Seminar, Regional forskningsstøtte i Helse Sør-Øst.

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Manuela Zucknick, Director for Oslo Centre for Biostatistics and Epidemiology, Regional forskningsstøtte i Helse Sør-Øst and IMB, MedFak, UiO

Tanker, forventninger og vyer for kliniske studier i årene som kommer:

A statistician's perspective

Biomedicine as a quantitative science:

Data centrality, quantity, and complexity keep growing

- Larger, more complex data, often collected for a different purpose (registries, real-world/EHR, omics, ...), needs causal inference
- "Real-world data": need to address biases and ensure data privacy
- Need for ML/ AI algorithms to be explainable & transparent, reliable, accounting for uncertainty and become more energy-efficient

In Clinical Trials:

- Causal inference in observational studies & clinical trials (addendum ICH E9 guideline)
- Personalised medicine: paradigm shifts in clinical trials (platform trials), increased need for integration of prior data, *in vitro* and *in silico* trials (digital twins)
- Pragmatic clinical trials: based on data from unselected patients recruited from common clinical practice.

Biomedical Data Science

- **Generative AI** uses data to create something new (text, images, videos)
- **Predictive AI** uses data to classify or predict (treatment response, survival prognosis)

- Machine Learning (ML) and AI are **hot topics** in biomedical research
- The future of biomedicine is heavily influenced by the impact of ML and statistics on the use of **next-generation (real-world, heterogeneous, multi-modal, …) data**

- Despite many advances in collection and analysis, positive impacts of ML are **not yet transformational**, and **potential of ML for life sciences** still underexplored in companies, organisations, science, and society at large

(Slide by Arnoldo Frigessi)

ML of today:

data centric and unable to integrate existing knowledge

Integreat

Knowledge-driven and data informed machine learning

Objectives of Integreat

- **Accurate ML**
- **Sustainable ML**
- **Explainable and trustworthy ML**
- **ML that quantifies uncertainty**

The "Integreat" SFF, centre of excellence (5 PIs from OCBE, including director A. Frigessi):

- will transform machine learning, by developing theories, methods, models and algorithms, which exploit **knowledge together with data**
- builds on a unique team of world-class **statisticians, logicians and machine learning researchers**, who will lay the foundations of knowledge-driven machine learning
- will solve important **real-world problems** in science, health and society, together with domain experts

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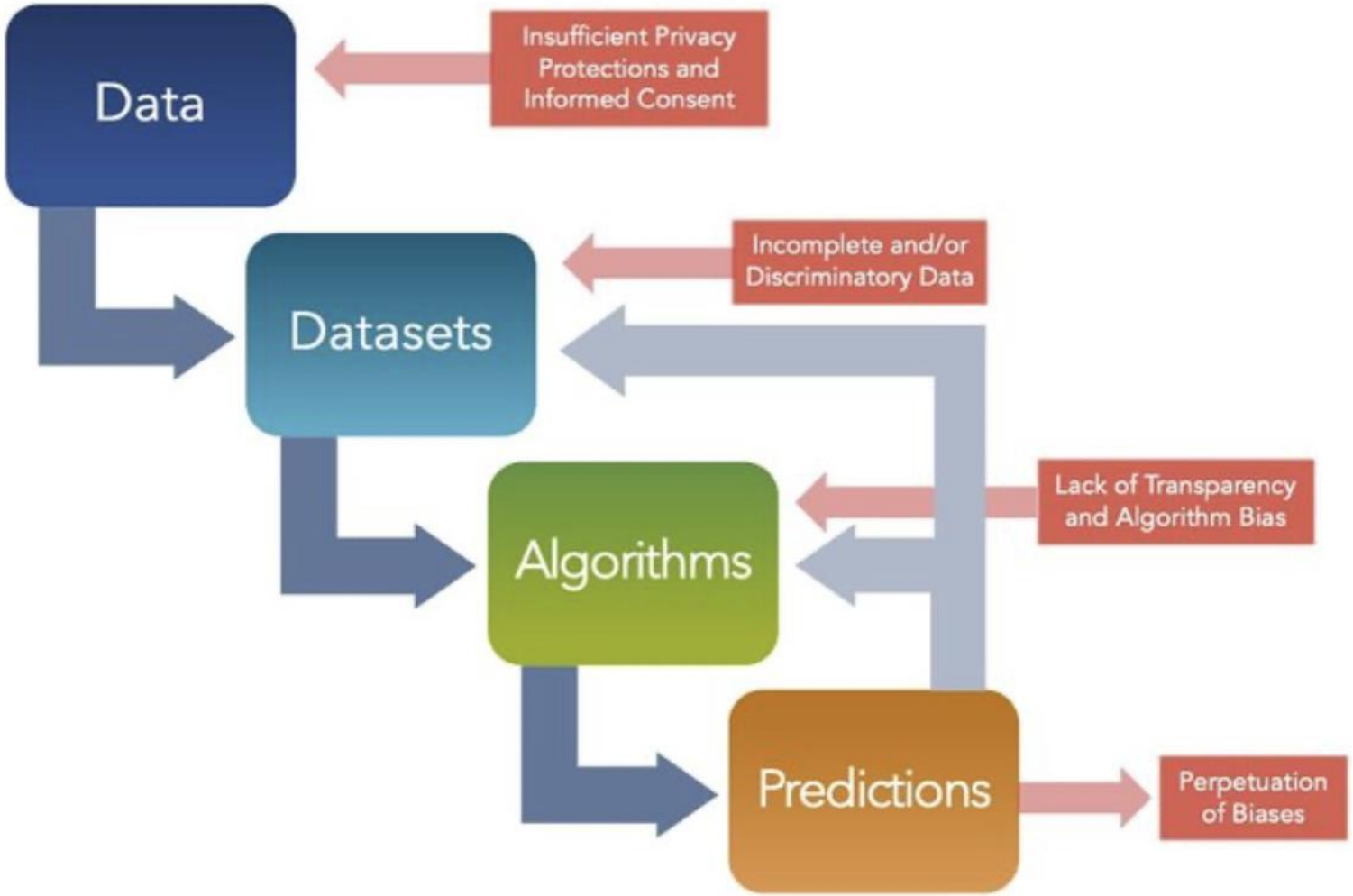
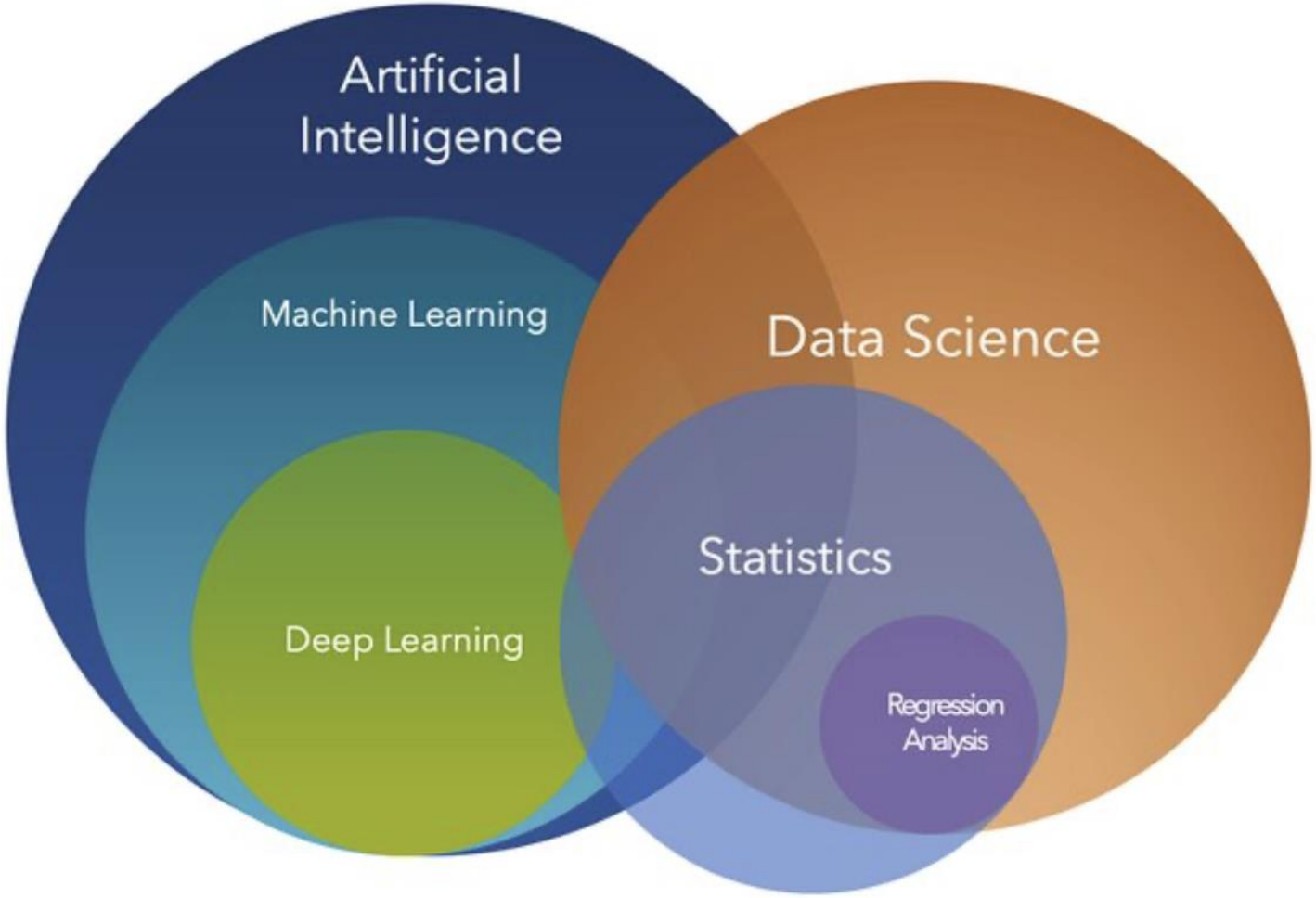
Domain experts

Challenges for real-world applicability of predictive AI



Morris et al. (2024). doi:10.3389/fsurg.2024.1393898

What is AI vs machine learning vs statistics vs …



An AI is the algorithm (ML or statistical) **plus** necessary frameworks to allow its use in praxis and at scale.

- **Integreat** will work on the core of the machinery: machine learning (and deep learning and statistics).
- **NEXTMAP** will build the full AI Factory.

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NEXTMAP

Harnessing AI for cancer prevention and treatment

Key partners:

- OCBE (RFST, HSØ and UiO)
- ICGI (OUS)
- IFI (UiO)
- OCC (OUS)

Plus many others

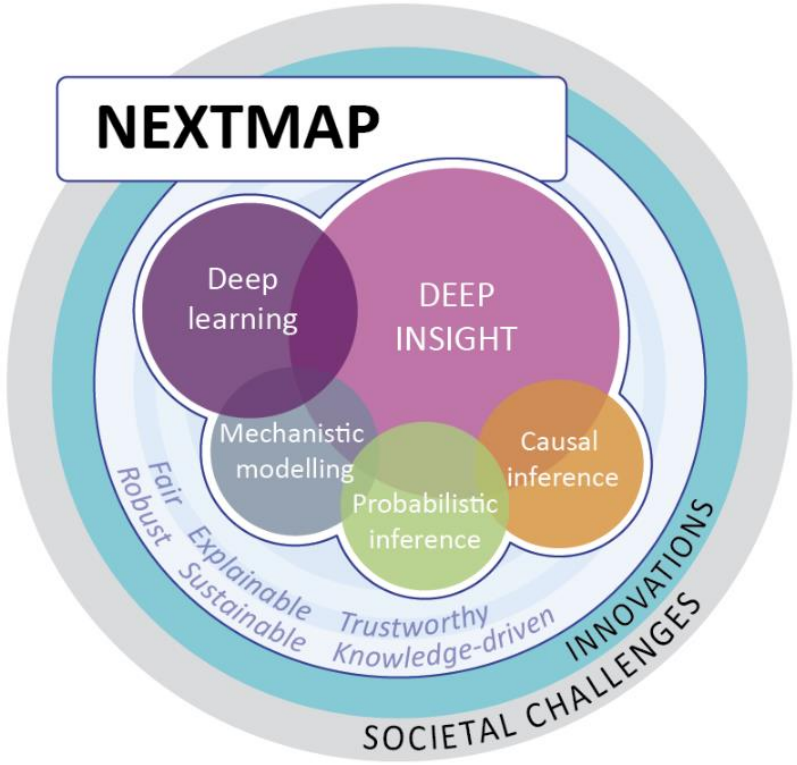

Figure 1: Nextmap core concept.

Mission:
Accelerate AI adoption by efficient use of multi-modal health data, robust and trustworthy AI deployment, and sustainability through reusable frameworks ("AI Factory")

NEXTMAP: From messy real-world data to clinical solutions

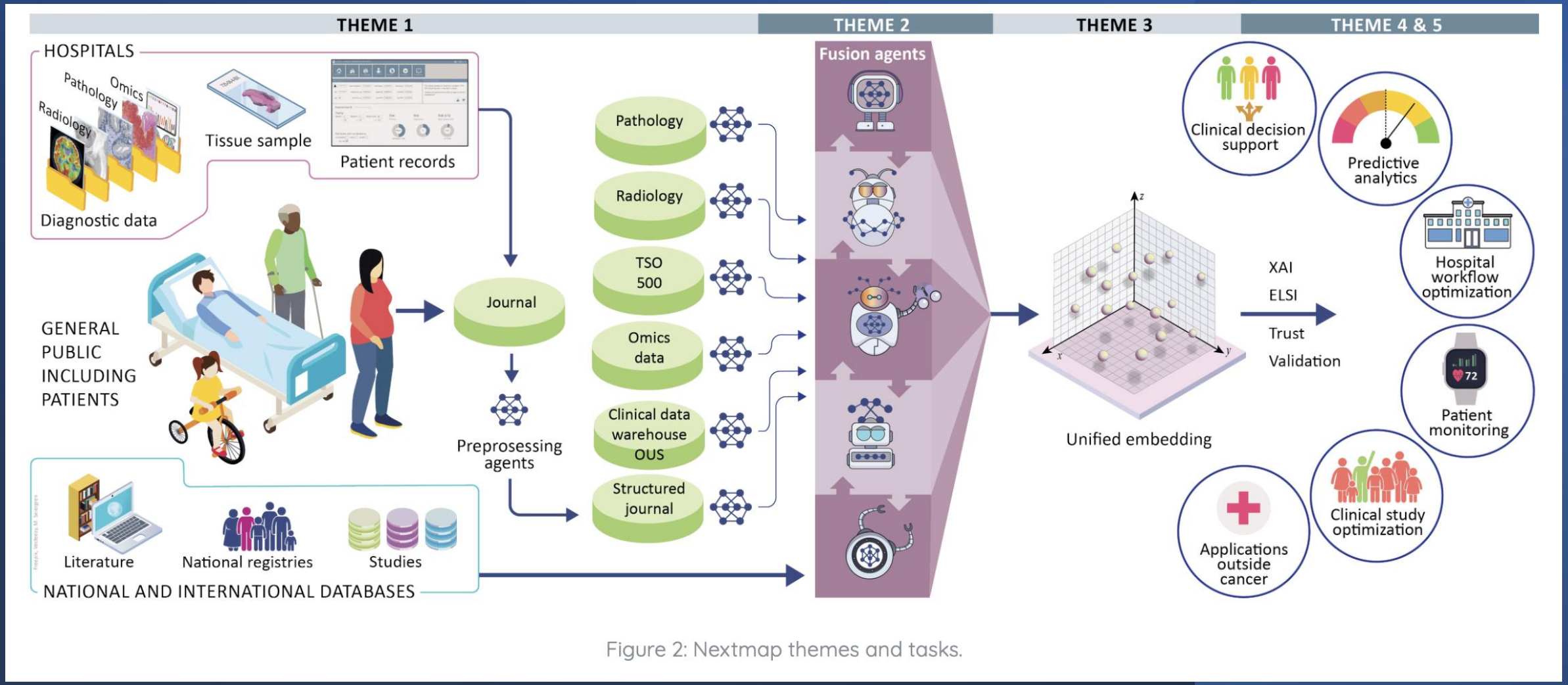

Figure 2: Nextmap themes and tasks.

AI for Clinical Trials (Goal: Halve the time for clinical trials.)

- **Pragmatic trials:** Design of matching external control arms, e.g. using registry data (presentation by Euloge Pagui, PRIME-ROSE, OCBE)
- **Precision medicine and platform trials:** Identification of suitable sub-populations to optimise treatment benefits and identify new target groups (across cancer types)

- **Example: Large-scale precise profiling of patients for treatment prediction**
  - All hospital patients over recent years (incl retrospective patient material)
  - Deep learning models using digital pathology to predict disease status relevant for targeted treatment.
- **Specifically: Response to immune therapy in lung cancer (e.g. PD-1/PD-L1 inhibitors)**
  - Deep learning prediction models identify responders based on multi-modal data including pathology
  - Apply to large hospital patient cohorts to find potential candidates for treatment across cancer types and identify new patient groups that benefit from treatment.

AI for (Cancer) Clinical Trials: Norway is in a unique position to lead

- **Unique data:**
  - Complete and linkable registry data: representing the entire population – the gold standard needed for building reliable AI
  - High degree of digitisation of a public health-care system: electronic health-care records, data on hospital operations
- **High trust** in the public health-care system and research
- **Strong research environments** in cancer and statistics/ machine learning. Knowledge-driven ML for accurate, trustworthy, sustainable AI.
- **Strong regulations** (EU and Norway): EU AI Act, GDPR. This is essential for maintaining trust.
- Bureaucracy makes real-world data use for research difficult and slow. The new Helseforsknings-loven needs to simplify processes, so we can benefit from our advantages and take the lead!

Thank you!