Statistical Power and Sample Size in Personalized Medicine

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ABSTRACT

Personalized medicine (PM) is a rapidly growing field of healthcare and medicine. The advantage of a personalized medicine is the availability of each person’s unique genetic and genomic print. The healthcare that incorporates personalized medicine provides coordinated, continuous patient-specific data. The goal of personalized medicine is to promote health wellness, satisfaction, and to increase the likelihood of a successful disease prevention, detection and treatment. This form of medicine, apart from patient’s personal data and medicine-biological measurements, uses genomic information data to understand the molecular structure of the disease and to optimize health care strategies and drug therapies. Clinical trials that investigate personalized approaches are subject to special rules, for example, pertain the selection of participating patients. In personalized medicine, a certain genetic profile must be identified so that the treatment can work. This is why potential participants are first screened and selected accordingly for clinical trials. The special design of such clinical trials has an impact on the evaluation of data collected during the given study.

Keywords: Cancer, Clinical Trials, Personalized Medicine (PM), Pharmacogenetics, Phase III, Targeted Therapy

INTRODUCTION

Traditionally drug discovery and development attempts to find medicines that show benefit across an entire disease population. Personalized Medicine however, attempts to define molecular predictors of drug safety and efficacy up front and to stratify patients and diseases into subsets based on their unique characteristics. This approach allows for an optimal response to therapy by enabling physicians to prescribe therapies more likely to benefit patients and minimizes patient exposure to drugs that are
not likely to provide a benefit. The aim of personalized medicine is to treat select patient groups with medicines that match the genetic profile of their cells. For example, special agents have been developed for lung cancer patients whose tumor cells exhibit specific genetic profiles. They disable certain gene mutations or gene rearrangements, thereby inhibiting the proliferation of cancer cells. Molecular biological profiling is part of the diagnostic process. Performed before start of a treatment regimen, this type of screening determines if a medicine will achieve the desired effect in a given patient. There are various synonyms for personalized medicine such as tailored, stratified, precision and individualized medicine (Veer & Bernards, 2008; Willard, 2012). They often mean the same thing—the targeted application of drugs tailored to the genetic traits of certain diseases. In this context, personalized medicine does not mean neither the doctor-patient relationship, nor the personal attention and care that are so important, too. Personalized medicine has the potential for great benefit because it enables targeted treatments with fewer side effects. These therapies are tailored to the needs of certain patient groups or populations and therefore not all patients can benefit, so it does give rise to several ethical questions (Haile, 2008; Shastry, 2006). These concern access to medical services, the handling of personal patient data, and the compatibility of personalized research and treatments with the principle of solidarity in healthcare. The design of clinical studies in personalized medicine is also changing for ethical reasons. In many cases, patients receiving standard or placebo treatments within the framework of the study will soon cross over to the personalized and therefore far more effective agent (Sadée & Dai, 2005).

Personalized medicine often involves developing genetic or other tests that can be used to determine which patients are most likely to benefit from a drug or which are most likely to suffer serious side effects (Wong, 2008). Relatively few drugs are now accompanied by such so-called companion diagnostic tests. They are most common in oncology. The breast cancer drug Herceptin, for instance, is effective only to women whose tumors have an abundance of a protein called Her2 (Lazakidou & Daskalaki, 2012).

**CANCER MANAGEMENT**

Oncology is a primary section of medicine consisting of various cancer phases, measured in centimeters in most cases, and types with regard to their anatomy and pathology. Cancer genetics is a subgroup falling under the category of oncology that is focusing on genes and is associated with inherited cancer risk (Mansour & Schwarz, 2008). There is a limited number of cancerous disorders in which homogeneity separates in an autosomal dominant fashion, leading to considerably higher risk for certain cancers types. It is considered that inherited cancer genetics factors explain only about 5-10% of all cancers cases. Nevertheless, other genetic modifications with more indirect effects associated to cancer risk may trigger detailed cancer risk valuation to patients who are not associated with a family history (Yan, 2008).

Examples of personalized cancer management:

1. Mutations in the **BRCA1** and **BRCA2** genes, associated with inherited breast-ovarian cancer disorders. Findings of a disease leading to genes modifications to a controlled group of individuals could provide information as to whether they potentially hold a higher probability for cancer and may prompt personalized prophylactic therapy such as mastectomy as well as removal of the ovaries. Such testing incorporates complex personal decisions and is commenced in the context of in depth genetic treatment.

2. Minimal residual disease (MRD) tests are used to compute residual cancer, permitting detection of tumor markers prior to any occurrence of physical signs or symptoms. This permits doctors in taking clinical decisions earlier than before.
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