Diagnostic testing in pediatrics: yield and drivers

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Chapter 1

Introduction

1 Rise in medical spending

Medical spending is steadily rising in the Netherlands and other OECD countries.[1-3] Drivers are higher expectations of what health systems should deliver in high-income countries with economic growth[4,5], lower productivity gains in the labour-intensive medical sector (Baumol effect), demographic change and lifestyle-related diseases. Biomedical technological advances that include drugs, medical devices and diagnostic modalities, constitute a key driver of spending.[6,7] New technologies often enter health care systems without prior evaluation of benefit. They do not necessarily lead to substitution of established test and treatments, and if newer technologies are associated with lower harms the indication for their use is frequently broadened.[8,9] Whereas some new technologies are successful and improve life expectancy or quality of life, other innovations prove to be less beneficial and are cost-inefficient.[2,3]

Over the past 40 years imaging facilities have expanded and the number of imaging exams increased in all OECD countries[10], including the Netherlands.[11] Similarly, technological advances in genetic testing resulted in increased utilization[12], and other diagnostic tests followed a steep upward trend with an annual growth of 7% between 2003–2006 in the US.[1,13]

The overall effect of increased testing on quality of health care is unknown. There are many examples where new technologies have increased the diagnostic quality such as in non-invasive prenatal screening[14] or replaced a harmful procedure by a less invasive test (genetic testing versus kidney biopsy in some cases of hematuria[15]). But there are also reports of testing without benefit or more harm than benefit. Examples are screening for abdominal aortic aneurysm[16], screening for thyroid cancer[17] and detecting and treating weak risk factors for disease such as mildly increased glucose levels.[18]

There is large practice variation with regards to testing volume among and even within countries, and volume is not directly linked to higher quality of care. For example, in the United States spending for diagnostic testing and health care in general is almost twice as high as in the Netherlands, while life expectancy, self-rated health and accessibility of health care are lower in the former.[10] In general, in high-resource countries more health care spending leads to lower incremental benefit for health.[19]

Further expansion of the already heavily funded health care system has economic consequences such as shortage in health care personnel. Also, increased health care expenditure diverts resources from public domains with higher return on investment in relation to health and wellbeing.[2] These domains include primary prevention, education, social welfare or measures against further climate change and depletion of resources, which will pose a substantial threat to health and wellbeing in the long term.[20-23] Through the associated opportunity costs, the unlimited rise in health care spending can eventually even lead to a decrease in health. The described consequences of uncontrolled growth have long been recognized. Through various policy instruments governments have tried to control volume, price and quality of health care over the past 60 years.

Health care activity can also lead to a direct loss of health, because of unintended and often unnoticed negative downstream consequences of health care activity for patient health.

Initiatives have sprung up to eliminate undesired and harmful care, which if successful both reduce costs and increase quality. Examples of these initiatives are Value-based health care, Preventing overdiagnosis[24] or international campaigns such a Choosing Wisely, the BMJ's Too Much Medicine program, and JAMA Internal Medicine's Less is More series. These are aimed at creating impetus to reduce low-value care and increase high-value care, e.g. by creating awareness, issuing recommendations, evaluation of practice and the generation of evidence on the effects of health care. This resulted in decision rules and guidelines containing differentiated diagnostic recommendations that take prior risk into account and are more restrictive, such as in head CT for minor head injury.[25] Next to the generation of knowledge, it is important to understand what drives health care providers and patients, the principal agents in health care decisions. Considerable research effort has been dedicated to studying the effects and underlying reasons of overuse (reviewed in[26-30]). This knowledge can inform interventions to improve care, of which some have been demonstrated to be effective.[31] However, the trend for overuse has not been reversed, and there is certainly room for improvement of care.

The focus of this thesis is diagnostic testing, in pediatrics more specifically. Diagnostic testing has large indirect costs due to many test-related consultations and downstream health care activity, and an impressive growth potential, as observed in the United States. Furthermore, the reliance on tests both reflects and shapes the perception of health and high-quality care: both concepts will be increasingly associated with testing. This thesis explores how a structured evaluation of expected positive and negative consequences can help in the diagnostic decision making. And it focuses on why we test, not solely why we test more and more.

2 What is known: The value of diagnostic testing and its harms

2.1 Diagnosis and the value of diagnostic test information

Defining a patients' health problem, is a core tasks for physicians.[8] Important information is provided by the clinical history and interview of the patient, to form a picture of a patient's relevant history and current signs and symptoms. Hypothesis building starts here, and is followed by a physical exam, where refinement of the hypotheses takes place.

Diagnosis is an iterative process of collecting information, integration and interpretation of that information, generating hypotheses, and updating probabilities as more information is available. As the diagnostic process proceeds, a sometimes broad list of potential diagnoses may be narrowed into fewer potential options.[32]

The diagnostic process is characterized by inherent uncertainty regarding the true disease status and different potential diagnoses. Hence, clinical reasoning involves judgment under uncertainty.[33] The number of symptoms with which a patient may present is finite, while the pool of potential diseases underlying the symptoms is large. Of major importance is the element of time, because the evolution of symptoms provides important cues for the direction of the differential diagnosis.

In some instances, signs and symptoms are recognized as typical for one specific diagnosis, such that a tentative diagnosis is made early in the diagnostic process and additional information obtained by testing is not required. Often unacceptable diagnostic uncertainty persists, and diagnostic tests can yield information and reduce uncertainty sufficiently to enable therapeutic decision-making.[34,35] The ensuing improvement of the therapeutic decision has been described as the 'medical value' of diagnostic testing.[36] Some refer to 'clinical utility' or 'patient outcome efficacy' of diagnostic testing, terms that also encompass other beneficial clinical effects of diagnostic test information, such as lifestyle changes or improved adherence to treatment.[37,38] Thus, diagnostic testing indirectly affects outcome via change in (self) management. Diagnostic information can also generate value in other domains. First, it can yield prognostic information (i.e. planning value).[39] Second, it may help patients understand the cause of their symptoms or give them reassurance (i.e. psychosocial value).[36,40]

2.2 Harms of testing

Diagnostic testing also has negative consequences. These include physical harms or discomfort of the test itself, psychological harms from anxiety or labelling, individual opportunity costs, individual costs, societal (opportunity) costs and environmental impact.[41,42] Besides, no test is perfect. This results in the occurrence of false positive and false negative test results. In laboratory tests a cut-off of 2.5% is usually used to define the lower and upper limit of the reference interval.[43] Small deviations from the normal range are especially likely to reflect normal physiological variation and do not necessarily indicate disease. Multiple testing, as occurs often when blood tests are ordered, increases the risk of detecting this normal variation. If this is falsely interpreted as pathological, this can lead to further downstream testing without any benefit for the patient. In imaging this is a well-known phenomenon, as are ambiguous and incidental findings. The prior probability of disease importantly determines the proportion of false positive and false negative results among all positive and negative results. Especially in situations with low probability of relevant disease testing carries the risk of false positive or ambiguous test results.

3 Other reasons for diagnostic testing

Physicians and patient decide together whether testing is performed, with the physician as the ultimate decision maker. In the field of pediatrics they do so together with caregivers as proxy decision makers, if decisions concern (young) children. Society is also a stakeholder in test decisions, because health care is collectively financed, resources have limits, and decisions bear opportunity costs. Testing can have value to all stakeholders. We described value of diagnostic information from the patient's perspective. Physicians also benefit from diagnostic information e.g. from reassurance of negative results that reduce their fear of missing a diagnosis. Society benefits from high-value testing through increased health, labour productivity and tax contributions. Members of society can also directly derive benefit from testing individuals, e.g. if isolation measures are imposed on individuals that test positive for contagious infectious diseases.

Irrespective of the information testing yields, patients and physicians can derive value from the act of testing. This includes perceived control for the patient, meeting patient's expectations regarding health care, or a validation of their concern and a symbol of their physician's care.[44,45] Physicians report that testing can help in conveying that everything possible has been done to find the cause for symptoms.[46,47] Also they describe that conceding to requests is less time and energy consuming than explaining why testing is not warranted; testing is perceived to increase the efficiency and ease of the consultation.[48]

Physicians have other personal interests. Conceding to requests is believed to be associated with higher patient satisfaction, which is an important quality measure and increases their competitiveness.[49-51] Also physicians feel they reduce the risk of a negative personal outcome such as a formal complaint due to missed diagnosis.[52] Other motives are financial gain[53], securing their job[54] or conforming to local practice.[55]

4 Trade-off in the test decision

A physician delivering high-quality care is bound to serve the patient's interest (beneficence, non-maleficence, autonomy), while respecting the boundaries of efficiency and effectiveness, in order to also serve society's interests (distributive justice).[56] High-quality care encompasses multiple interconnected aspects, i.e. safety, patient-centeredness, effectiveness, efficiency, timeliness, and acting according to professional norms.[57] In test decisions, physicians need to weigh the anticipated positive and negative consequences of testing. As described in paragraph 2.1, positive consequences encompass expected medical value such as improving outcome through better management and non-medical value such as reassurance or planning value. The expected net value of testing results from the balance of the expected frequency and importance of positive and negative consequences of testing for patient, physician and society. Net value of testing is therefore a multifaceted outcome.

In some situations the decision to perform or refrain from testing is straightforward, e.g., if the positive consequences of testing unequivocally outweigh the negative consequences such as patient burden and (societal) costs. Sometimes, however, these decisions are complex. For example, in situations in which patients request tests despite a low probability of serious disease physicians need to weigh the potential value of reassurance for the patient and themselves against the consequence of false positive test results for the patient and the costs for society. In these instances, preferences regarding testing between society and patients may not agree, reflecting a conflict between efficiency and patient-centeredness. In summary, the interests of different stakeholders are sometimes conflicting. Testing occurs at the physicians' discretion, usually in the consulting room in concordance with patients. Because society is not a direct partner in diagnostic decisions, society's interests are not directly represented and receive less weight.

5 Aim

In this introduction we described that testing volume has increased, a part of which constitutes testing with limited or negative value. To identify low-value care, one has to estimate the information gain of testing and its downstream effects on outcome through management or behaviour. This requires a structured analysis, which needs to be repeated if new insights or developments alter (the magnitude of) the projected benefits and harms. Because test decisions depend on other factors than information gain alone, we need to understand what drives testing. This information can help in efforts to reduce low-value testing.[58] Research has mainly addressed these topics in adult medicine, but low-value testing certainly plays a role in pediatrics.[59] The research reported in this thesis therefore aims to generate knowledge in the field of pediatrics. Insights in the diagnostic process, the information yield of testing and test behaviour are necessary to advance the discussion on the desired diagnostic yield of testing and to find ways to improve the quality of testing.

6 Outline of this thesis

In this thesis we assessed the information diagnostic testing yields in two different studies. In Chapter 2 we assessed the yield of imaging in the example of unilateral hearing loss, and explored multiple dimensions from the patient's perspective. In Chapter 3 we reviewed the yield of PET/CT in children with suspected infection, an imaging modality that is increasingly used without prior assessment of its benefit for different indications. In Chapter 4 we analysed differences in test behaviour between physicians working at 5 European emergency departments, and underlying reasons for these differences. In Chapter 5 and 6 pediatricians' considerations when deciding on test ordering were explored, as well as their views on circumstances that influence their decisions. In Chapter 7 we provided the rationale for a regular re-evaluation of recommendations on screening and give suggestions for a transparent and unbiased process.

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