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## Outline

Innovation and regulation

Clinical innovation and regulation

Personalized medicine – as a case study

Innovation of regulation

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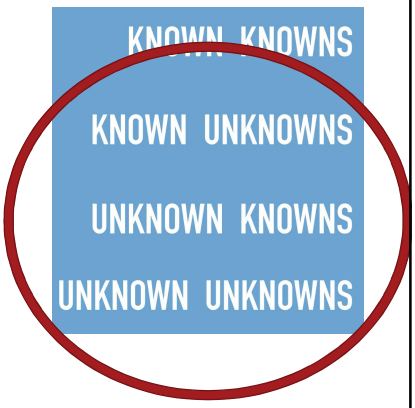
## Innovation and regulation

Regulating to stimulate and promote innovation

Regulation to mitigate possible harms

Innovation and uncertainty – a regulatory challenge

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## Innovation and regulation

Balancing in a landscape of unknowns

What are the potential benefits and risks?

- Assessment criteria
- Broad or narrow perceptions


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## Regulation and clinical innovation

Clinical innovation in the clinic

- In accordance with science and proven method

Clinical innovation in research/clinical trials

- "In a clinical trial the rights, safety, dignity and well-being of subjects should be protected and the data generated should be reliable and robust. The interests of the subjects should always take priority over all other interests." (EU Regulation 536/2014)

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## Personalized medicine

Personalized medicine can be defined as procedures that separate patients into different groups based on genetic and (numerous) other data. The purpose is to provide more tailored prevention and treatment to the individual patient.

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## Personalized medicine

How could a protocol look like?

- Project aims at establishing patterns to stratify patients with rheumatological conditions, diabetes and coronary artery disease to more tailored treatment
- Data used will be GWAS analysis of 150.000 samples from a population biobank storing residual samples from the clinic, as well as data from a number of other registries with health and socio-economic data.
- Due to the vast number of research subjects and the lack of perceived risks, individual consent will not be obtained.

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## REC assessment of personalized medicine project

- Scientific quality of the project?
- Are the rights, safety, dignity and well-being of research subjects protected?
- Do the interests of research subjects take priority?

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# Scientific quality

## Hypothesis-driven versus data-driven

*Section 18 Danish Act on Research Ethics Review of Health Research Projects*

3) the project's **scientific standard** meets the requirement that the project should lead to **new knowledge** or investigate existing knowledge, which **justifies** the implementation of the research project, cf. Section 1 (1) 2nd sentence, and

4) there is **sufficient reason** to undertake the project and expectations as to the project's **conclusions are justified**.

- Is is possible to make these assessments?
- Are the REC's equipped with the right competences (e.g. data specialists, bioinformatics, statistics etc)

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# Scientific quality

## Hypothesis-driven versus data-driven

Mol Endocrinol. 2015 Nov; 29(11): 1531–1534.
doi: 10.1210/me.2015-1253
PMCID: PMC4627602
PMID: 26524008

**Editorial: Would You Like A Hypothesis With Those Data? Omics and the Age of Discovery Science**

W. Lee Kraus

On the other hand, applicants must provide a clear justification for the need, as well as an explanation of the benefit, of their proposed omics experiments. Importantly, they must present clear hypotheses for the discovery- and mechanism-focused omics experiments that they propose, as noted above, and avoid descriptive omics experiments. Furthermore, applicants must include 1) a clear plan for analyzing and sorting through the data, 2) a proposal for establishing the priority of targets for follow-up analyses, 3) a description of the expected outcomes, and 4) how the outcomes will provide a test of the hypothesis. The

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## Protection of research subjects

Are the rights, safety, dignity and well-being of research subjects protected?

- Which rights? Privacy, autonomy, equity, right to non-discrimination …… ?
- Who's rights? Research subjects, family members, childrens' rights …?
- Which risks/harms? Bodily harm, mental and social harm? (infringement of privacy, incidental findings, risk of discrimination, lack of control, etc)

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## Protection of research subjects

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PLoS One. 2018; 13(10): e0204937. PMCID: PMC6181558
Published online 2018 Oct 11. doi: 10.1371/journal.pone.0204937 PMID: 30308031

### Considerations for ethics review of big data health research: A scoping review

Marcello Ienca, Conceptualization, Data curation, Formal analysis, Investigation, Methodology, Writing – original draft, Writing – review & editing,[#1] Agata Ferretti, Conceptualization, Data curation, Formal analysis, Investigation, Methodology, Writing – original draft, Writing – review & editing,[#1] Samia Hurst, Conceptualization, Funding acquisition, Methodology, Project administration, Supervision, Writing – review & editing,[2] Milo Puhan, Conceptualization, Funding acquisition, Methodology, Writing – review & editing,[3] Christian Lovis, Conceptualization, Funding acquisition, Methodology, Writing – review & editing,[4] and Effy Vayena, Conceptualization, Funding acquisition, Methodology, Project administration, Supervision, Writing – review & editing[1,*]

Godfrey Biemba, Editor

analysis were performed on the full-texts of all articles included in the synthesis. Our findings suggest that while big data trends in biomedicine hold the potential for advancing clinical research, improving prevention and optimizing healthcare delivery, yet several epistemic, scientific and normative challenges need careful consideration. These challenges have relevance for both the composition of ERCs and the evaluation criteria that should be employed by ERC members when assessing the methodological and ethical viability of health-related big data studies. Based on this analysis, we provide some preliminary

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## Balancing of interests

Do interests of research subjects take priority?

- Balancing the unknown versus the unknown
- Scientific freedom
- The power of hopes (and hypes)

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## Innovation of regulation

Regulatory frameworks needs to be better aligned with new research designs

Composition of REC must reflect the necessary expertise

The legal framework should be more open to both factual an evaluative changes

- Inger Johanne Sand, The Regulation of Vital Risks, Uncertainties and Scientific Controversies, in *Ret & Usikkerhed*, 2005-

Compensating those who have suffered harms

- Prainsack and Buyx, *Solidarity in biomedicine and beyond*, 2017.

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**Policy, practice and patient experience in an age of intensified data sourcing (POLICYAID)**

See more on https://policyaid.ku.dk

**Personalized Medicine in the Welfare State (Me*In*We)**

See more on https://meinwe.ku.dk

**Standards for personalized medicine (EU-STANDS4PM)**

See more on https://www.eu-stands4pm.eu

**PM-Heart**

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