Abstract for Nordic Health Economics Study Group

**Orphan Drugs: Does Society Value Rarity?**

**Background**

Public policy has successfully encouraged development of drugs to treat orphan diseases (prevalence < 7 per 10,000), but because such drugs are usually extremely expensive they often exceed threshold requirements in cost-effectiveness analyses. Arguments in favor of granting special funding status to orphan drugs generally rely on characteristics that are equally applicable to more common diseases, e.g. severity of condition, lack of alternative treatments, etc. Whether national health care systems should make exceptions for reimbursement of orphan drugs ultimately depends on whether society values rarity per se. The question has become increasingly important because advances in the field of genetics, in addition to providing treatment options for known orphan diseases, have also led to the realization that certain common diseases, primarily cancer, can be subdivided into many “orphan” categories, each with its own tailored drug treatment.

**Methods**

We will survey a random sample of the Norwegian population (survey to be conducted via an internet panel by TNS Gallup Norway in May) as well as a sample of informed individuals, e.g. health economists, doctors, policy makers, to determine if a preference for prioritizing treatment of rare diseases over common ones exists. The survey employs person trade off (PTO) exercises between treatment of a rare versus a common disease under two different scenarios: (1) identical per person costs and (2) higher costs for the rare disease. The diseases are described identically with the exception of incidence. Respondents are randomized to different amounts of information provided about severity of the diseases (severe vs. moderate) and expected benefits of treatment (high vs. low). Respondents have the opportunity to provide information about factors that most influence their decisions. A pilot study improved the design of the final survey.