

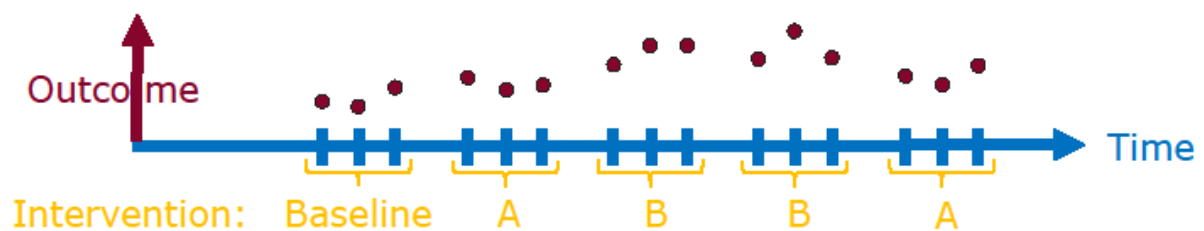
User-centered just-at-the-right-time visualization of results in personalized trials

What is the motivation for personalized trials?

What can you do if you want to know which physical exercise (and in which intensity and duration) helps you best to decrease your back pain? Or if you want to know which dose of drug xyz helps you best to control your high blood pressure? You could look at medical research studies that have investigated this on the population level. But they typically do not tell you whether it will work for you, they will tell you at best that a drug works in maybe 60% of the population. You could just try out whether a particular exercise helps you or which dose is best? Sure! And if you want to do this systematically to avoid biases, you can perform an experiment called N-of-1 trial to evaluate it scientifically with valid statistical inference.

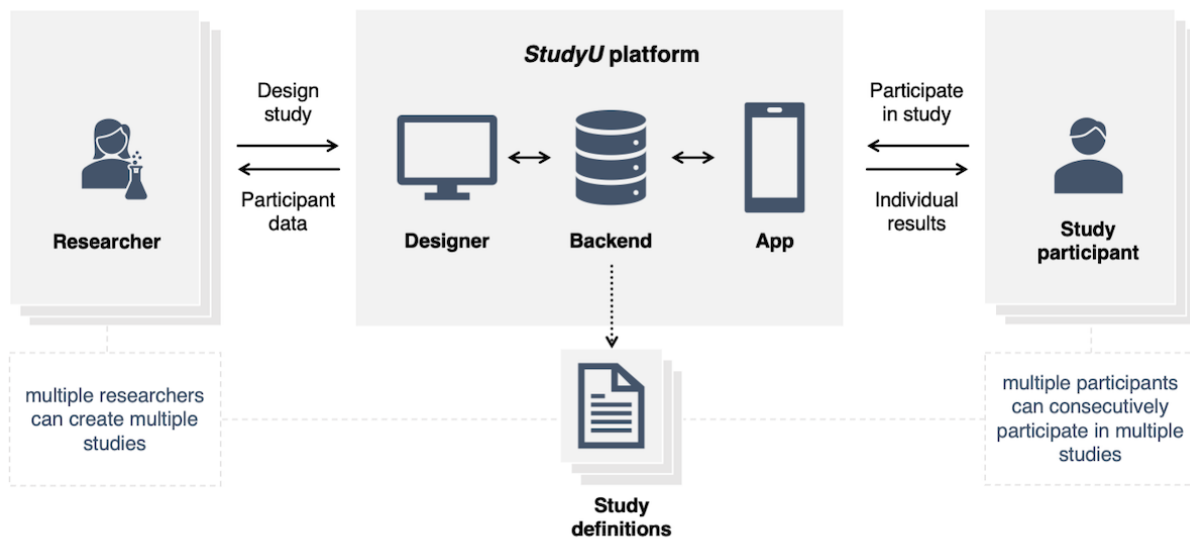
What are N-of-1 trials?

More formally, N-of-1 trials are cross-over randomized controlled trials where one or more health interventions are applied sequentially in one person over time. This is illustrated below, where two interventions A and B are administered in blocks, and the outcome is measured at all time points. If multiple people perform such N-of-1 trials, you can make efficient statistical inference what might help others in the population.



What have we already developed?

In recent work, we have developed the StudyU platform (<https://www.doi.org/10.2196/35884>), which consists of a study designer web app (<https://designer.studyu.health/>) that allows researchers to design and implement N-of-1 trials, and a study app that allows study participants and patients to take part in these studies (<https://app.studyu.health/>). StudyU is open source (<https://github.com/HIALab/studyu>) and free and the participant app is available in Android and Apple app stores. We have the vision to build a free resource that contains a repository of anonymized data of personalized trials and can empower users in their health behavior. The current tech stack of StudyU is Flutter/Dart, PostgreSQL/Supabase, Git/Gitlab, Jupyter Notebooks and there is ongoing work on its design. Clinical studies are ongoing or planned with UKE Hamburg, Mount Sinai, Weill Cornell Medicine, and University of Queensland.



Overview of your Master project

As N-of-1 trials are personalized, their results are of interest to the study participant. The data can be analyzed automatically and the results shown in the StudyU app to the patient. But

- When should the results be shown?
- Which results should be shown?
- How should the results be visualized and described?

These are the leading questions you will tackle in the Master project. There are still many open questions around these questions, which you will investigate:

For example, if you have results from a Bayesian linear model (a standard model used in N-of-1 trials), how do you display the posterior distribution of the treatment effect estimate and explain it to a patient?

And at what time should you display the results? In order to increase patient adherence, maybe it is best to show intermediate results already during the trial. But will this create biases that might affect subsequent data entry of the patients? In other trial designs (micro-randomized trials), results feedback is explicitly incorporated as part of an intervention. In which situations might this create bias? In which situations might displaying results just be the right thing to do? And how can you guarantee valid statistical inference for such intermediate peeking at results?

Details of your Master project

This master project will contain 2 main work packages, and combine aspects of statistics/causal inference/machine learning, app development, user-centered design and empirical user studies:

(1) Visualization of results in N-of-1 trials

You will research and design good visualizations of results of N-of-1 trials and implement them in StudyU. They can be based on descriptive statistics, standard linear regression or standard Bayesian linear models. Part of this work package will be in collaboration with clinical partners at Weill Cornell Medicine, who have developed a patient-centered visualization for N-of-1 trials. You can iterate your visualization designs and also evaluate them in user studies.

(2) Display interim results of N-of-1 trials

In a second working package, you will investigate the display of interim results of the data collected up to that date to the patient. Options will be to display descriptive statistics or visualizations developed in work package 1. Also, recent methods for anytime valid inference can be implemented (Malenica et al., 2023). A major part of this work package can be the investigation of biases when showing intermediate results. You can develop conceptual frameworks and also investigate it empirically by designing and running a multi-center study that investigates potential biases of showing results. The work on statistical methodology and of setting up the multi-center study will be co-supervised by collaborators at Harvard, Cornell, and UW-Madison.

What you should bring with you

The project is open to students from all programs at HPI. To carry out this project successfully, you will need expertise in at least one of the following areas to contribute to the team:

1. App development (front end, back end)
2. Programming skills (e.g. mobile app development, web development, Python/R)
3. Interest in learning about study designs and the evaluation of interventions for personalized medicine
4. Machine learning/statistics/causal inference
5. Data visualization
6. User-centric design, user studies

Further references

1. Kravitz RL, Duan N (eds), and the DEcIDE Methods Center N-of-1 Guidance Panel (2014). *Design and implementation of N-of-1 trials: a user's guide*. AHRQ Publication No. 13(14)-EHC122-EF. Rockville, MD: Agency for Healthcare Research and Quality.
2. Nikles J, Mitchell G (eds) (2015). *The essential guide to N-of-1 trials in health*. Dordrecht: Springer.
3. Malenica I, Guo Y, Gan K, Konigorski S (2023). Anytime-valid inference in N-of-1 trials. *Proceedings of Machine Learning Research* 225:307–322. <https://proceedings.mlr.press/v225/malenica23a.html>
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Contact

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