Sample Size Calculation and Evaluation of Progression Criteria in Pilot and Feasibility Studies: A New App (SS-PROGRESS)

Kieran Bromley, Keele University (UK)

Keele UNIVERSITY

Background

In clinical research there is often uncertainty about whether a study design is feasible.

Pilot and feasibility studies are designed to provide early indicators of potential success of a main trial.

Progression criteria are used to decide whether to continue the study, implement remedial actions, or stop.

There is no consensus on estimating a suitable sample size for these studies.

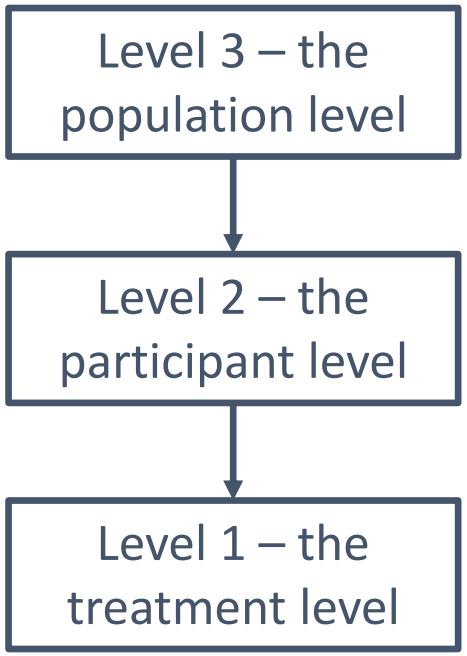
The SS-PROGRESS study

Lewis et al. (2021) proposed a hypothesis testing framework to determine thresholds for progression criteria, based upon a traffic light system.

Using a one-sided hypothesis test:

- Null hypothesis: the red limit is not greater than ρ,
- Alternative hypothesis: the red value is greater than ρ.

Progression criteria considers feasibility outcomes at different hierarchical levels.



Aims of the project

To produce a simple and freely available application implementing the previous methodology.

To allow researchers to easily determine sample sizes for their study, and to evaluate their outcomes.

Example study

Feasibility trial for oral protein energy supplements as flavoured drinks to improve nutritional status in children with cystic fibrosis

Progression criteria:

1. Recruitment uptake: at least 35% of children screened should be eligible (20% is unacceptable)
2. Follow-up: 85% or more children should be retained in the study (65% is unacceptable)
3. Treatment fidelity: 75% or more children should be given the correct treatment plan (50% is unacceptable)

Sample sizes are calculated for each outcome:

The total sample size needs to be extrapolated through the levels of the study.

Output and application

SS-PROGRESS | Welcome Page | Instructions | Sample Size Calculator | Evaluation | Examples | Contact Information | FAQs, Events, Publications | References

Test	Alpha (α)	Beta (β)	Allocation ratio	Expected recruitment
Normal Approximation	0.05	0.1	1:1	0.35

Level	Objective	R_{UL}	G_{LL}	SS	SS $_{tot}$	Power (SS_{tot})
Level 3 - Population	Recruitment uptake	0.20	0.35	79	200	1
Level 2 - Participants	Follow-up	0.65	0.85	44	70	0.99
Level 1 - Intervention	Treatment fidelity	0.50	0.75	35	35	0.91
□						
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Recommendations

The required sample size at the population level will be **200**.
The required sample size at the randomised level will be **70**.
The required sample size at the intervention level will be **35**.

Bookmark...

Output and application

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Objective	R_{UL}	G_{LL}	Numerator	Denominator	Current proportion
Recruitment uptake	0.20	0.40	70	167	0.42
Follow-up	0.65	0.85	57	70	0.81
Treatment fidelity	0.50	0.75	24	35	0.69

Enter alpha value here:

0.05

Additional plot options:

- [x] Display the critical value for rejecting the null hypothesis, for each objective (marked by a red cross)
- [ ] Display lower confidence interval (CI) for each objective

Select the distribution for the CI:

- (•) Normal approximation
- ( ) Binomial exact

Enter a custom title for your plot here:

Progression Criteria Evaluation

Here you can adjust the plot height:

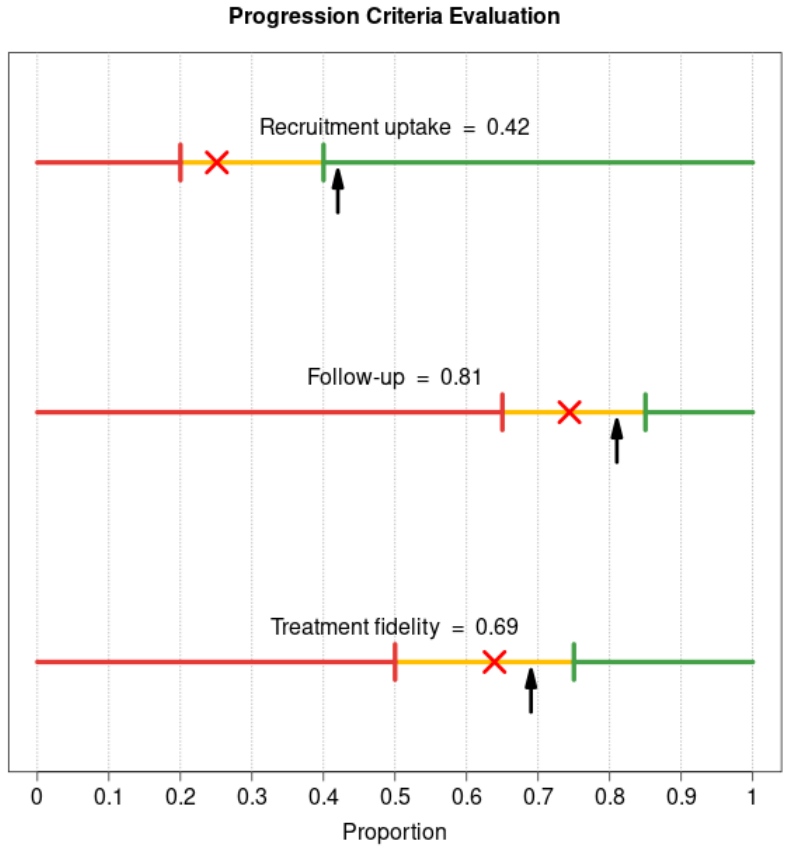
700

Here you can adjust the plot width:

700

Gridlines on plot:

- [x] Add dotted vertical lines to the plot



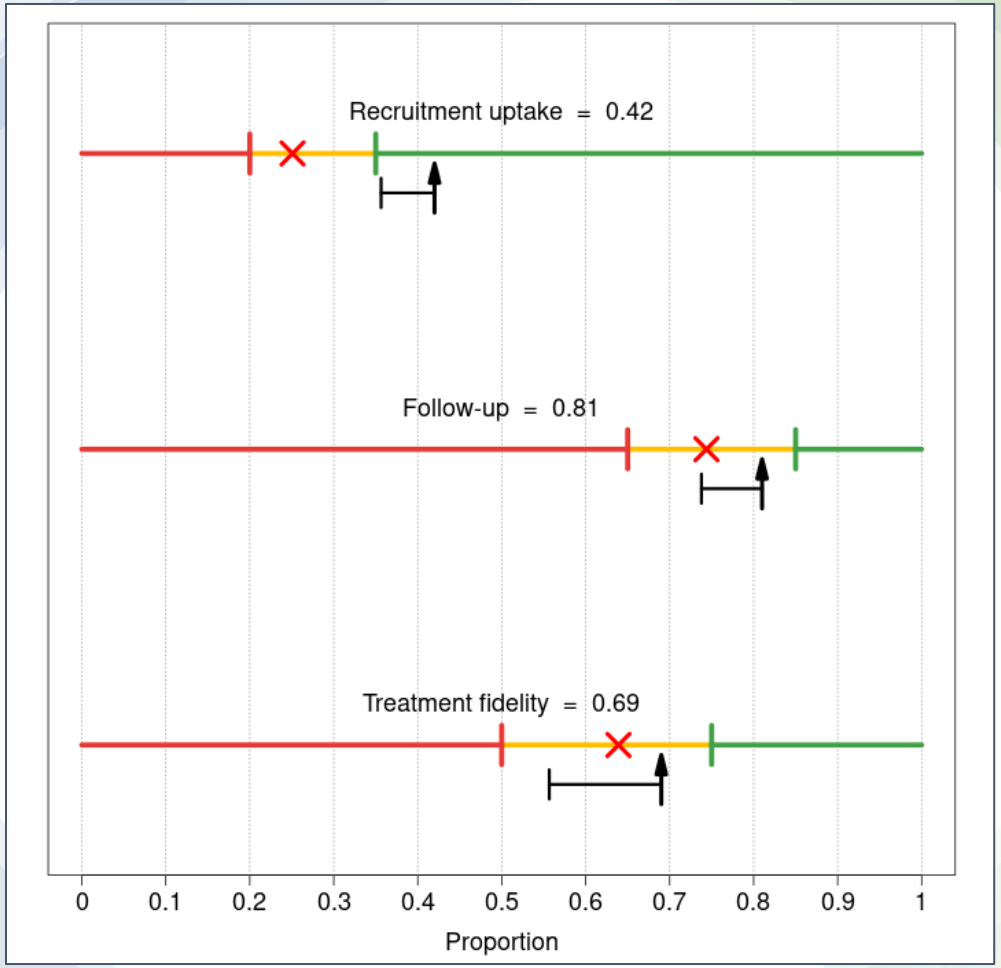
Study recommendations from the app

Recruitment uptake was better than expected (green).

Follow-up and Treatment fidelity need some minor work to be improved (amber).

All objectives sit above the critical value and provide statistically significant evidence that these outcomes are achievable.

Recommend progression to a main trial given minor amendments.



Conclusion

The application allows the newly proposed methodology to be easily implemented.

It facilitates improved design, monitoring, and evaluation of pilot and feasibility studies.

Promoting better research helps to better inform stakeholders regarding future progression of the study.

Thank you for listening!