Action Line 2: *Outcome-Based Health Care* - Summary

1) Background and vision

Healthcare systems are under pressure and face important challenges. In a context of rapidly expanding diagnostic and treatment options and increasing concern about the unsustainability of health spending growth, policymakers have been looking for methods and approaches to increase value for money, that is, realizing the best possible outcomes for patients, delivered in a patient-centered way at the lowest possible costs. To achieve this, policymakers aim to reduce waste, unwarranted variation in patient outcomes and fragmentation in care delivery. Two specific focus areas that are crucial to develop adequate policies are (1) gaining insight in patient outcomes and using this information to improve quality of care, and (2) developing and evaluating reform of the incentives embedded in the methods used to pay for health care, which currently tend to reward volume instead of value of care.

In practice, 'best possible outcomes' means a maximum score on a balanced set of medical and patient-reported outcome measures, which are developed in dialogue with patients. These measures can be used 'internally' by healthcare providers to assess and improve care for individual patients, and in aggregated form to track performance over time and to identify areas for improvement within providers' own patient populations. In addition, aggregated outcome information can also be used 'externally' for benchmarking purposes and it can be incorporated in alternative provider payment models. Together, these efforts are expected to lead to a continuous learning environment.

Despite many efforts, insight in outcomes and their link with quality and costs of care remains limited for many diseases. In addition, knowledge is lacking on appropriate methods for quantifying and reporting outcomes and variation therein between providers. Moreover, the 'actionability' of aggregated outcome measures for providers to improve quality of care is being questioned. Furthermore, little is known about how outcome information can best be incorporated in alternative payment models, and what implementation of these models can achieve. The ambition of this action line is to fill these knowledge gaps and thereby facilitate the transition the transition towards better care with improved outcomes at lower costs

2) Our objective

This action line aims to contribute to providing the scientific basis for the drive towards outcome-based health care. Specifically, it addresses the following two main objectives:

1. Development of methods for quantifying (between-provider differences in) outcomes, and assessment of the reliability (e.g. the role of random variation) and validity (e.g. the role of case-mix) of the resulting measures as indicators of quality of care.
2. Development and testing of feasible methods for steering on aggregated outcomes (both internally and externally) and costs – incl. benchmarking and alternative payment models (e.g. bundled payment, pay-for-performance) – and assessing their impact in terms of improvement in care processes, redesign of workflows, multidisciplinary collaboration, and quality and costs of care.

3) Our impact

We build on the work started and the insights obtained during SCBH 1.0. In doing so, this action line will yield several key insights that are of considerable scientific and societal interest. First, it will result in improved insight in the association between (variation in) medical and patient-reported outcomes on the one hand, and variation in quality of care on the other hand. Second, it will result in improved insight in the appropriate design of methods for steering on outcome and costs, as well as in their effects. We believe these insights will contribute to better approaches to realizing improved patient care at lower costs.

Part of this action line will be two PhD-projects, which are described in more detail below. For both projects, PhD-researchers have already been recruited. These and other projects under this action line (will) involve close collaboration with several internal and external partners, including clinical departments within Erasmus MC, Netherlands Comprehensive Cancer Organisation (IKNL), the Dutch Institute for Clinical Auditing (DICA), Menzis health insurance company, National Institute for Public Health and the Environment (RIVM), Dutch Health Care Authority (NZa), and LUMC-Campus The Hague. In addition to strengthening societal impact, these collaborations come with access to great data and opportunities to analyze and evaluate ongoing initiatives with steering on outcomes and costs. A clear example of this are the alternative payment approaches implemented by Menzis (see PhD-project 2 below).

Our results will be presented at (inter)national conferences and will be published in professional and scientific journals and two PhD theses. In addition, we will actively contribute to the implementation of our findings in Erasmus MC and other providers in the Rotterdam region, as well as elsewhere. Also, we will incorporate our work and findings in our education (including relevant courses taught at ESHPM and Erasmus MC), and use social media and our (inter)national network to disseminate our findings as much as possible.

4) Methodology

In order to achieve our objectives, we will conduct a range of studies:

1. Analysis of large databases using comparative effectiveness research methods. Using information on outcomes for steering medical practice and policy presupposes the validity and reliability of underlying measurements and resulting measures as indicators of quality, including sensitivity of these measures for improvements in quality of care. We will therefore conduct studies analyzing the association between clinical and patient-reported outcomes on the one hand, and variations in quality of care (measured by evidence-based process indicators derived from clinical practice guidelines) on the other hand, while accounting for random variation, missing values, and differences in case-mix. In addition, we will apply novel methods (including the regression discontinuity design and instrumental variable analysis) that allow for causal inference regarding the relation between treatment and outcome in observational data. We will collaborate with other action lines (e.g. Prevention, Equity) in order to optimally use the knowledge on these quasi-experimental methods within SCBH.
   For studies on analyzing and learning from outcome data internally, we will use routinely collected outcome data from Erasmus MC for a variety of diseases. For studies on using outcome data in an external setting (benchmarking) we will use data from clinical registries, including data from the Netherlands Comprehensive Cancer Organization (INKL) and clinical registries from the Dutch Institute for Clinical Auditing (DICA)). If possible, we will also conduct primary data collection.
2. Design and testing of methods for steering on outcomes. In this action line, the emphasis will lie on two methods: (a) novel benchmarking and ranking approaches using aggregated outcomes to steer on quality, including assessment of the conditions under which steering on outcomes can be actionable; and (b) alternative payment models using aggregated outcome information. Based on literature and previous experiences in the Netherlands, for both approaches we will develop several modalities which will be tested on their feasibility in various (hospital) settings.
3. Conduct several (small-scale) experiments with steering on aggregated outcomes (and possibly costs), using the insights obtained from the previous studies. The main question to be answered is whether the analyzed approach leads to actionable information and indeed improves care. Erasmus MC will provide the setting for some of these experiments, but ideally other providers in the region will also be involved. In addition, in collaboration with external partners we will evaluate ongoing payment reform experiments on their effects on quality, outcomes and costs, including identification of the (contextual) factors that mediate and moderate these effects. The latter will be done using qualitative research methods and, if possible given available data, formal mediation and moderation analysis techniques. Again, we will collaborate with other action lines (e.g. Prevention) to benefit from the available knowledge there.

5) Core team

Action Line Leaders: Nikki van Leeuwen (Erasmus MC), Frank Eijkenaar (ESHPM)
Postdocs/Assistant professors: Daniëlle Cattel (ESHPM), Raf van Gestel (ESE/ESHPM)
PhD students: Margrietha van der Linde (Erasmus MC), Tadjo Gigengack (ESHPM)
Affiliated steering group members: Erik Schut (ESHPM)
Affiliated MT members: Hester Lingsma (Erasmus MC)
Other affiliates: Arthur Hayen (LUMC/Menzis)

6) PhD project 1. Identifying and exploiting meaningful variation in outcome to improve patient care

- Start: October 2021
- PhD researcher: Margrietha van der Linde
- Supervisory team: Hester Lingsma (promotor), Nikki van Leeuwen (copromotor), Frank Eijkenaar (copromotor), Raf van Gestel, Daniëlle Cattel

Over the past decade, measuring outcomes with the aim to have more insight and to improve quality of care, has increased in popularity. However, evidence is lacking that outcome measures are valid indicators of quality of care. Specific challenges complicating the discrimination between health care providers on outcome include for example large amounts of missing baseline characteristics and outcome data in clinical registries, and high degrees of statistical uncertainty.

Aggregated outcome information can be used both internally (insight in health outcomes of own population) and externally (benchmarking care providers on health outcomes). It remains unclear if and how aggregated health outcomes are actionable for healthcare professionals, and the extent to which they facilitate the improvement of patient outcomes and quality of care.

This PhD-project aims to provide the scientific basis for the drive towards outcome-based health care, by addressing the following specific objectives:

(1) Development of valid and reliable methods to discriminate between health care providers, and the assessment of the validity of outcome measures as indicators of quality of care;
(2) Evaluation of the feasibility and effectiveness of steering on aggregated outcomes (internal and external/benchmarking) to drive improvement in care processes and outcomes of care;
(3) Exploiting (between-provider) variation in outcomes to evaluate treatment effectiveness using novel methods in large observational datasets.

Possible studies to address these objectives include assessing and comparing different ranking methods to appropriately discriminate between health care providers, providing an overview of the current scientific evidence on the effectiveness of collecting and benchmarking outcome measures to improve quality of care, conducting small-scale experiments in a clinical setting evaluating the use of aggregated health outcomes to steer on quality of care as well as assess under which conditions steering on outcomes might be actionable, and selecting and applying novel methods for causal inference to evaluate treatment effectiveness in clinical registries.

The project will be performed in close collaboration with clinical groups, including clinical departments and research groups in- and outside the Erasmus MC.

7) PhD project 2. Design and evaluation of outcome-based payment models in the Dutch healthcare system

- Start: September 2021
- PhD researcher: Tadjo Gigengack

- Supervisory team: Erik Schut (promotor), Frank Eijkenaar, Nikki van Leeuwen (copromotor), Daniëlle Cattel (copromotor), Arthur Hayen (copromotor), Raf van Gestel

Over the past decade, healthcare purchasers and policymakers have increasingly been exploring alternative provider payment models (APMs) to help steering healthcare systems towards more value in terms of lower spending and better patient outcomes. Examples include explicit financial rewards for measured performance, prospective payments at the level of medical conditions or populations, shared-savings/risk models, and all kinds of combinations. Despite broad consensus about the potential and need for payment reform, evidence for positive effects is limited and inconclusive. In addition to a lack of rigorous evaluation studies, this seems to be related to lacking insight in how APMs are ideally designed and implemented in a given context, and in the (contextual) factors that moderate and mediate their effects. These knowledge gaps hamper informed policy on incentive (re)design and outcome-based care.

When it comes to APMs little is still known about what works, why and under what conditions. This is especially the case in the Dutch healthcare system, where APMs have only recently gained momentum. By detailed evaluation of APM initiatives initiated by a large Dutch health insurer (Menzis), this PhD project seeks to contribute to broadening and deepening the APM evidence base by providing knowledge on effects (both intended and unintended), and underlying mechanisms and facilitating/inhibiting factors. More specifically, the objectives are to:

1. review key design and implementation issues with regard to (specific types of) APMs;
2. assess the (un)intended effects of existing APM initiatives in the Netherlands;
3. uncover the mediating and moderating factors that drive the (lack of) effects;
4. formulate lessons for APM initiatives in the Netherlands and possibly other countries.

In addition, the project may also entail examination of the design and development process of new APMs within Menzis and conducting data simulations for design purposes.

For the quantitative part, longitudinal individual-level data on insurance claims and socio-demographic and morbidity characteristics will be available from Menzis. These data can be used for evaluating APMs on their impact on spending, health outcomes and unintended behaviors, as well as for simulation studies. Among the APM initiatives to be studied are the “all-in tariff + shared-shavings model” for general practices[1] and the “value-based healthcare/payment trajectories” for providers of medical specialist care[2]. For the qualitative part, data will need to be collected from the stakeholders involved in the APMs under study. These data are needed for insight in the underlying mechanisms driving the (lack of) effects and relevant contextual factors.

1. https://www.artsenzorg.nl/actueel/unieke-afspraken-tussen-arts-en-zorg-en-menzis
2. https://www.menzis.nl/zorgaanbieders/zorgsoorten/medisch-specialistische-zorg/contractering/waardegericht-inkopen