# Personalized Medicine and True eSource

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#### eSources



#### **Danish Medicines Agency's Data Analytics Centre (DAC)**

#### Vision:

"Through use of clinical trial and real world data and advanced analytical methods we want to increase the accessibility of safe and effective medicines and medical devices"

## By utilizing the available data in new ways DAC can help usher in a new regulatory paradigm

Business area	Now	Future
Post-approval	<ul> <li>Development and approval &gt; 10 years</li> <li>Approval based on RCT</li> <li>Subsequent detection of suspected side effects and signal generation</li> </ul>	<ul> <li>Precision Medicine</li> <li>Breakthrough / PRIME</li> <li>Conditional approvals expands</li> <li>ADR supplemented with real world data</li> <li>RWD: Registries, EHR, SoMe etc.</li> </ul>
Pre-approval	<ul> <li>Authorities review summaries of data</li> </ul>	<ul> <li>Authorities have access to applicant's raw data (CDISC)</li> </ul>
Scientific advice	<ul> <li>Increasingly complex advanced clinical trial designs</li> <li>No quantitative scientific advice</li> </ul>	<ul> <li>Authorities expanded capacity -&gt; quantitative scientific advice</li> <li>Better regulatory framework</li> </ul>

#### **DAC platform is shared with National Genome Centre (NGC)**



- Data silos
- High transaction costs
- Low level of cross-fertilization of data
- Not full utilization of High Performance Computing



- Full utilization of High Performance Computing
- Private cloud to cloud hosting
- Realtime data access in highly secure computer environment into analysis / data lake platform
- Full traceability of data use and reproducibility of analysis
- Ability to control access to fully anonymized data and permissioned access for researchers
- Allowing for future development such as automated additional data collection, real time AI/ML into data analysis

### **DAC** and life science ecosystem



- We aim to be
  - a trusted data broker
  - using high quality data
  - fully transparent and reproduceable
  - playing a direction setting role in life science in an ethical sound use of big data and AI/ML
- What we do should help industry and patients to make drugs and technology faster and safely available
- Ultimately this should benefit patients by reducing inequality in health care with data analytics while protecting data

## **First example: DAC COVID**

#### cohort and analyses done in collaboration with academia and other national authorities

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ENEPP	European Network of Centres for Pharmacoepidemiology and P	narmacovigilance
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ews bout Us NCePP Documents raining in PhEpi and PV ode of Conduct	Administrative Details Ta	rets of the Study Methodological Aspects Documents First registered on: 15/04/20 Last updated on: 21/04/20
tandards & Guidances NCePP Study Seal	1. Study identification	
ublic Consultation	EU PAS Register Number	EUPAS34734
lossary of terms	Official title	Use of non-steroidal anti-inflammatory drugs and clinical outcome of COVID-19: a Danish nationwide cohort study
esources Database	Study title acronym	NSAID COVID-19
	Study type	Observational study
artners forum U PAS Register bout EU PAS Register	Brief description of the study	In the early stages of the COVID-19 pandemic in Europe, case reports from southern France described young patien without comorbidities who developed severe COVID-19 at exposure to ibupcrofen. This led to warnings against use of ibuprofen and other NSAIDs in patients with COVID-19 by multiple parties, including the French health ministry. However, no data has been published regarding the safely NSAIDs in COVID-19. We aim to study the association between NSAID use and rick of death in patients with COVID-19. In secondary analyses, associations between NSAIDs and hospitalisation, ICU admission and mechanic ventilation will be investigated. This is a Danish nationwide registry-based cohort study. All individuals tested positive severe acute respiratory syndrome coronavirus 2 will be followed from the date of positive test and 30 days onward for occurrence of death, and from the date of positive test and 14 days onward for occurrence of hospital admission, ICU admission, and mechanical ventilation. Use of NSAID will be compared to non-use using an exposure assessme window of 30 days prior to the positive test. Risks, risk difference and relative risk will be estimated for each outcome.
	Is the study required by a Risk Ma Plan (RMP)?	agement Not applicable
	Regulatory procedure number (R/	P
	Category 1 and 2 studies only)	

- Use of NSAIDs and risk of critical adverse outcomes in patients with COVID-19
- Renin–angiotensin–aldosterone system inhibitors and severe outcomes in patients with COVID-19
  - The role of inhaled anti-inflammatory pharmaceuticals in COVID-19 incidence, morbidity, and mortality
  - Prognosis of coronavirus disease in patients with immune-mediated inflammatory diseases treated with immunomodulating agents and biologics
- Risk of venous thromboembolism in patients with COVID-19: A nationwide, population-based matched cohort study
- Impact of use of proton pump inhibitors on susceptibility to infection and risk of severe outcomes in patients with COVID-19
  - + two papers describing the governance and the database

#### Priority Recommendations of the HMA-EMA joint Big Data Task Force

 Deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis and Real World Interrogation Network -DARWIN). Build the business case with stakeholders and secure funding to establish and maintain a secure EU data platform that supports better decision-making on medicines by informing those decisions with robust evidence from healthcare.

•Establish an EU framework for data quality and representativeness. Develop guidelines, a strengthened process for data qualification through Scientific Advice, and promote across Member States the uptake of electronic health records, registries, genomics data, and secure data availability.

Enable data discoverability. Identify key meta-data for regulatory decision-making on the choice
of data source, strengthen the current ENCePP resources database to signpost to the most
appropriate data, and promote the use of the FAIR principles (Findable, Accessible, Interoperable
and Reusable).

 Develop EU Network skills in Big Data. Develop a Big Data training curriculum and strategy based on a skills analysis across the Network, collaborate with external experts including academia, and target recruitment of data scientists, omics specialists, biostatisticians, epidemiologists, and experts in advanced analytics and AI.

 Strengthen EU Network processes for Big Data submissions. Launch a 'Big Data learnings initiative' where submissions that include Big Data are tracked and outcomes reviewed, with learnings fed into reflection papers and guidelines. Enhance the existing EU PAS register to increase transparency on study methods.

 Build EU Network capability to analyse Big Data. Build computing capacity to receive, store, manage and analyse large data sets including patient level data (PLD), establish a network of analytics centres linked to regulatory agencies, and strengthen the Network ability to validate AI algorithms.

Modernise the delivery of expert advice. Build on the existing working party structure to
establish a Methodologies Working Party that encompasses biostatistics, modelling and simulation,
extrapolation, pharmacokinetics, real world data, epidemiology and advanced analytics, and
establish an Omics Working Party that builds on and reinforces the existing pharmacogenomics
group.

•Ensure data are managed and analysed within a secure and ethical governance framework. Engage with initiatives on the implementation of EU data protection regulations to deliver data protection by design, engage with patients and healthcare professionals on data governance, and establish an Ethics Advisory Committee.

Collaborate with international initiatives on Big Data. Support the development of guidelines
at international multilateral fora, a data standardisation strategy delivered through standards
bodies, and bilateral collaboration and sharing of best practice with international partners.

 Create an EU Big Data 'stakeholder implementation forum'. Dialogue actively with key EU stakeholders, including patients, healthcare professionals, industry, HTA bodies, payers, device regulators and technology companies. Establish key communication points in each agency and build a resource of key messages and communication materials on regulation and Big Data.

- Platform (DARWIN)
- Data Quality
- Discoverability
- Skills
- Submissions
- Capabilities
- Expert advice
- Security and ethics
- Collaboration
- Stakeholder implementation forum

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