where science and ethics meet

Sprint #2 Workshop
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eHealth, Innovation and Patients Centredness

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‘Patient Centricity’ is ‘in’

We listen...

UCB advertisement at the Brussels airport:

Inspired by *patients* –
Driven by *science*

AstraZeneca:

Science and patients
…the heart of everything we do

Sanofi:

A global integrated health-care leader focused on patients’ needs

Patients' Need:
Access to Effective and Safe Treatments

- Reliably effective
- No (or bearable) side effects
- Affordable for me and my healthcare system
- Available when I need it
- Well investigated – in general terms

An Illusion:

To believe
that patients
WANT
to join a clinical trial

Current Paradigm for Access to Treatment

- Demonstration of evidence for efficacy and safety in randomised clinical trials in patients
  - Performed according to strict protocol conditions in selected patient populations under high quality standards
  - Required patient numbers defined by statistical rules
  - Data generated for the purpose of the clinical trial
  - Access to efficacious treatment stopped after patient's end of trial participation
  - High drug attrition rates during clinical development make patient participation in a trial often useless
- Outcome of the clinical trials, benefit-risk assessment and quality of documentation assessed by competent authorities as basis for marketing authorisation
- Request for demonstration of positive cost-benefit balance and added value by HTA/payers based on additional studies

Patients' Reasons for Trial Participation

Patients consent to participate in a clinical trial

- Because they have no other choice to get access to a new treatment option
- Because they get access to free medication and diagnostic
- For altruistic reasons
- If they want to follow their doctor's advice

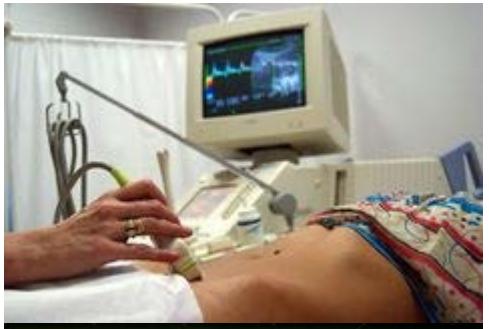
But not because they <u>want</u> to expose themselves to the risk and burden of research -

especially in a moment when they fight with being sick

Consequence: Change of Paradigm?

No,

- we will not be able to change the paradigm of data generation from exposure of a new medicine to patients
- we will not want to move back from governmental responsibility for release of efficacious and safe treatments assessed from reliable and secure data
- our societies cannot afford anymore to provide unjustified access to all types of treatments

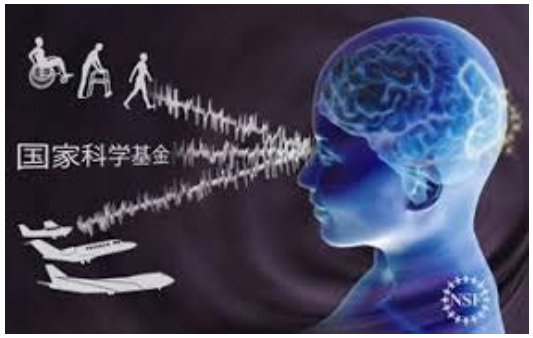


Mandatory Consequence: Change of Conditions

We have ethical and societal obligations **to find better ways** to achieve access for patients to efficacious and safe treatments

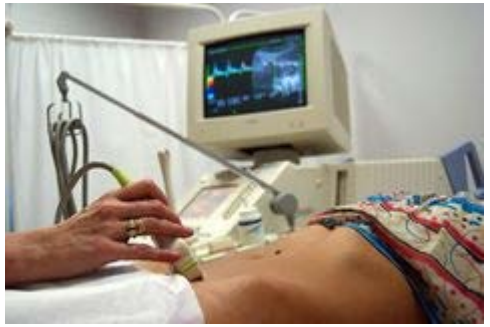
- Faster growing and better managed scientific knowledge
- Innovative R&D methodology
- Imaginative, reliable and affordable technology
- Integrative IT infrastructure in healthcare

and **to have the courage to implement demonstrated advancement quicker**



Key Areas for Change of Conditions

- We need to find ways to make clinical trials an element of better healthcare
- We need to enable other reliable but secure data sources than clinical trials to demonstrate efficacy, safety and added value
- We need to minimise the number of patients required to participate in clinical trials
- We have to find ways to facilitate patients' involvement in clinical trials:
  - Minimise their risks
  - Minimise their burden
  - Maximise their chance for effective treatment



Innovative Tools for Change of Conditions

- Decentralised, digital studies, “trials at home”
- Computer Modelling & Simulation, *in silico* clinical trials, digital patients
- eHealth Records to collect Real World Data, “Big Data”
- Biobanks, Registries, global databases
- Distributed Ledger Technologies (“BlockChain”) for increase of security, privacy and interoperability of all data relating to health
- Etc.

Future
“Patient Centredness”

- Systematic educated patient involvement in all processes of new treatment development to ensure focus on patients needs
- Responsible use of resources to achieve gradual and break-through improvements of treatments affordable by all societies
- Innovation to be limited by patients’ protection and rights

Thank you for your attention!