Proposal on effectiveness indicators

09-2017

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Background of the project

- Improving effectiveness is one of the central aims of the ongoing social and healthcare reform
- Information on the effectiveness of treatments is increasingly needed in future healthcare decision-making
- Pharma Industry Finland wants to participate in the societal discussion on treatment effectiveness by presenting its own model on how impact assessments could be made in the future
  - http://www.laaketeollisuus.fi/uutiset/laaketeollisuus-ry-kaynnistaa-tutkimuksen-laakehoidon-vaikuttavuudesta-terveydenhuollossa (in Finnish)
- The aim of the development project is to produce indicators for measuring effectiveness in healthcare
- Medaffcon conducts the project commissioned by Pharma Industry Finland
  - http://www.medaffcon.fi/en/vaikuttavuuden-arviointi-terveydenhuollossa/ (in English)
- The development project is funded by Pharmaceutical Industry Research Foundation

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The project is part of national and international discussion

Providing effective services is part of the social and healthcare reform

ICHOM-consortium aims at advancing the measurement of outcomes by defining disease-specific outcome sets in international cooperation.

Cost and effectiveness indicators by THL / MoSAH

Project: Indicators for measuring effectiveness (Pharma Industry Finland & Medaffcon Oy)

Effectiveness evaluation as part of the PERFECT project

Sweden has extensive experience in the use of quality registers and an ongoing wide cooperation project to improve the measurement of treatment outcomes

Government suggestion for the national performance measurement framework for social and healthcare services

A unified quality register system will be adopted in all hospital districts

THL, National Institute for Health and Welfare
MoSAH, Ministry of Social Affairs and Health
ICHOM, International Consortium for Health Outcomes Measurement
PERFECT, PERFormance, Effectiveness and Cost of Treatment episodes

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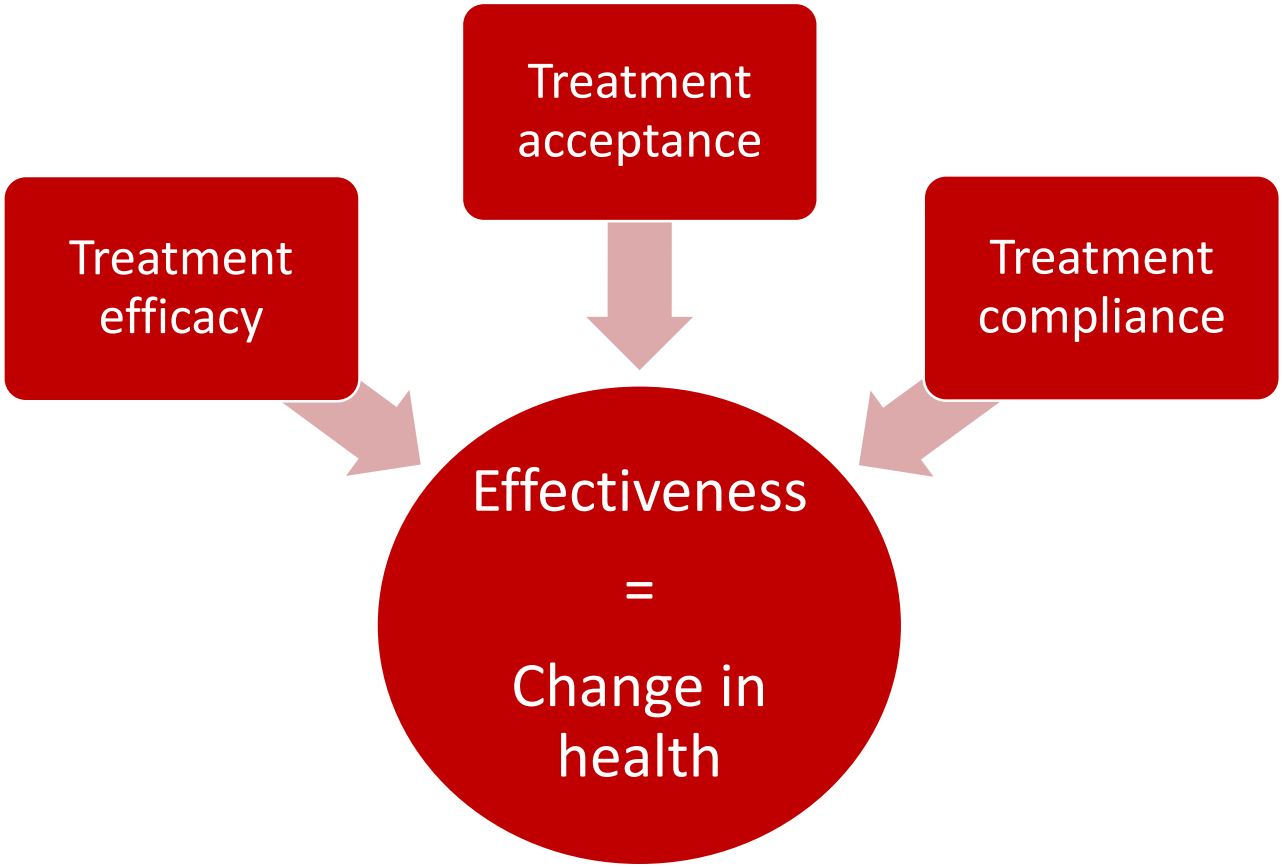
Perspective and considered diseases

- The practical level of patient and healthcare professional was selected as the perspective
- Pediatric diseases were excluded from the analysis
- Costs were not observed
- The considered diseases cause a significant disease burden in the Finnish population:

Pulmonary cancer	Inflammatory bowel diseases (IBD)	Type 2 diabetes (T2D)	Coronary disease
Cerebral apoplexy	Alzheimer's disease	Depression	

Effectiveness means health benefit (change in health) obtained with <u>treatment conducted in daily healthcare practice</u>



Sintonen 2013, Konu et al. 2009

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Sources used in compiling the proposal

- Care Guidelines
  - Pulmonary Cancer, Crohn's disease, Diabetes, Coronary disease, Cerebral infarction and TIA, Memory Diseases, Depression
- European Network for Health Technology Assessment (EUnetHTA): The HTA Core Model® 3.0
- International Consortium for Health Outcomes Measurement (ICHOM)
- TOIMIA Functioning Measures Database
- Patient Reported Outcome and Quality of Life Instruments Database (PROQOLID)
- Literature on the topic

Experts consulted in the compilation of the proposal

- **Pauliina Molander**, Specialist in Gastroenterology, MD, HUS Peijas Hospital Gastroenterology
- **Aija Knuutila**, Specialist in Pulmonology, Head of Department, Docent, HYKS (Helsinki University Central Hospital) Pulmonology
- **Leo Niskanen**, Specialist in Endocrinology, Chief Physician, Docent, HYKS Endocrinology and Metabolism
- **Mikko Syvänne**, Specialist in Cardiology, Professor, Terveystalo
- **Risto O. Roine**, Professor in Neurology, Director of Division, TYKS (Turku University Hospital) Neurology
- **Merja Viikki**, Specialist in Psychiatry, Docent, Tampere University and Tampere City Psychiatric day hospital
- **Harri Sintonen**, Professor (emeritus) in Health Economics, University of Helsinki

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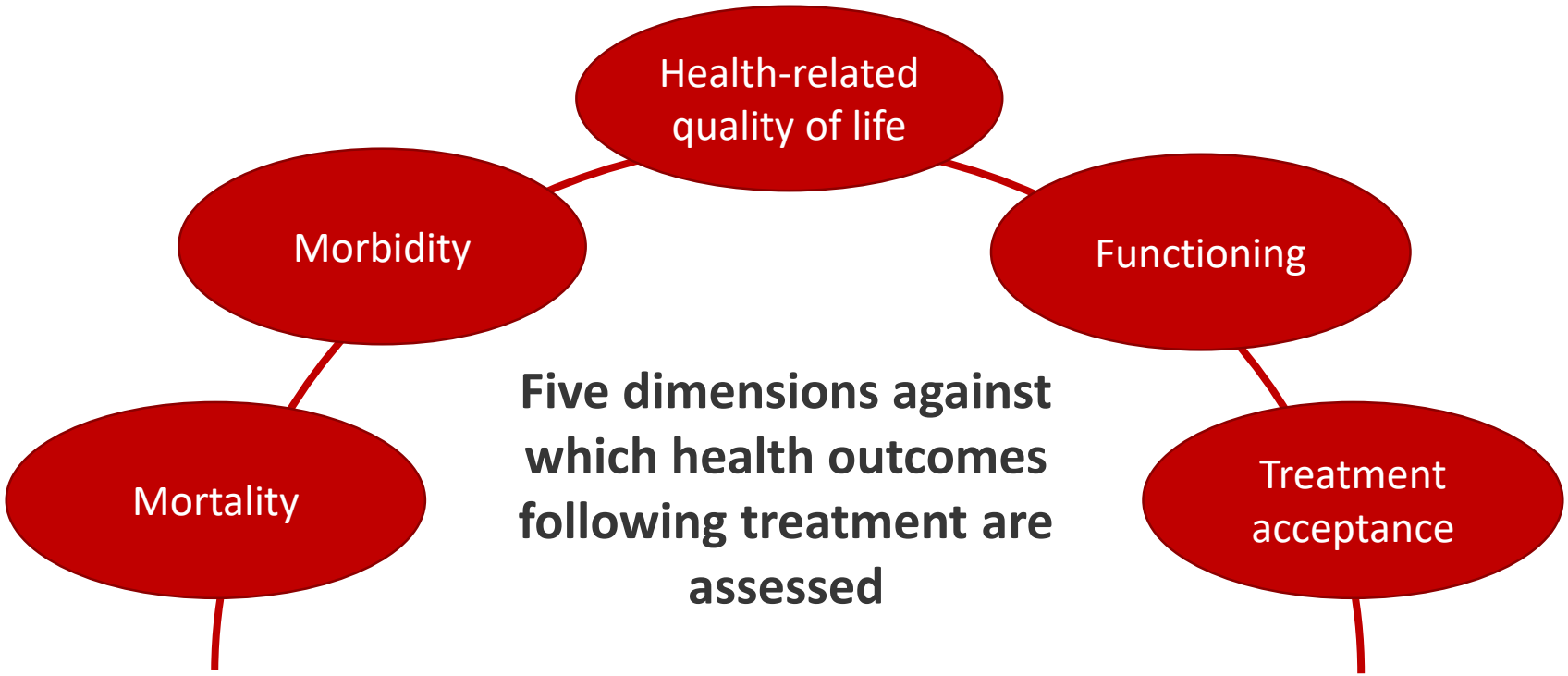
Effectiveness dimensions covered by the indicators



Modified from the EUnetHTA Core Model 3.0

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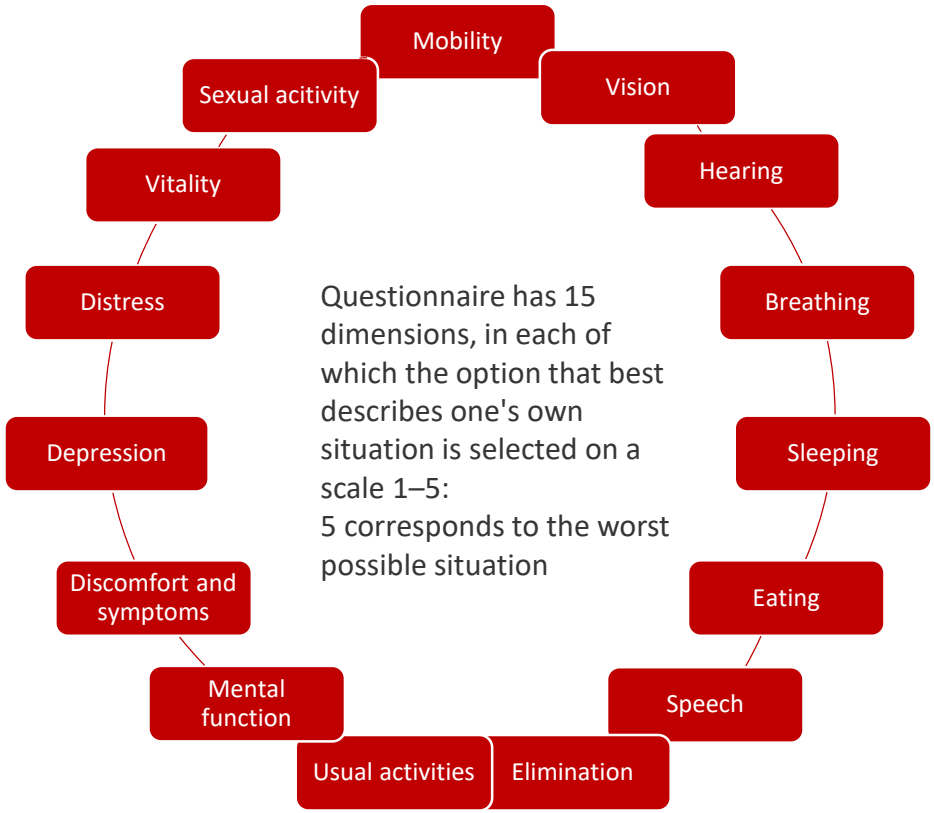
Indicator selections were based on the sources utilised in compiling the proposal

- The aim is a comprehensive analysis of health changes
- Mortality was considered in case of a life-threatening disease
- Care Guidelines had strong emphasis in the selection of the indicators describing morbidity
- Functioning and health-related quality of life were considered, because these are significant matters both for the individual and for the society
- Treatment adherence describes treatment acceptance

Selection of the health-related quality of life instrument

- 15D was selected as the instrument
  - More comprehensive compared to ie. EQ-5D
- If needed, it was complemented with disease-specific symptom queries or measurement tools describing functioning
- Another generic instrument (such as EQ-5D) could also be selected as the health-related quality of life instrument
  - This would require a new review of the necessity of disease-specific measures

We propose the 15D quality of life instrument for the measurement of health-related quality of life with all considered diseases



- A generic (non-disease specific) instrument has been developed in Finland
- It is more detailed than EQ-5D
- Finnish population values available for the interpretation
- Questionnaire is quick to fill (5–10 min)
- If necessary, it is applicable to count quality-adjusted life-years (QALY), which enables economic evaluation
- Has been used in studies evaluating the quality of life of the population and several patient groups

15D: http://www.thl.fi/toimia/tietokanta/mittariversio/138/ (in Finnish)

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Adherence is used as the treatment acceptance indicator

- Poor adherence may decrease the effectiveness of treatments, and therefore measuring it is justified in the impact assessment
- Adherence describes the commitment of the patient to the prescribed treatment
- Compliance (so-called secondary adherence) describes the precision in complying with the treatment instructions
  - Indirect measuring is possible with a patient interview
  - Measurement of compliance was omitted due to the challenges related to an indirect measurement method

Aarnio et Martikainen 2016; Simpson et al 2006; Lam et Fresco 2015

Effectiveness indicators

Dimension	Indicator/Measure	Observation period
Mortality	Overall mortality Disease-specific mortality	Period for which we propose effectiveness to be assessed Also measurement time
Morbidity	**Disease-specific indicators**	
Health-related quality of life	15D	
Functioning	Incapacity for work due to disease (disability pension and sick leave) **Disease-specific indicators**	
Treatment acceptance	Adherence (commitment to treatment)	

Consideration of baseline data is critical in the evaluation:

- Demographic data
- Risk factors, including lifestyle factors such as smoking
- Co-morbidities with diagnosis codes
- Difficulty level and type of the observed disease
- Previous treatments and their total duration

Selection of the observation period

- Observation period describes two issues:
  - Analysis period
    - collecting the information from the register for impact assessment
  - Measuring point (if applicable)
    - entering the information in the patient data system
- The following matters have impact on the length of the observation period:
  - Nature of the disease
    - Suddenly appearing vs slowly progressing disease
  - Prognosis of the disease
    - e.g. pulmonary cancer vs T2D
  - Estimated time before treatment response

Pulmonary cancer

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to pulmonary cancer	6 months
Morbidity	• **Disease-free survival with operated patients** • **Progression-free survival in spread disease (RECIST)** • **Serious complications from treatment**	12 months 6 months 12 months
Health-related quality of life	**15D questionnaire**	6 months
Functioning	• Incapacity for work due to pulmonary cancer (disability pension and sick leave) • **Home/not home and need for external assistance** • **ECOG-PS -classification**	6 months
Treatment acceptance	Adherence (in outpatient care)	6 months

RECIST=Response Evaluation Criteria in Solid Tumors,
ECOG-PS=Eastern Cooperative Oncology Group Scale of Performance Status

Patient self-assessment Disease-specific indicator

Inflammatory bowel diseases (IBD)

Dimension	Indicator/Measure	Observation period

Morbidity	• **Dysplasia findings and new colorectal cancer diagnoses**
	• **Disease activity**
	  • **Ulcerous colitis: Result of HUS symptom questionnaire and Mayo-score**
	  • **Crohn's disease: H-BI and SES-CD**
	  • **Calprotectin in stool (if not endoscopy)**
	• **Number of corticosteroid treatment periods and ward days due to IBD**
		12 months
		
		3 months and thereafter every 6 months
		
		12 months

Mortality (secondary indicator)	• Overall mortality • Mortality due to IBD	12 months
Health-related quality of life	**15D questionnaire**	6 months
Functioning	Incapacity for work or study due to IBD (absence)	12 months
Treatment acceptance	Adherence (in outpatient care)	12 months

IBD=Inflammatory bowel diseases, HBI=Harvey-Bradshaw Index, SES-CD=Simple Endoscopic Score for Crohn's Disease

Patient self-assessment

Disease-specific indicator

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Type 2 diabetes (T2D)

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to T2D or its complications	12 months
Morbidity	• **Microvascular complications and their progress (diabetic retinopathy*, nephropathy**, neuropathy***)** • **Macrovascular complications (myocardial infarction, cerebral apoplexy, periferic arterial disease****)** • **Sugar balance (HbA1c)** • **Serious hypoglycaemia (=patient has needed help from a healthcare professional)** • **Risk factor status (blood pressure, BMI, lipids, exercise, smoking)** • **Ward days due to T2D or its complications**	12 months
Health-related quality of life	**15D questionnaire**	6 months
Functioning	Incapacity for work due to T2D or its complications (disability pension and sick leave)	12 months
Treatment acceptance	Adherence (in outpatient care)	12 months

BMI=Body mass index

Patient self-assessment

Disease-specific indicator

* Laser treatment / poor sight

** Proteinuria / eGFR < 60 / dialysis treatment

*** Diabetic foot ulcer

**** revascularisation procedure of a lower limb / amputation

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Coronary disease

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to coronary disease or its complications	12 months
Morbidity	• **Cardiovascular events (myocardial infarction, acute heart failure, cerebral infarction)** • **Invasive procedures (angioplasty, bypass surgery)** • **Severe complications caused by invasive procedures** • **Complications describing the disease progress (unstable coronary disease, chronic heart or renal failure)** • **Risk factor status (blood pressure, BMI, lipids, sugar balance, exercise, smoking)** • **Ward days due to coronary disease or its complications**	12 months Severe complications caused by the invasive measure: 1 month
Health-related quality of life	**15D questionnaire**	6 months
Functioning	• **CCS classification** • Incapacity for work due to coronary disease or its complications (disability pension and sick leave)	12 months
Treatment acceptance	Adherence (in outpatient care)	12 months

BMI=Body Mass Index,
CCS=Canadian Cardiovascular Society

Patient self-assessment Disease-specific indicator

Cerebral apoplexy (cerebral infarction and cerebral hemorrhage)

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to cerebral infarction or cerebral hemorrhage	1 month, 3 months, 12 months
Morbidity	• **Relapse of stroke, cerebral hemorrhage or TIA** • **Complication of the acute treatment (severe bleeding: cerebral hemorrhage, retroperitoneal bleeding, gastrointestinal bleeding, deep phlebothrombosis, pulmonary embolism)** • **Disease complication (other severe cardiac or vascular event, MACE, pneumonia)** • **Procedures (decompressive craniectomy)** • **Risk factor status (blood pressure, BMI, sugar balance, lipids, exercise, smoking)**	12 months Complications of acute treatment: 1 month
Health-related quality of life	**15D questionnaire**	6 months
Functioning	• Incapacity for work due to cerebral apoplexy (disability pension and sick leave) • **Home/not home and need for external assistance** • **mRS class**	12 months mRS: 3 months, 12 months (last measure)
Treatment acceptance	Adherence	12 months

TIA=Transient ischemic attack, BMI=Body Mass Index,
MACE=major adverse cardiac events, MRS=Modified Rankin Scale

Patient self-assessment

Disease-specific indicator

Alzheimer's disease

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to Alzheimer's disease	12 months
Morbidity	• **Cognition status with CERAD exercise series** • **Depression status with Cornell's depression scale** • **Behavioral symptoms Cohen-Mansfield's agitation inventory (CMAI) (at least in institutional care)**	12 months Cornell and CMAI: 6 months
Health-related quality of life	• **15D questionnaire** filled by the patient or proxy • **Measured with 15D questionnaire also from the informal caregiver**	6 months
Functioning	• Incapacity for work due to Alzheimer's disease (disability pension and sick leave) • **CDR-SOB scale (can be also used to assess the response capacity of 15D questionnaire*)** • **Home/not home and need for external assistance**	12 months CDR-SOB: 6 months
Treatment acceptance	Adherence (in outpatient care)	12 months

CERAD=The Consortium to Establish a Registry for Alzheimer's disease,
CDR-SOB=The Clinical Dementia Rating Scale Sum of Boxes

Patient self-assessment Disease-specific indicator

* Hongisto et al. 2015

Solutions. Delivered.

Depression

Dimension	Indicator/Measure	Observation period
Mortality	• Overall mortality • Mortality due to depression	12 months
Morbidity	• **MADRS depression scale** • **Depression relapse** • **Risk factor status (exercise, sleep, smoking, alcohol, intoxicants)**	12 months MADRS: 6 months
Health-related quality of life	**15D questionnaire**	6 months
Functioning	• Incapacity for work due to depression (disability pension and sickness absence) • **SOFAS for under 65 years old**	12 months SOFAS: 6 months
Treatment acceptance	Adherence	12 months

MADRS=Montgomery-Åsberg Depression Rating Scale,
SOFAS=Social and Occupational Functioning Assessment Scale

Patient self-assessment

Disease-specific indicator

Applications of effectiveness indicators

- Assessment of the benefit received by an individual patient
- Characterisation of the patients who benefit from the treatment
- Comparison of alternative treatments within a treatment unit
- Comparison between treatment units or regionally
- Comparison between service providers
- Basis for resource allocation when connected to cost data
- After social and healthcare reform, producing and assessment of effectiveness data part of the system

Possible information sources of impact assessment

Mortality	Morbidity	Health-related quality of life	Functioning	Treatment acceptance
Cause of death (Statistics Finland)	Secondary and primary healthcare notifications (THL)	15D can be utilised within quality registers	Institutionalisation from Care Register for Health Care (THL)	Medication (Kela, ePrescription/ Kanta)
	Patient records (municipalities, hospital districts)		Occupational absence (Kela, Finnish Centre for Pensions)	
	Secondary and primary healthcare treatment data (Kanta)			
Information collection part of quality register?				

ISAACUS – the Digital Health HUB ?

THL=National Institute for Health and Welfare, Kela=Social Insurance Institution of Finland

Solutions. Delivered.

Current situation of information sources

- Information is behind separate permission process, change expected (legislative proposal on secondary use of data)
- Some disease-specific measures are not systematically entered in patient records or registers
- Quality registers are being built but national coverage still poor
- Regional data lakes under development, currently only one effectively in use
- Development of advanced data mining methods is fast but in child shoes and there is a lack of extensive knowledge

What next?

- Common principles for entering information
  - improve the quality of usable information (e.g. diagnoses, disease stage, causes of death etc)
- Automatisation of data transfer and mining
  - decreases the need for separate record in treatment units; these methods should be adopted as soon as possible
- Discussion on the realisation of the effectiveness evaluation
  - Who is the purchaser and who the provider of an impact assessment?
  - What is the assessment level? (healthcare unit, county or national)
- National pilot projects on effectiveness indicators are needed to assess their usability

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