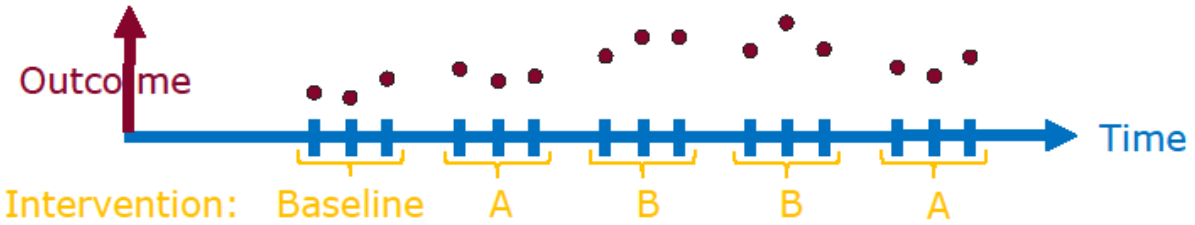
Research group: Personalized Medicine in the Digital Health Center
Head: Prof. Dr. Erwin Böttinger
Master project in summer semester 2020

App-based N-of-1 trials in a clinically-relevant context for personalizing digital health interventions

Background

Digital health tools like apps and mobile sensor devices enable individuals to track and quantify their own health. The goal of this project is to empower individuals to perform app-guided health interventions with the goal of improving their health status, for example to reach a fitness goal or to manage chronic back pain, while longitudinally quantifying putative health benefits in the app.

This setup represents so-called N-of-1 trials, meaning that the sample size of this form of interventional trial is 1, i.e., the individual him or herself. Statistically speaking, N-of-1 trials are multi-crossover randomized controlled trials in a single participant, i.e. where participants use the different interventions of the study in a pre-specified order:



This allows investigating which intervention works better for each single participant of the study and deriving personalized medicine approaches in a clinical context. Also, N-of-1 trials of single participants can be aggregated to obtain population-level intervention estimates [1–4].

Motivation

N-of-1 trials are most powerful when they can integrate sensor data from wearables and be linked to electronic health records in a clinical context. This can be done seamlessly by developing mobile apps for digital N-of-1 trials. Moreover, such an app helps to empower citizens through directly feeding back the study results.

There exist current apps for N-of-1 trials (e.g. N1, Trialist) but among others they are limited in their functionality and ability to integrate domain knowledge, limited in their visualization and underlying statistical analysis models, only implemented for iOS, not open source, and cannot readily integrate sensor data and be linked to electronic health record data. In their

current state, they do not allow to unlock the full potential of app-based N-of-1 trials and their application in a clinical context.

Description

In the main work package of this Master project, we will design and build an app with an appropriate user-friendly interface to address the above challenges. In additional work packages, the public launch of this app will be prepared by planning and performing pilot studies for two N-of-1 trials within the Digital Health Cohort at the Hasso Plattner Institute for Digital Health at Mount Sinai (HPI-MS) in New York.

Work packages:

(1) App design and development

a) App design and functionalities: app architecture, general questions in app set-up and study onboarding, UI, visualization, data storage and security, interface to wearables, results visualization and communication
b) Build the front and back end of the app
c) What is a good visualization and communication of the results to participants?
d) How can you implement an automated appropriate N-of-1 study design dependent on the specific interventions and outcome?
e) What statistical models are appropriate to analyze data, e.g. from sensors?

(2) Use case I: Polygenic risk scores & visualization

Polygenic risk scores (PRS) accumulate genetic variant information into a single measure that represents the genetic risk for specific diseases or traits, such as psychiatric disorders [5] and cardiovascular disease [6]. By presenting PRS to citizens, possibly in combination with other clinical data, they can be empowered to make informed healthcare and lifestyle decisions [7]. To fully leverage this opportunity, citizens need appropriate and understandable visualizations and descriptions, which will be developed in this use case.

a) How do you visualize and communicate PRS?
b) Design, plan, and pilot an evaluation of the communication of PRS in N-of-1 trial in the Digital Health Cohort at Mount Sinai using the app from work package (1): interventions are (i) conventional risk score, (ii) PRS score, (iii) both; outcomes are patients' reaction/behavior change

(3) Use case II: Measuring pain

Pain is described as unpleasant sensation and one of the major reasons to seek medical advice. It is basically categorized into "acute" and "chronic" pain pointing to the period of time when pain is subjectively perceived [8]. Especially chronic musculoskeletal pain is a challenging problem for the healthcare system as it is highly prevalent and costly. Pain measurements are commonly highly subjective, self reported and focus on different aspects such as pain intensity, quality or localization. However, methods to

objectively assess pain are scarce due to its subjective perceived nature. Automated pain recognition [9] or sensor-based technologies could be possible solutions of measuring pain in an objective manner. Therefore this use case aims to evaluate if objective approaches (e.g. wearables) could reliably assess pain.

a) Design and perform a pilot study to evaluate sensors to assess pain, for example back pain
b) Design, plan and pilot an N-of-1 trial on pain using the app from work package (1) in the Digital Health Cohort at Mount Sinai, in Smart4Health, or the HPI.

A successful development of the app can have the following impact and potential:

- Application of the app in Digital Health Cohort and integration into the Mount Sinai Health System in New York
- Follow-up of the app as a new medical product in Germany through health insurances and world-wide

What you should bring with you

To carry out this project successfully, the team will need expertise in:

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. Fundamentals of machine learning or statistics
5. Data visualization

References

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