

# CURING RARE DISEASES

# TREATING DATA.

## 80%

"Percent of rare diseases with no treatment."

## 2000

"At the current rate that we are going through innovation it will take over 2000 years before every rare disease has a treatment."

## 5 - 8

"An average person with a rare disease will take 5 to 8 years to get diagnosed."

## 8%

"Out of all the money spent on drugs in the US, only 8% is spent on RARE diseases."

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### By the numbers:

Quantifying some of the United States' greatest health concerns and the medical field's research to combat these concerns

<h2>200,000</h2> <p>Number of people that rare diseases affect in the United States.</p>	<h2>7,000</h2> <p>Number of different rare diseases that are affecting families in United States today</p>	<h2>39,602</h2> <p>Number of clinical studies with posted results to ClinicalTrials.gov</p>	<h2>25 Million</h2> <p>Number of Americans who are affected by rare diseases, as a whole</p>
<h2>95%</h2> <p>Percentage of rare diseases that lack any FDA-approved treatment</p>	<h2>\$176 Billion</h2> <p>The amount of dollars projected to be spent on drugs to treat rare diseases</p>	<h2>\$137,000</h2> <p>Average cost for rare disease treatment, per person per year</p>	<h2>33%</h2> <p>Percentage of drugs approved by the FDA that were used to treat rare diseases</p>

### Most trialed diseases (study type = interventional):

#### Which diseases had the most clinical trials?

Multiple Myeloma	~1500
Melanoma	~1400
Acute Myeloid Leukemia	~1200
Ovarian Carcinoma	~1100
Alzheimers Disease	~1000
Pancreatic Cancer	~900
Non Hodgkin's Lymphoma	~800
Myelodysplastic Syndromes	~750
Chronic Lymphocytic Leukemia (CLL)	~700
Cystic Fibrosis	~650

These diseases are considered "common" (more than 200k cases per year)

#### Which RARE diseases had the most clinical trials?

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Acute Myeloid Leukemia	~1200
Ovarian Carcinoma	~1100
Pancreatic Cancer	~900
Myelodysplastic Syndromes	~800
Chronic Lymphocytic Leukemia (CLL)	~750
Cystic Fibrosis	~700
Renal Cell Carcinoma	~650
Glioblastoma	~600
Tuberculosis	~550

The majority of RARE diseases studied by the FDA are cancerous. These three are non-cancerous

### What makes a clinical trial successful?

We define success through the lens of monetization if a trial makes it to **Phase IV**. Success, as defined from the scientific perspective is much more complex, but is quantified here as **Phase III**.

Only 3% of the trials are in Phase IV. Phase where the company see's a ROI.

Trials involve several hundred participants.

Involves up to 3,000 participants with the condition that the new medication is meant to treat

Effects of the medication on 20 to 80 people

\*The FDA estimates that about 33% Trusted Source of medications move on to Phase III

\*https://www.healthline.com/health/clinical-trial-phases#phase-iv

### How does sampling effect trial success?

#### Does the AGE of the participants make a difference?

83% of the drugs are trialed on volunteers >18 y/o

The age variable does not necessarily correlate with a trial's success. The **key finding** is that a trial should not be limited by age constraints. By limiting participants through age restrictions, a trial has less chance of moving to **Phase III**

#### Does GENDER dictate a trial's success?

Drugs trialed on both male/female (All) are the most successful. When there's no gender constrain it's easier to find volunteers

### Who's funding the trials?

Private and public sponsors both participate alone or together

single fund	1,656
2 funds	978
3 funds	297
4 funds	71
5 funds	40
6 funds	18

- The most successful trials are the ones sponsored by only one company. One trial, one company reaches **Phase III** in 14.2% of the cases and **Phase IV** in 1.8% of the cases.
- Trials funded by only private institutions are a bigger piece of the cake when sponsors get together to fund a trial.
- Though only private fundings position 1.5% of all private funded trials in **Phase IV** both, private only and private & public or only public fundings manage to move a drug all the way to **Phase III** in 13% of the cases.

### Examining the 1,443 cases with combined sponsors:

Combined private institutions working on a same drug trial are 10% effective in going through all three Phases. When it gets to **Phase IV**, only 1% of the trials succeed.

Only 162 trials have a combined sponsorship including fundings from a public institution. When a trial is funded by both public and private institutions, there is no evidence that it can reach **Phase IV**.

Contains Public	only public	79
	public & private	83

### Where are clinical trials occurring?

#### Which states saw the most clinical trials per capita?

New York has the second highest amount of Clinical trials per capita

#### Which states saw the most "successful" trials per capita?

However, New York has one of the lowest amount of per capita trials that have made it to Phase III and Phase IV

### Magnifying the Texas case:

Is Texas' rank in per capita on clinical trials in Phase I & Phase II

Is Texas' rank in per capita on clinical trials in Phase III & Phase IV

1<sup>st</sup> TEXAS  
Ranks number ONE in the list of states with most trials in Phase III and Phase IV

## FINDINGS

- MOST OF THE RARE DISEASES ARE CANCEROUS
- 90% OF ALL INTERVENTIONAL TRIALS ARE FUNDED BY PRIVATE SPONSORS
- TRIALS THAT ARE SINGLE FUNDED ARE MORE SUCCESSFUL
- LOCATION MATTERS: NY STATE TRIALS LIKELY STAY IN PHASES I & II. TEXAS TRIALS MAKE IT TO PHASES III & IV