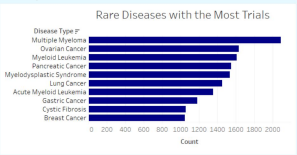
RARE DISEASE TRIALS

FINDING BETTER TREATMENTS FOR PATIENTS

SUMMARY

Our goal was to find what makes rare disease clinical trials successful. We defined trial success as a trial that reached phase four. We completed a series of analyses to see what could impact trial success in order to bring more treatments to market. We found that the following factors were statistically significant in determining whether a trial would reach phase four: age group, study type, intervention type, and funding type.

Top 10 Rare Diseases with the Most Trials



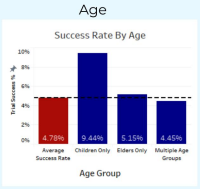
MULTIPLE MYELOMA

This is the disease that underwent the most testing with 2,091 clinical trials.

PREDICTORS

We found that the following factors were statistically significant for the success of a clinical trial

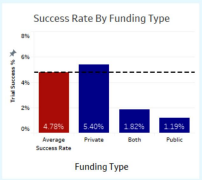
Age



97.49%

Trials involving children only, nearly double trial success rate

Funding Type



12.97%

Trials involving only private funding have a 12.97% higher chance to succeed over the average trial

6.79%

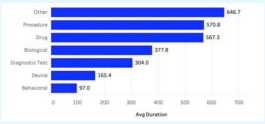
Looking at intervention types , drug studies had the highest success rate at 6.79% whereas genetic and radiation interventions are the lowest both under 1%

0%

Observational Trials had a 0% success rate most likely due to their lack of interventional method

Decreasing the Duration of Trials

This graph highlights average duration the clinical trials took to be completed by intervention type. We found the shortest average trial duration was behavioral with 97 days. This is because behavioral studies do not require large amounts of interventions. They are less invasive in that they just monitor the behavior of the patients. The other intervention types such as device, diagnostic test, biological, drug, and procedure require more invasive methods which extends the duration..



Increasing Chances of Success

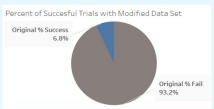
Modified Data Set

In building the model, we used a modified data set removing observational studies because they have a 0% success rate and, incomplete studies.

Regression Model

We calculated a binomial regression model that can calculate which trials are more likely to succeed.

$$x = 1.12\beta_{\text{Private Funding}} - 489\beta_{\text{Public Funding}} + 768\beta_{\text{Child Only Studies}} + 128\beta_{\text{Elder Only Studies}} - 469\beta_{\text{Procedure Interventions}} - 99\beta_{\text{Behavioral Interventions}} - 319\beta_{\text{Biological Interventions}} + 128\beta_{\text{Drug Interventions}} - 1.33\beta_{\text{Genetic Interventions}} - 69\beta_{\text{Other Interventions}} - 1.98\beta_{\text{Radiation Interventions}}$$



If we only run the top 5% of trials from the model, we would nearly triple your success rate from 6.8% to 15.2%

RUN MORE SUCCESSFUL TRIALS TO SAVE MORE

Using the model, if we remove the bottom 5% of trials, success rate increases by half a percent which would decrease the cost per average trial by $21,737, from $456,556 to $434,819. This will allow us to have more money to fund trials with a higher likelihood of success, in order to find better treatments for patients

$21,737

Savings per Successful Trial

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