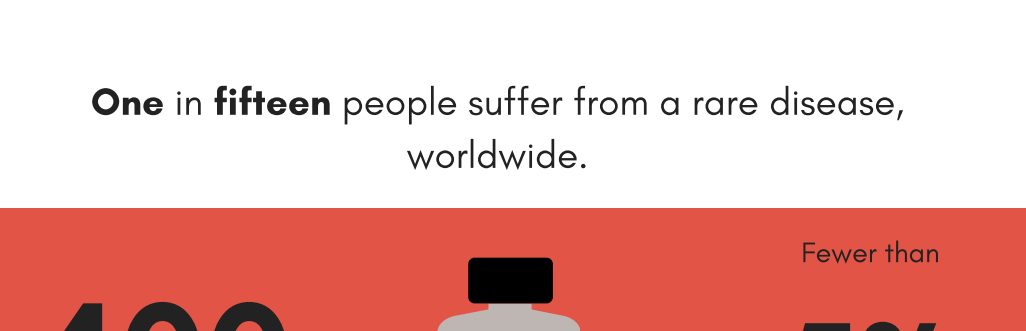


# WHAT MAKES RARE DISEASE TRIALS SUCCESSFUL?



Rare diseases are serious, chronic, and sometimes life-threatening conditions affecting fewer than 200,000 individuals in the United States.



One in fifteen people suffer from a rare disease, worldwide.

**400**

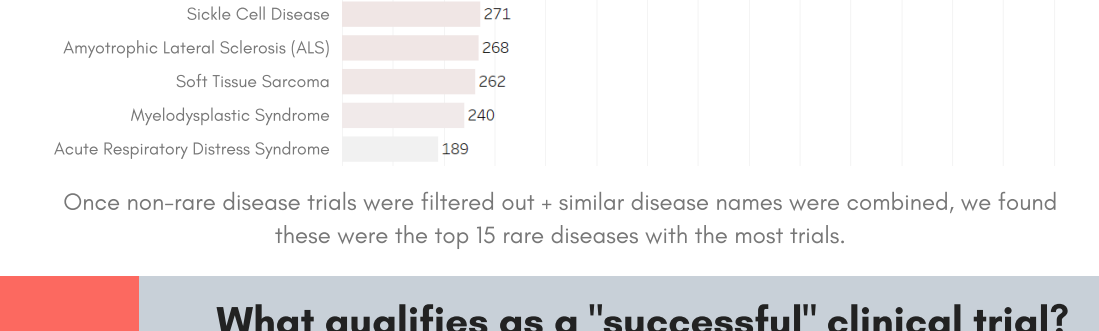
Rare disease treatments in development (approx).



Fewer than **5%**

of rare diseases have treatments.

## Which rare diseases have the most clinical trials?



Once non-rare disease trials were filtered out + similar disease names were combined, we found these were the top 15 rare diseases with the most trials.

## What qualifies as a "successful" clinical trial?

In most businesses, the only products deemed "successful" after the R&D process are those which can be sold for profit.

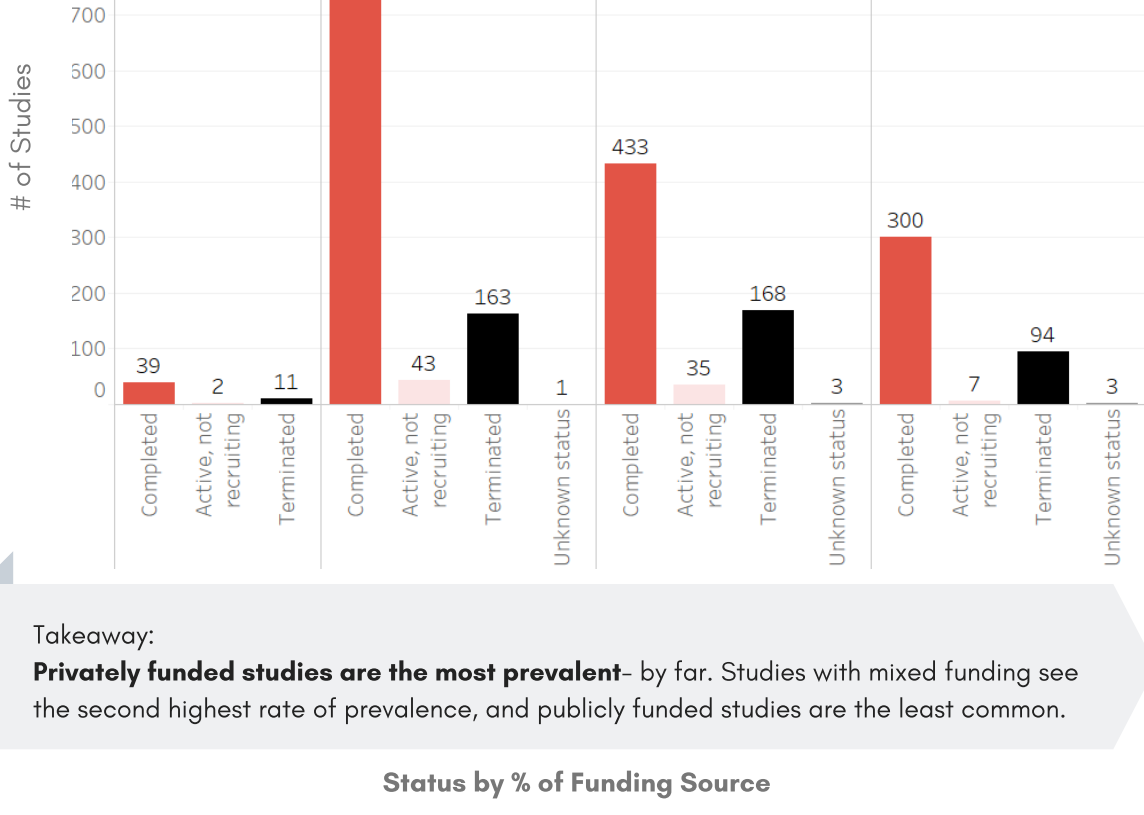
In the pharmaceutical business, however, our aim is to improve the lives of the patients and communities we serve.

Consequently, we believe any trial that reaches a status of "completion" is "successful" - because any trial that reaches its conclusion provides more information that can improve the standard of care and bring us closer to an effective treatment.

## Factors that Affect Trial Success

### 1. FUNDING SOURCES

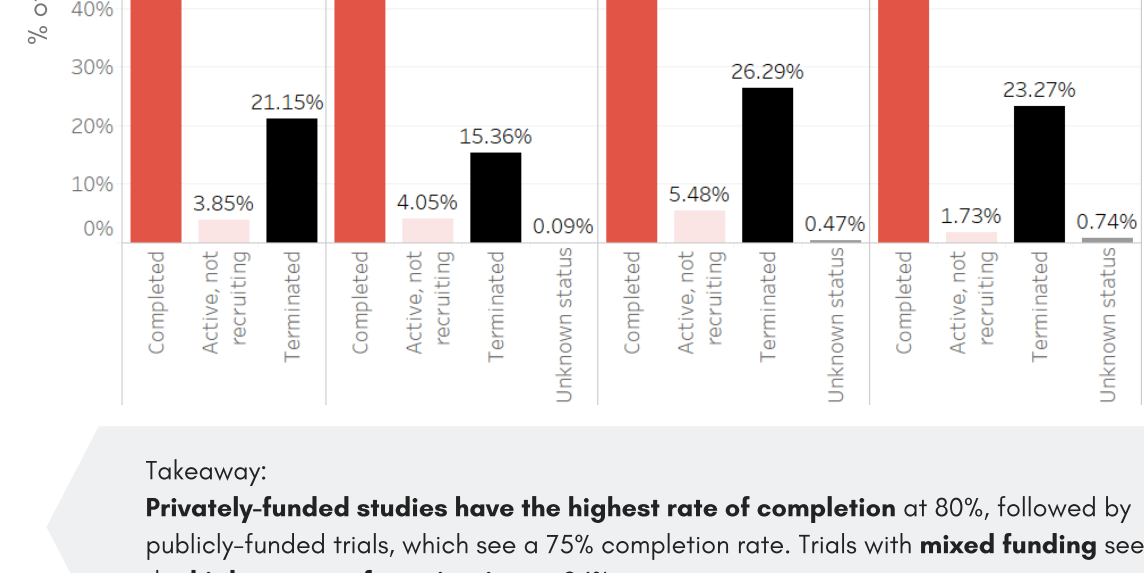
Count of Status by Funding Source



Takeaway:

Privately funded studies are the most prevalent - by far. Studies with mixed funding see the second highest rate of prevalence, and publicly funded studies are the least common.

Status by % of Funding Source

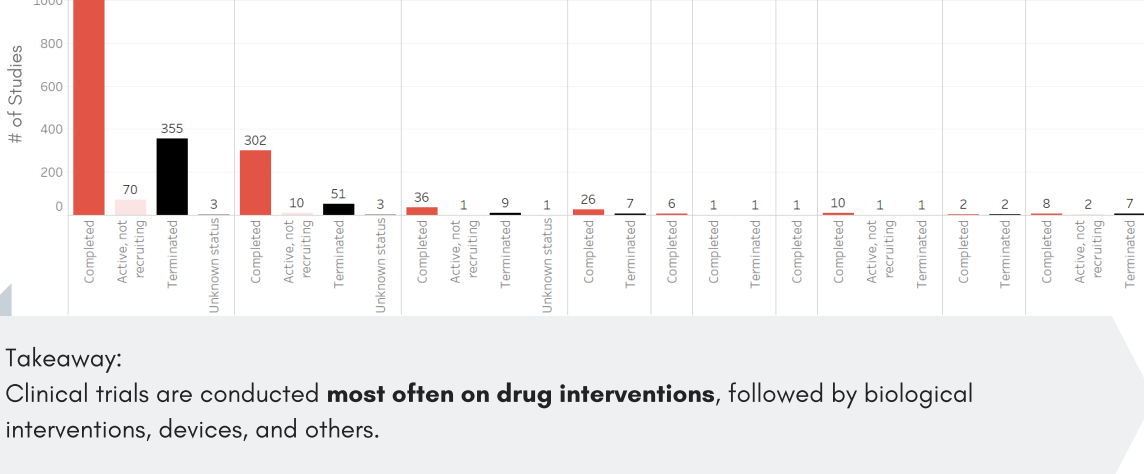


Takeaway:

Privately-funded studies have the highest rate of completion at 80%, followed by publicly-funded trials, which see a 75% completion rate. Trials with mixed funding see the highest rate of termination at 26%.

### 2. INTERVENTION TYPE

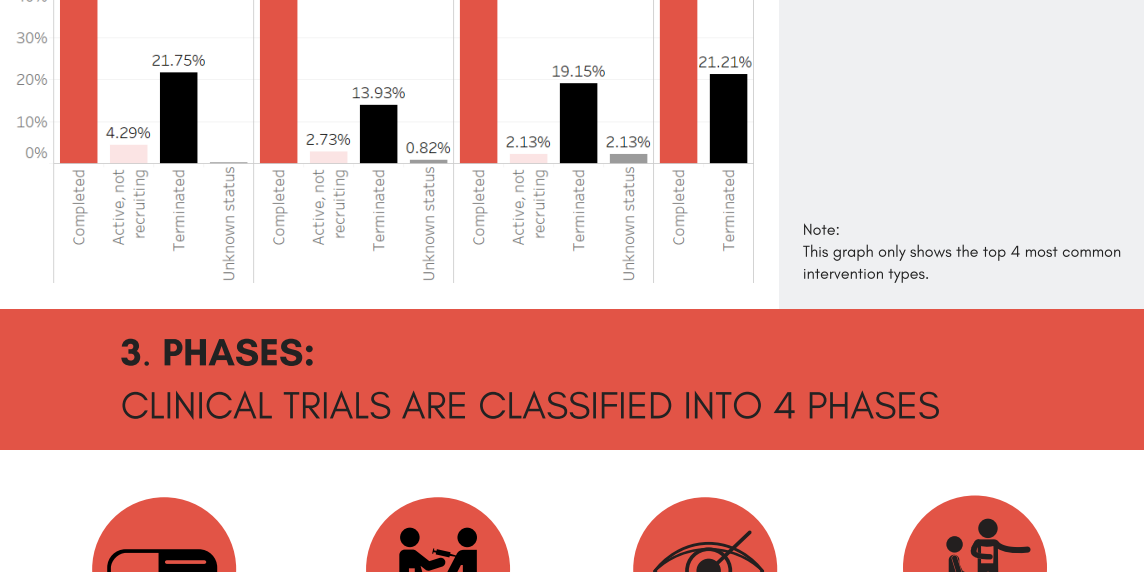
Count of Status by Intervention Type



Takeaway:

Clinical trials are conducted most often on drug interventions, followed by biological interventions, devices, and others.

% of Status by Intervention Type



Takeaway:

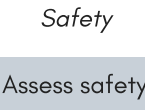
Biological interventions see the highest rate of trial completion, and drug interventions see the lowest rate of trial completion and the highest rate of termination.

Note:

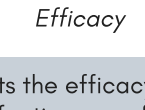
This graph only shows the top 4 most common intervention types.

### 3. PHASES:

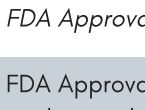
CLINICAL TRIALS ARE CLASSIFIED INTO 4 PHASES



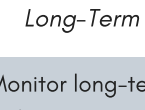
**Phase I**  
Safety



**Phase II**  
Efficacy



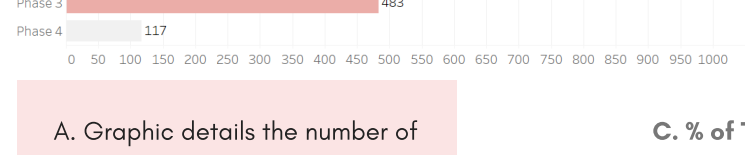
**Phase III**  
FDA Approval



**Phase IV**  
Long-Term

| GOALS                         | Phase I                       | Phase II  | Phase III  | Phase IV  |
|-------------------------------|-------------------------------|---|--|---|
| Assess safety + effectiveness | Assess safety + effectiveness | Tests the efficacy and effectiveness of the drug using placebos | FDA Approval, understand if benefits > risks           | Monitor long-term effectiveness, compare to other drugs |
| SAMPLE                        | 20-100 Healthy volunteer      | Several hundred volunteers with the condition                   | Several hundreds - thousands, randomized blind testing | Several hundreds - thousands                            |
| TIME                          | Several months                | Several months to a couple of years                             | 3-5 Years  | Typically, less than Phase III*<br>*Post-FDA Approval   |

A. # Trials with Results by Phase



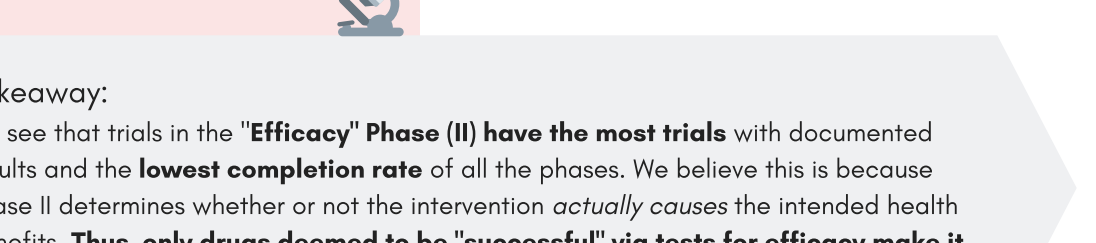
B. Average Phase Length

| PHASE   | YEARS |
|---------|-------|
| Phase 1 | 1.87  |
| Phase 2 | 2.28  |
| Phase 3 | 2.77  |
| Phase 4 | 1.32  |

A. Graphic details the number of recorded trials with results in each phase category.

B. Details the average length of completed trials in each phase, verifying that Phase III trials take the longest.

C. Graphic breaks down the percentage of each trial status by trial phase, giving us a view of the rate at which each phase is completed and terminated.



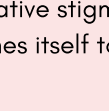
Takeaway:

We see that trials in the "Efficacy" Phase (II) have the most trials with documented results and the lowest completion rate of all the phases. We believe this is because Phase II determines whether or not the intervention actually causes the intended health benefits. Thus, only drugs deemed to be "successful" via tests for efficacy make it through to the following phases, Phases III and IV.

### 4. PATIENT-CENTRIC CARE

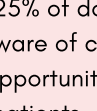
Patient recruitment and retention for trials is a key part of a trial's success.

Three factors that strongly affect a patient's likelihood of participating in a trial are:



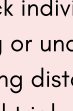
**PERCEIVED RISKS**

A negative stigma still attaches itself to clinical trials.



**PERCEIVED BENEFITS**

Only 25% of doctors are aware of clinical trial opportunities for their patients.



**DISTANCE FROM TRIAL**

Many sick individuals are unwilling or unable to travel long distances for a clinical trial.

## HOW DO WE IMPROVE CLINICAL TRIALS?

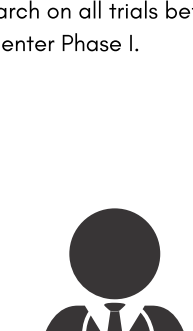
To ensure success, future clinical trials should:



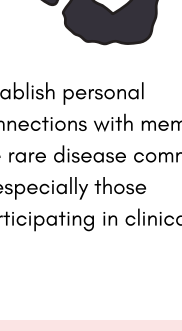
Examine and replicate techniques unique to privately funded trials in all trials.



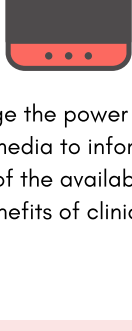
Invest in the future of clinical trials, such as digitalization of data collection and automation of data analysis.



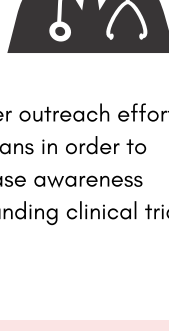
Conduct more thorough research on all trials before they enter Phase I.



Establish personal connections with members of the rare disease communities & especially those participating in clinical trials.



Leverage the power of social media to inform the public of the availability and benefits of clinical trials.



Further outreach efforts to clinicians in order to increase awareness surrounding clinical trials.

SOURCES:

HTTPS://WWW.CENTERWATCH.COM/IMAGES/INFOGRAPHICS/UNDERSTANDING-CLINICAL-TRIALS-INFOGRAPHIC.PDF  
 HTTPS://WWW.NCCN.ORG/PATIENTS/RESOURCES/CLINICAL\_TRIALS/PHASES.ASPX  
 HTTPS://WWW.CANCER.ORG/TREATMENT/TREATMENTS-AND-SIDE-EFFECTS/CLINICAL-TRIALS/WHAT-YOU-NEED-TO-KNOW/PHASES-OF-CLINICAL-TRIALS.HTML  
 HTTPS://WWW.NCBI.NLM.NIH.GOV/PMC/ARTICLES/PMC3061013/  
 HTTPS://WWW.PWC.COM/US/EN/HEALTH-INDUSTRIES/HEALTH-RESEARCH-INSTITUTE/PDF/PWC-HRI-CLINICAL-TRIALS-IN-THE-NEW-HEALTH-ECONOMY.PDF  
 HTTPS://AACT.CTTI-CLINICALTRIALS.ORG/DOWNLOAD