EURORDIS
RARE DISEASES EUROPE

HTx PROJECT
Next Generation HTA

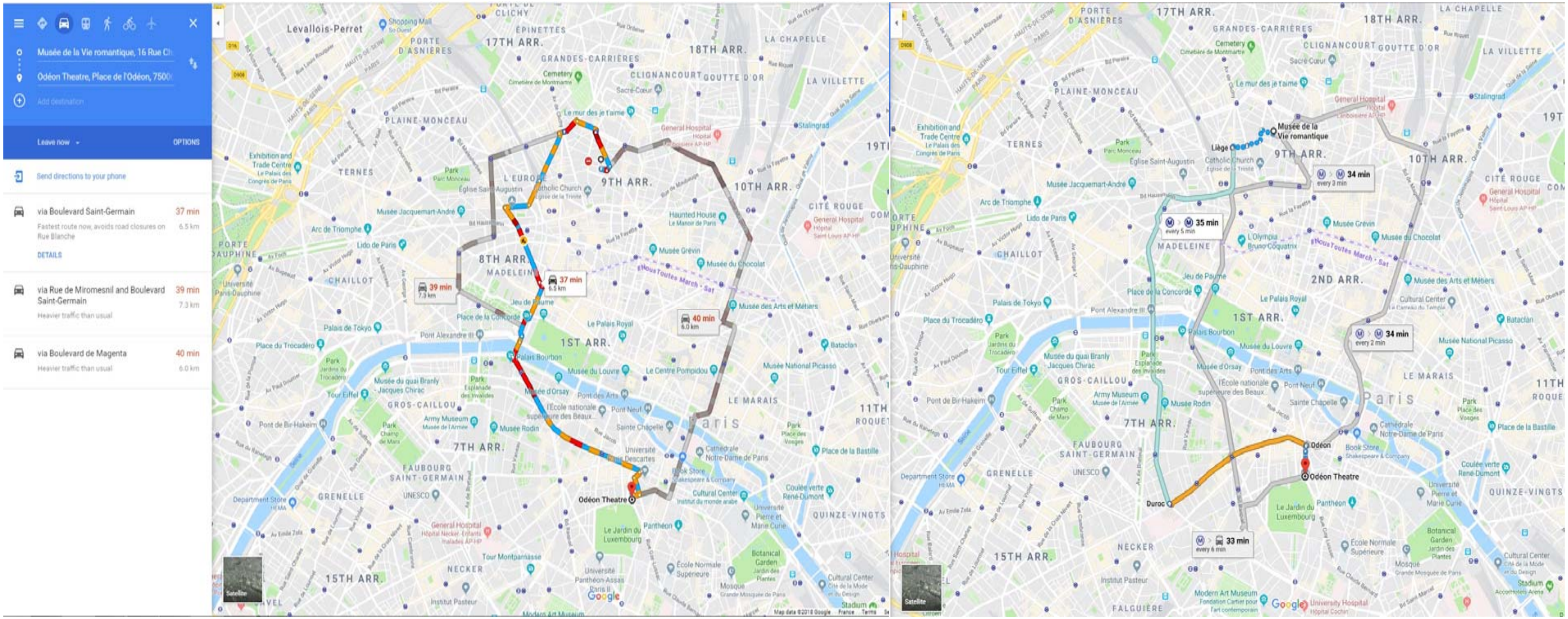
A European Horizon2020 project

Matteo Scarabelli, Patient Engagement Manager HTA, EURORDIS

HTx: next generation HTA

- Building framework and tools for a new HTA model for decision-makers,
  - which makes full use of Real World Evidence (RWE)
  - through Artificial Intelligence (AI) and Machine Learning (ML)
  - fitting with tailored and personalized healthcare
  - simulating models to predict the most *cost-effective* treatment pathways for each patient and new payment models
- Making the whole HC system enter in the new digital era

Hey Google, calculate the best way to…



Hey Google, calculate the best way to…

- PROCESSING…
  - Background information (maps, rules)
  - Real-time information (lights, traffic) and variables
  - Options (no ferry/tramway)
  - Costs

➢ …OUTPUT: range of solutions

- LEARNING…
  - On working days, at 8am and 7pm it shows schedule and delays about your usual way home/work
  - At 8am and 7pm itineraries are tailored on your home/work segment and are different than other hours
  - If you are circling around with your car, it suggests you where to find a parking
  - (Trip information)

- Artificial intelligence
  - having uncountable quantities of data simultaneously, it’s the ability to process them based on given rules
- **Machine learning**
  - **accessing large databases, the software can “extract” and “learn” new rules and apply them (and even new ones, not given by its code)**
- **(applying what we call Deep Learning: accessing huge stock of background data from which the software can extract “new” rules/output)**

Exemple of Machine Learning

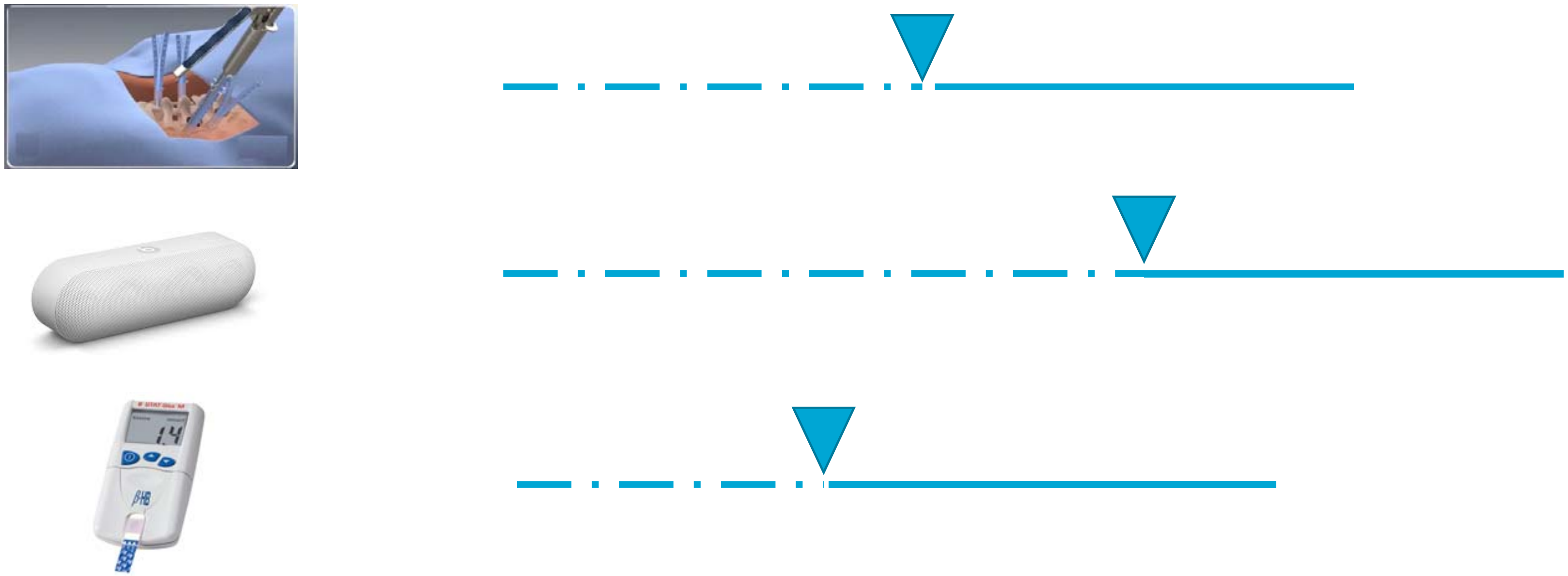
- Online translation
  - These are usually « stupid » software
    - They can translate word-by-word only
    - No flexibility or ability to recognise different meanings for different « contexts », or the sense of idiomatic phrases
    - e.g. EN: learning by heart -> FR: apprendre par cœur …. (-> IT: imparare a memoria… -> GER: auswendig lernen)
  - You can set a software with certain commands ("translate A into B"), but rules which relies on skills/faculties (as recognise idiomatic phrases or jokes) are more complex
- BUT…
  - if you put thousands/millions/billions of texts, novels, articles translated by literary experts in a database, the software is able to analyse them and "extract" new rules, and so start to apply them (it can go as far as to write a novel)

In medical field

- Improve diagnostic accuracy:
  - algorithm trained to detect skin cancer or melanoma using 130,000 images of skin lesions representing over 2,000 different diseases
  - A facial recognition software combined with machine learning can diagnose rare genetic disorder: patient photos are analysed using facial analysis and deep learning to detect phenotypes that correlate with rare genetic diseases.
- Medical imaging:
  - Checking a brain tumor take 30-40 minute to the surgeon in real-time
  - A ML software can analyse billions of scans/images and identify brain patient’s tumour mass, size, form and features, exact 3D position, tumour composition based on cells’ distribution: in a few minutes (before the surgery)
- →(same principle) Better -personalized- treatment decisions:
  - using patients’ medical information and history to optimize the selection of treatment options

Technologies and HTA so far…

- Single technologies
  - Each one goes through a single HTA (approximatively at the moment of the authorisation/approval)

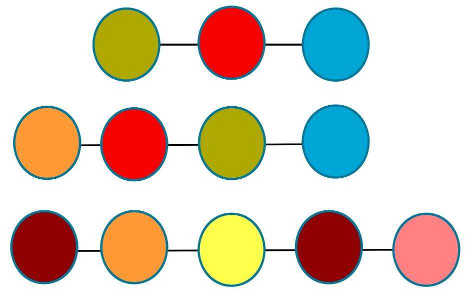


…from now on:
combination of technologies & tailored treatment pathways

- Single technologies

VS

- Complex combinations of technologies
  - Combination tailored on patients: personalised medicine
    - Surgical procedures
    - First, second, third line medicines
    - (e-)diagnostic/monitoring



…..

about 720 combinations*

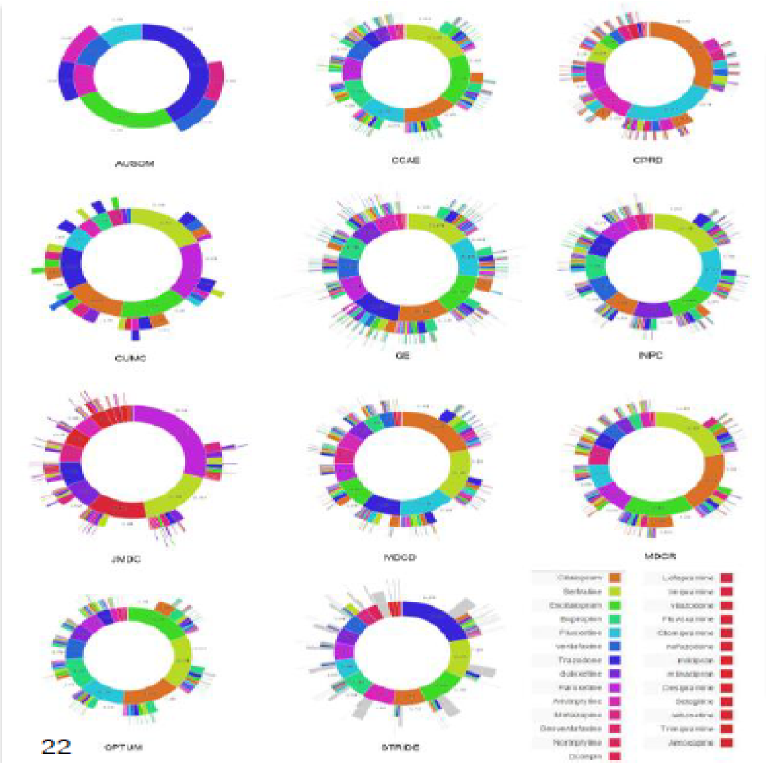
*$P(n,n) = n(n-1)(n-2)\ldots = n!$

Combination of technologies & tailored treatment pathways: Data Science in healthcare

Understanding Drug Utilisation

EUROPEAN MEDICINES AGENCY

Depression



Observational Health Data Science and Informatics (OHDSI)

Substantial variation in treatment practice for depression across data sources, health systems, geographies, and over time

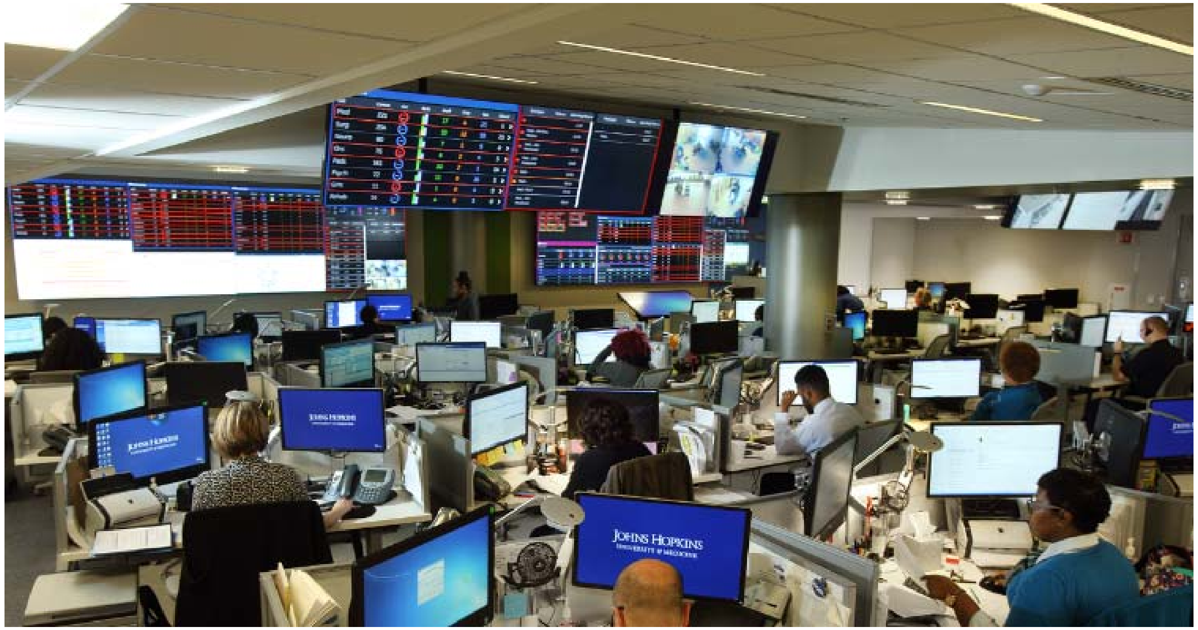
Consistent heterogeneity in treatment choice as no source showed one preferred first-line treatment

11% of depressed patients followed a treatment pathway that was shared with no one else in any of the databases

Use of RWE can help identify the most appropriate comparator group

"From the project abstract"

"Clinical history, use of health technologies (drugs, medical devices and e-health technologies), preferences and outcomes: a system of patient-centred information about risks, benefits, outcomes and costs associated with a range of possible strategies"



eunethta
EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

"The same information is made available to HTA agencies, whose decisions are informed by means of this information for the benefit of payers and decision-making: **that's framework is what we envision as HTx**"

What HTx is ?

- HTA for complex and personalised treatment pathways with combinations of technologies
  - Using RCTs data and RWE (treated with AI and Machine Learning)

= HTx (effectiveness and cost-effectiveness analysis) to inform decision-making,

- No more single HTA at the time of the approval only
- (real-time) HTA based on constant flow and processing of RWE
- highly differenced and tailored scenarios
  - simulating models to predict the most *effective and cost-effective* treatment pathways for each patient
  - clinicians and patients can be provided in real-time with the information on the more cost-effective treatments, *at the moment of prescription/decision*
- new economic and payment models

Project length

Kick-off Meeting	
21 - 22 January, 2019	@ Utrecht University

Implementation and Transferability

- **How that should work in the practice of clinicians and HTA experts?**
- **Can these tools be implemented in different systems across Europe?**

PILOTS COUNTRIES AND HTA AGENCIES:

NETHERL	SWEDEN	ENGLAND
ZIN	TLV	NICE
Mr Wim Goettsch	Mr. Niklas Hedberg	Ms Jacoline Bouvy Dr. Pall Jonsson

OTHER PARTNERS

- NETHERLANDS
  - **Dr. Mr Wim Goettsch & Prof. Mr Olaf Klungel**
  - *Utrecht University (UU)*
- DENMARK
  - **Prof. Ms Marie Louise (Marieke) De Bruin**
  - Copenhagen Center for Regulatory Science, *University of Copenhagen* (UoC)
- FINLAND
  - **Prof. Ms Juha Röning & Prof Mr Pekka Siirtola**
  - Biomimetics and Intelligent Systems Group, *University of Oulu (UoU)*
- United Kngdom
  - **Prof. Lr Andrea Manca**
  - Center of Health Economics, *University of York (UoY)*
- NETHERANDS
  - *University of Maastricht (UoM)*
- SPAIN
  - *Synapse research management (SYNAPSE)*
- BULGARIA
  - **Prof. Guenka I. Petrova,**
  - *Medical University of Sofia (MUS)*
- SWITZERLAND
  - **Prof Georgia Salanti**
  - *University of Bern (UBERN)*
- SPAIN
  - **Prof Gema García-Sáez**
  - *Universidad Politecnia de Madrid (UPM)*
- BELGIUM
  - **Mr Lifang Liu, MD**
  - *European Organisation for Research and Treatment of Cancer (EORTC)*
- HUNGARY
  - **Prof Zoltan Kalo**
  - *Syreon Research Institute*

Pilot therapeutic areas

CARACTERISED BY HIGHLY UNMET MEDICAL NEEDS and MULTI-TYPE TECHNOLOGIES

1. ONCOLOGY
   - *Head and Neck Cancer*
2. COMMON DISEASES
   - *Diabetes Mellitus Type 1 and 2*
3. RELAPSING-REMITTING FORM WITH LARGE THERAPEUTIC ARSENAL
   - *Multiple Sclerosis*
4. RARE DISEASES
   - *Myelodysplastic syndrome MDS (blood cells disorder)*

Eurordis role?

Information

- Patients in the pilots and beyond
- Explaining how it works and the impact
- Meetings & Webinar

Training

- For patients advocates
- Summer School module

Communication

- Patients – HTAs – Public
- Dissemination toolkit

How can patients contribute to this project?

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Questions?