How should we measure rare disease trial success?

- When considering rare disease trials, it is important to take into account the proportion of patients in the trial, as well as the proportion of patients in all genders. This is because rare diseases are often more common in certain populations, and it is important to ensure that the trial results are representative of the general population.

- We used median as our measure of efficacy in a study's ability to prove orphan designation. This is because median is a robust measure that is not affected by outliers.

- Subsequent indications are an important consideration when evaluating the success of a rare disease trial. They can provide additional information about the drug's safety and efficacy in different patient populations.

- Scientific success is also an important consideration. It is important to ensure that the trial results are consistent with the scientific literature and are not due to chance.

- Clinical trial design is another important factor to consider. It is important to ensure that the trial design is appropriate for the disease being studied and that the results can be generalized to the general population.

- Funding mechanisms are also important to consider. It is important to ensure that the funding is adequate to support the trial and that the funding is consistent with the scientific and monetary success of the trial.