11 aprile 2019

Ospedale Papa Giovanni XXIII
Auditorium «Lucio Parenzan»
Piazza OMS 1 - Bergamo

LE SFIDE DELL'INNOVAZIONE TECNOLOGICA NELLA RICERCA CLINICA

La posizione degli Enti Regolatori

Noemi Porrello

La storia di Joanne

Genomic medicine saved Joanne Hackett's life. As a child, she was hospitalised repeatedly for what were thought to be epileptic seizures—once suffering 50 in just one day.

Il sequenziamento del genoma è diventato accessibile e il suo ruolo determinante per le scelte terapeutiche

	Blockbuster medicines	Targeted therapies	Personalized treatments
			
Target population	Large: unspecified	Medium: sub-group	Small: individual patient
Diagnostics	No specific biomarkers	Single disease marker	Comprehensive NGS & response monitoring
Treatment	Empirical and “One drug fits all”	Targeted agents biomarker guided	Profile-guided with personalized clinical decision support

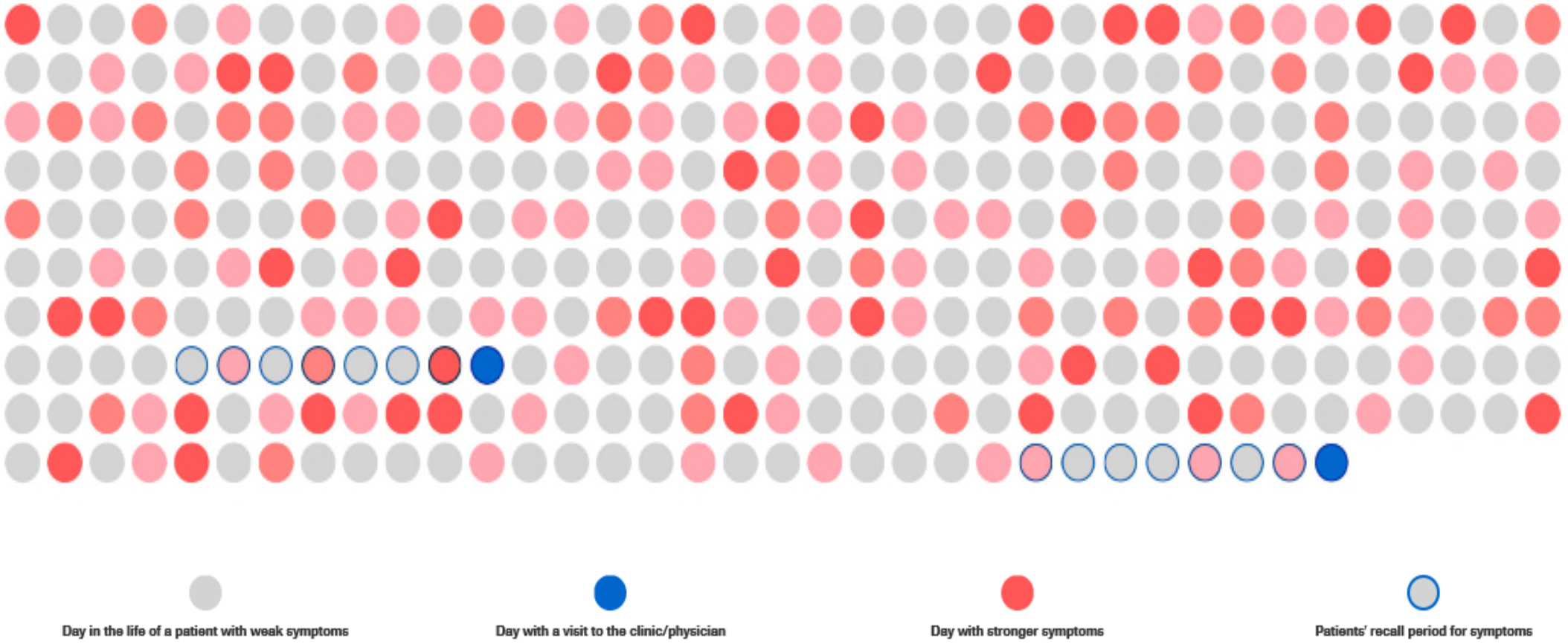
Increasing need for advanced data analytics capabilities

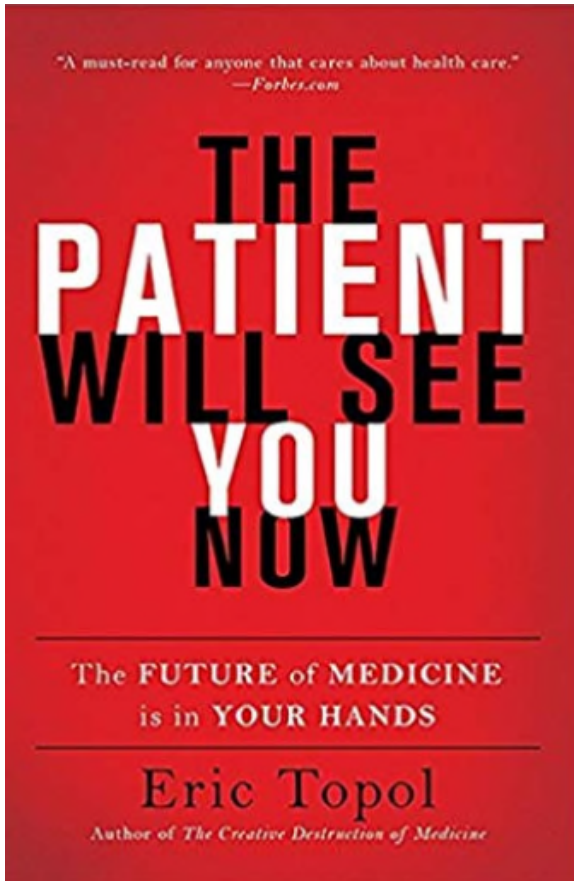
NGS=Next generation sequencing

La digitalizzazione in ambito sanitario ha fortemente influenzato anche l'ambito della ricerca clinica

- Cell phones
- Wearables
- Robots
- Sensors
- Medical devices
- Monitoring equipment
- Chat Bots
- Mobile apps
- EHR
- Video conferencing
- Computer programmes
- Social media
- Artificial intelligence

365 giorni


Day in the life of a patient with weak symptoms
Day with a visit to the clinic/physician
Day with stronger symptoms
Patients' recall period for symptoms



"*Democratizzazione della medicina*":
la digitalizzazione consente al paziente di assumere un ruolo sempre più centrale nella gestione della propria salute.

Il contesto sanitario presenta significative opportunità da cogliere, per migliorare outcome e sostenibilità

UTILIZZO DELLE INFORMAZIONI NON OTTIMALE

PERCORSI DI CURA FRAMMENTATI

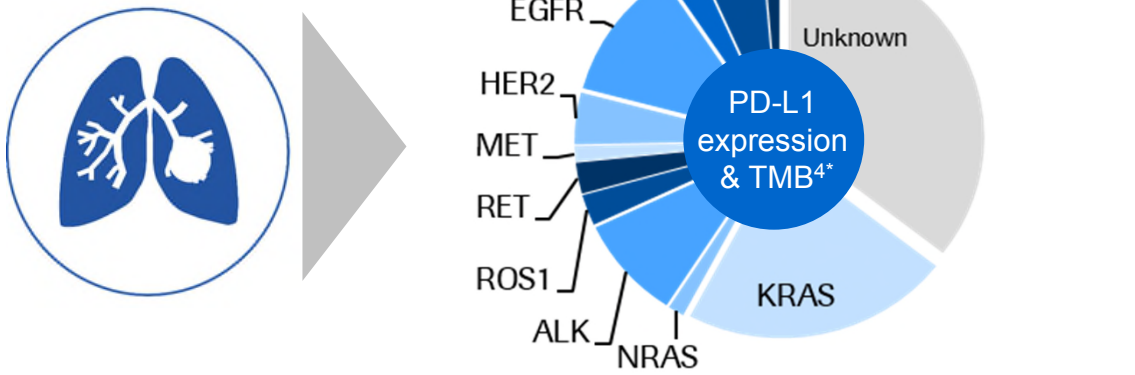
FOCUS SULLA PATOLOGIA VS FOCUS SULLA SALUTE

LENTA ADOZIONE DELL'INNOVAZIONE

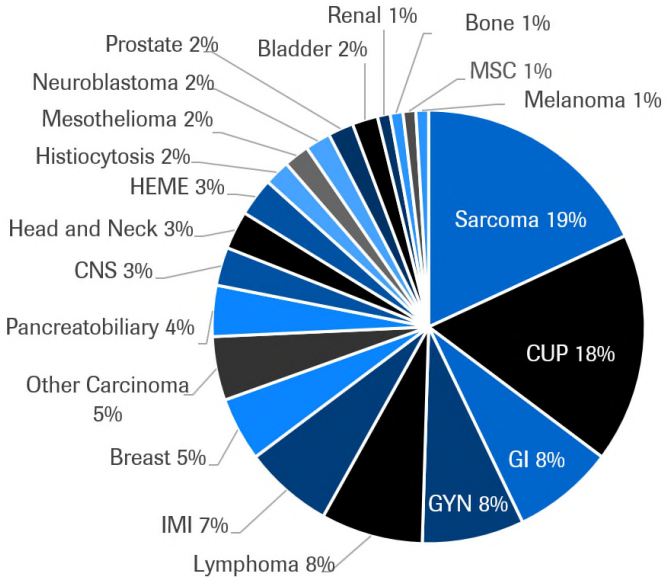
La sfida per gli Enti Regolatori

La diagnosi molecolare permette di identificare sottogruppi con specifiche caratteristiche e di personalizzare i percorsi di cura

NSCLC – unica sede, diversi sottogruppi di popolazione



Alterazione del gene ALK nelle diverse sedi tumorali



The NEW ENGLAND JOURNAL of MEDICINE

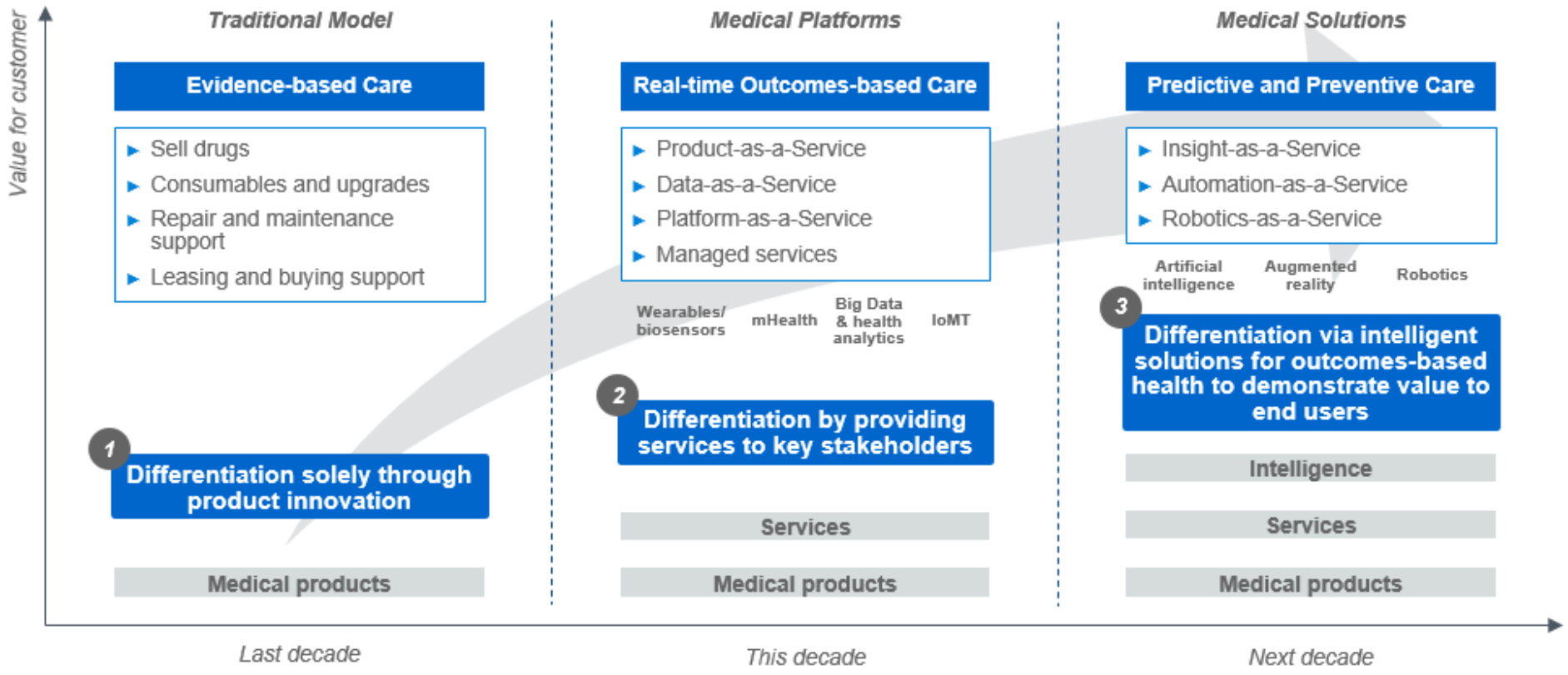
Perspective

OCTOBER 12, 2017

First FDA Approval Agnostic of Cancer Site — When a Biomarker Defines the Indication

Steven Lemery, M.D., M.H.S., Patricia Keegan, M.D., and Richard Pazdur, M.D.

La valutazione si sposta dal singolo farmaco al valore integrato di farmaco, servizi e tecnologie sul percorso di cura



Source: Transforming Healthcare through Artificial Intelligence Systems. Frost & Sullivan - AI Health and Life Sciences Conference, London, October 2016; EY analysis

STATE OF THE ART

Roche

Data Rich, Information Poor: Can We Use Electronic Health Records to Create a Learning Healthcare System for Pharmaceuticals?

Hans-Georg Eichler[1], Brigitte Bloechl-Daum[2], Karl Broich[3], Paul Alexander Kyrle[2], Jillian Oderkirk[4], Guido Rasi[1], Rui Santos Ivo[5], Ad Schuurman[6], Thomas Senderovitz[7], Luke Slawomirski[4], Martin Wenzl[4] and Valerie Paris[4]

CLINICAL PHARMACOLOGY & THERAPEUTICS | VOLUME 105 NUMBER 4 | APRIL 2019

A "**Learning Healthcare System**" based on electronic health records and other routinely collected data will be required to harness the full potential of RWD to complement evidence based on randomized controlled trials.

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EMA Regulatory Science to 2025

Strategic reflection

Draft strategy published in December 2018 for a six-month public consultation

Foreword by Prof. Guido Rasi, EMA Executive Director

The pace of innovation has accelerated dramatically and regulators need to be ready to **support the development of increasingly complex medicines** that more and more deliver **healthcare solutions by converging different technologies**.

https://www.ema.europa.eu/en/about-us/how-we-work/regulatory-science-2025

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EMA Regulatory Science to 2025

Strategic reflection

The five **key goals** of the strategy include:

1. catalysing the integration of science and technology in medicine development
2. driving collaborative evidence generation, improving the scientific quality of evaluations
3. advancing patient-centred access to medicines in partnership with healthcare systems
4. addressing emerging health threads

https://www.ema.europa.eu/en/about-us/how-we-work/regulatory-science-2025

HMA
Heads of Medicines Agencies

EUROPEAN MEDICINES AGENCY
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13 February 2019
EMA/105321/2019

HMA-EMA Joint Big Data Taskforce
Summary report

CORE RECOMMENDATIONs WS4

1. Interoperability
2. Data Quality
3. Data Sharing and Access
4. Data Linkage
5. Validation of analytical approaches
6. Regulatory guidance on evidence acceptability
7. Implementation of the new regulations for devices
8. New skills
9. Regulatory engagement with external stakeholders

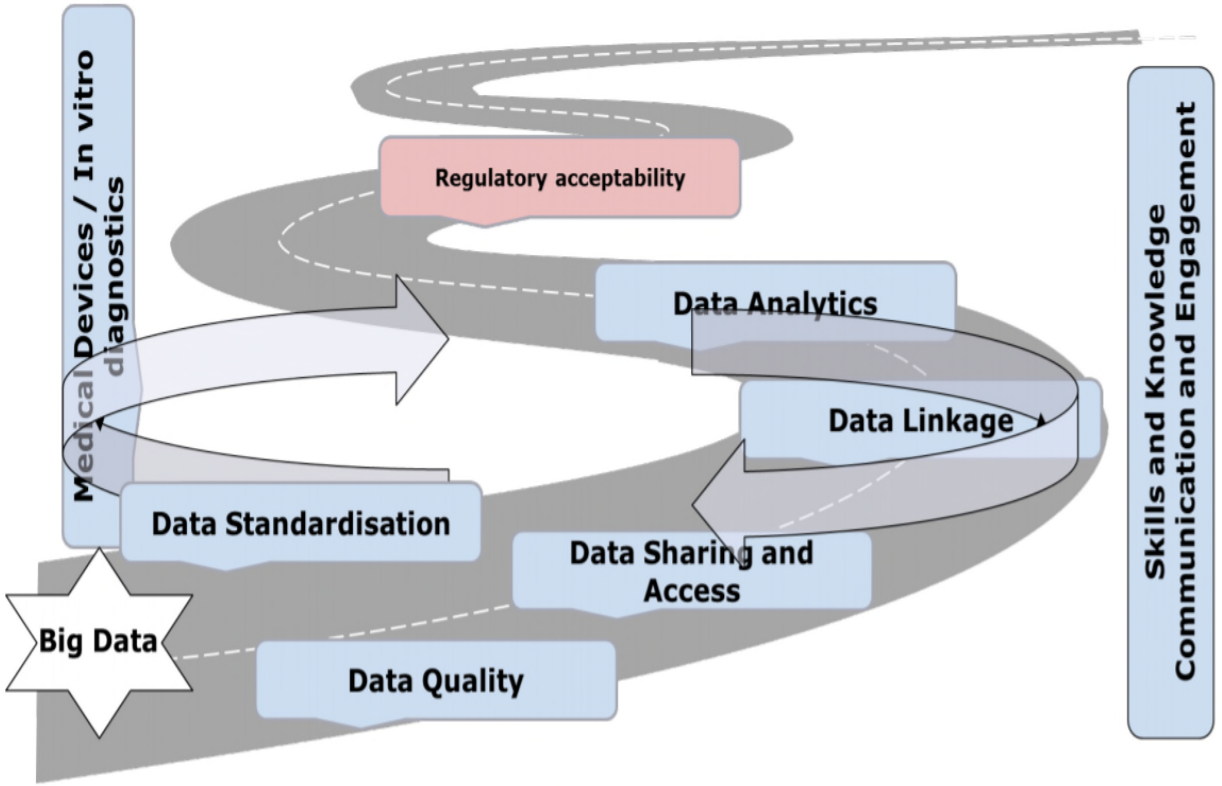

Figure 1: The Road to Regulatory acceptability: an integrated strategy reflecting core recommendations to support the use of Big Data in the assessment and monitoring of medicinal products in Europe. The individual steps are not necessarily sequential, may not be required across all datasets, many are interdependent and all will require active and iterative communication between all stakeholders.

https://www.ema.europa.eu/en/documents/minutes/hma/ema-joint-task-force-big-data-summary-report_en.pdf

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REGULATIONS

REGULATION (EU) 2017/745 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 5 April 2017
on medical devices, amending Directive 2001/83/EC, Regulation (EC) No 178/2002 and Regulation (EC) No 1223/2009 and repealing Council Directives 90/385/EEC and 93/42/EEC
(Text with EEA relevance)

REGULATIONS

REGULATION (EU) No 536/2014 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 16 April 2014
on clinical trials on medicinal products for human use, and repealing Directive 2001/20/EC
(Text with EEA relevance)

REGULATIONS

REGULATION (EU) 2016/679 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 27 April 2016
on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC (General Data Protection Regulation)
(Text with EEA relevance)

L'evoluzione tecnologica è decisamente più rapida dell'evoluzione normativa