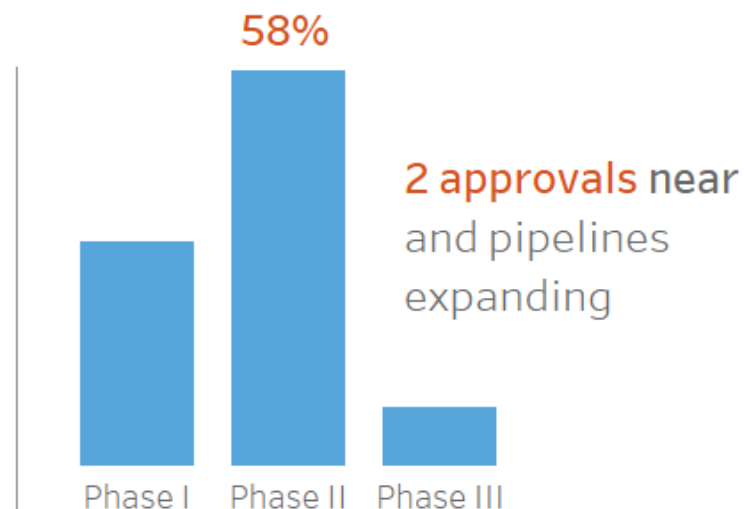


HOT! Gene Therapy

of Gene therapy clinical trials approved worldwide



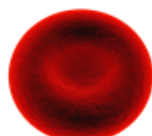
% of Clinical trials in phase, 2019 Q1



HOT! Immunotherapy



Cancer



Blood disorder



Eye disease

At least **2** patients **treated** from CRISPR-based therapy trials in the US.

FDA has **approved CAR-T** therapy and **PD-1** pathway inhibitors that don't use genome editing.

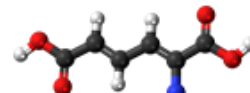
Top Areas

1 Oncology

2 CNS

3 Infectious disease

4 Metabolic Disorder





Meeting Unmet Medical Need Leads To Faster FDA Approval

However, Approval Time Varies Depending On Diseases

Example: Comparison of Interventions for Leukemia

Clinical Trials On Phase 3



Drug

7 Years

Faster effect for observation



Biologic

8 Years

Complex mechanism needs evaluation



Genetic

**Positive
Prediction**

Great potential for one-time fix

Faster Approval From FDA



Unmet Medical Need



Cancer Therapy

Sources

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