Con il patrocinio della

CONFERENZA DELLE REGIONI
E DELLE PROVINCE AUTONOME

Regione Emilia-Romagna

BOLOGNA

HOTEL BOLOGNA FIERA

Piazza della Costituzione, 1

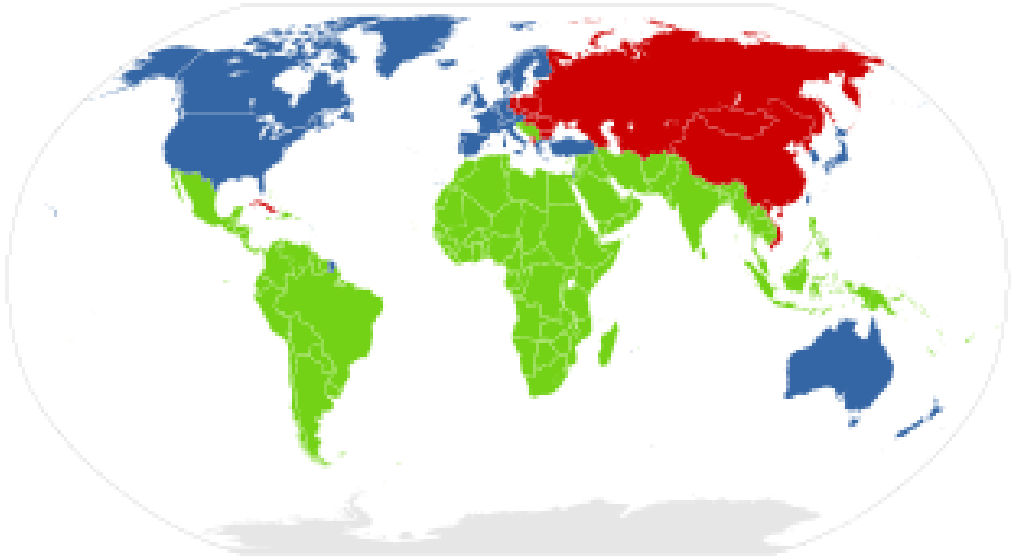
8 MAGGIO 2019

DALLA BIOLOGIA MOLECOLARE ALLA TERAPIA DI PRECISIONE

IL FAST TRACK DI UNA ONCOLOGIA PERSONALIZZATA

2019 MOTORE SANITÀ
Gestire il Cambiamento

Solfrini Valentina
Area Farmaci e Dispositivi Medici
Servizio Assistenza Territoriale
Direzione Generale Cura della Persona Salute e Welfare
Regione Emilia Romagna

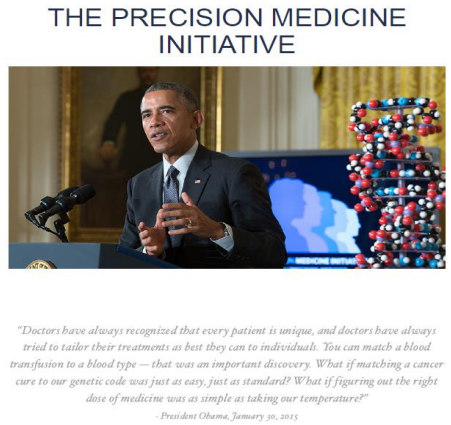


L’AGENDA ISTITUZIONALE NEL MONDO RICCO DELL’OCCIDENTE

THE PRECISION MEDICINE INITIATIVE

"Doctors have always recognized that every patient is unique, and doctors have always tried to tailor their treatments as best they can to individuals. You can match a blood transfusion to a blood type — that was an important discovery. What if matching a cancer cure to our genetic code was just as easy, just as standard? What if figuring out the right dose of medicine was as simple as taking our temperature?"

- President Obama, January 30, 2015



The conceptual distinction between personalized and precision medicine

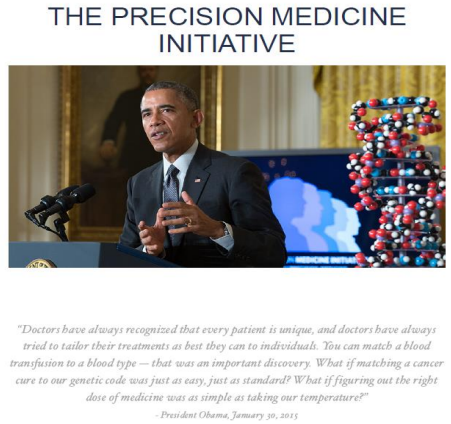
Personalized medicine refers to an approach to patients that considers their genetic make-up but with attention to their preferences, beliefs, attitudes, knowledge and social context, whereas precision medicine describes a model for health care delivery that relies heavily on data, analytics, and information. This model goes beyond genomics and has vast implications for our nation's research agenda and for its implementation and adoption into health care. **Precision medicine – and the ecosystem that supports it -- must embrace patient centered-ness and engagement, digital health, genomics and other molecular technologies, data sharing and data science to be successful.**

THE PRECISION MEDICINE INITIATIVE

"Doctors have always recognized that every patient is unique, and doctors have always tried to tailor their treatments as best they can to individuals. You can match a blood transfusion to a blood type — that was an important discovery. What if matching a cancer cure to our genetic code was just as easy, just as standard? What if figuring out the right dose of medicine was as simple as taking our temperature?"
- President Obama, January 30, 2015

Data Sharing and Infrastructure Needs

The implementation of PM will require access to large-scale, detailed, and highly integrated patient data. Thus, many initiatives are focused on increasing inter-operability of patient data and enhancing data systems that enable the use of PM data at the point of care. Although great strides have been in recent years towards achieving a "paperless health care system" that is based on EMRs, **much more needs to be done to integrate data across systems and to mine data that already exist but remain in silos**.



An Action Plan for Precision Medicine

The full realization of precision medicine's disruptive potential will require a multipronged scientific, clinical and policy agenda. Democratization of data underpins both the scientific advances that enable not only precision medicine but medicine itself. **A culture with proper incentives for sharing of data will be required**. The precision medicine ecosystem's stakeholders - participants, patients, providers, payers and regulators – each **will require evidence of value in terms of quality of life, quality of medical care and efficiency and effectiveness optimized for cost**. If successful, more care will occur before disease is apparent – **a shift from disease treatment to disease prevention and early detection**. Precision medicine is not uniquely American – it is a global agenda - and requires global leadership and perseverance to see it through to its rightful place in health and society.

Foresight

Precision Medicine

Targeted scenario N°14

Glimpses of the future from the BOHEMIA study

European Commission

Foresight

Precision Medicine

Targeted scenario N°14

Glimpses of the future from the BOHEMIA study

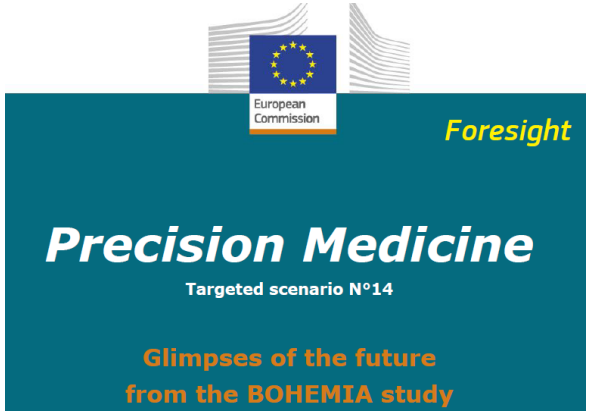
Targeted scenario N° 14

Precision Medicine

Summary

It is 2040. Individualized precision medicine combining mass data analyses, genetic engineering, epigenetics, and knowledge about the personal microbiome and the biotic environments helps anticipate and cure illnesses. Human enhancement is an issue of ethical and regulatory concern.

UN Sustainable Development Goals (SDGs) most relevant to this scenario:



Future Directions for EU R&I policy recommended by the public consultation

- Making use of biotechnologies for personalized medicine
- **Personalised disease prevention for every-day life (including personalised diet and physical activity programmes)**
- International standards and quality assurance for precision medicine
- **Strong public health orientation: precision medicine for all, as a part of the way 'towards health for all'**
- **Providing a healthy start to our next generation**
- Understanding the human microbiome
- Understanding epigenetic mechanisms and first applications

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Personalised medicine challenges: hype or hope?

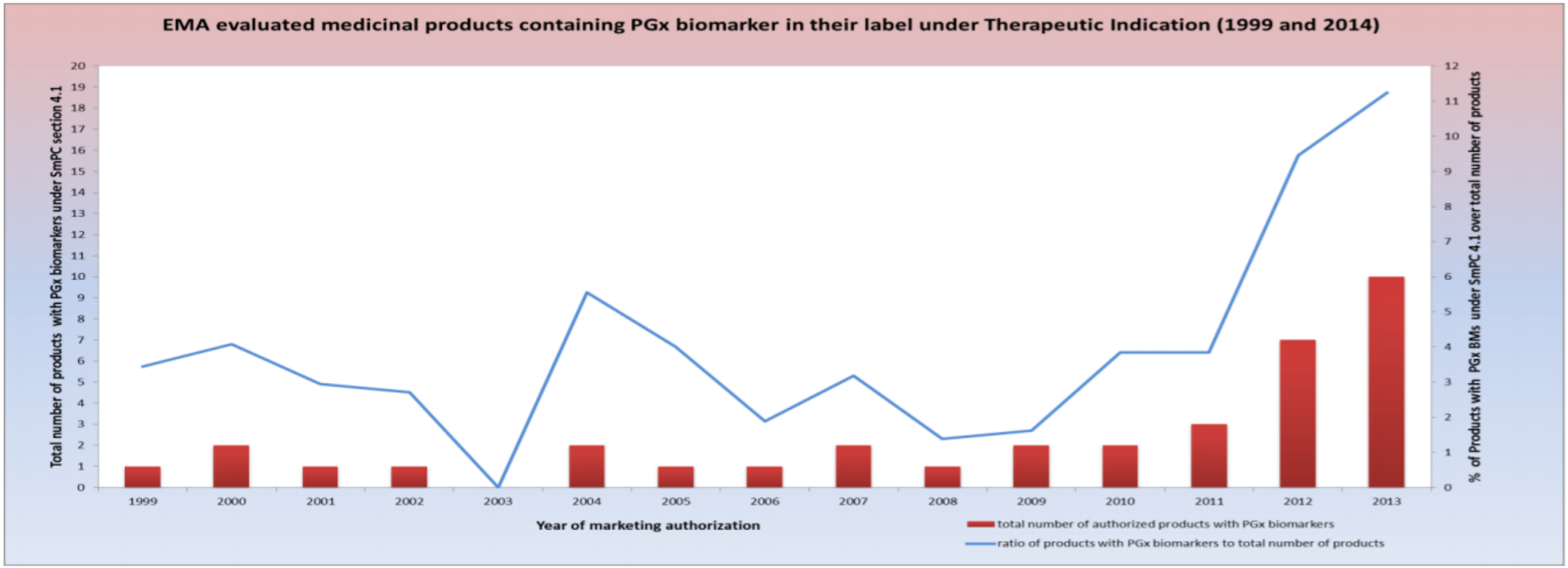
Presented by Marisa Papaluca

An agency of the European Union

Outlook: targeted therapies on the increase

EUROPEAN MEDICINES AGENC

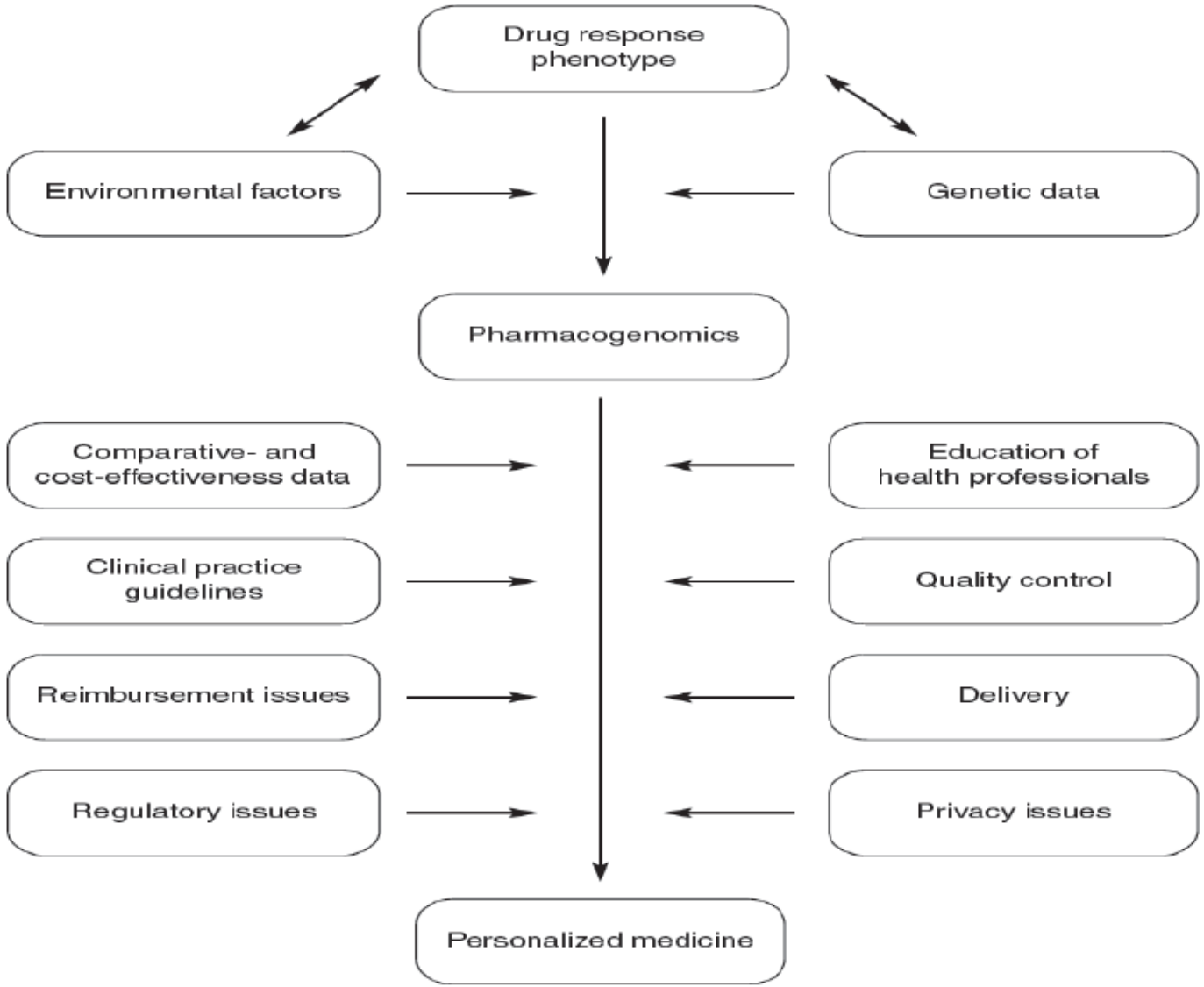
Figure 2: Number of medicinal products and ratio of medicinal products containing a genomic biomarker (gene) in their product label under "Therapeutic Indication" per year.



The number of pharmacogenomic biomarker in EU product label have been steady between 1999 and 2010 and since then gradually increasing in recent years. Initially, they have been intended for information only, progressing into becoming one of the important determinant for selection of patients likely to benefit from treatment and "more" individualised dose selection. Biomarker information may also be included in the labelling in case of negative selection (i.e., if the biomarker is used to select a population unlikely to respond) or in case of uncertainty about the value of the biomarker but where a negative selection is

Personalised Medicines: healthcare challenges

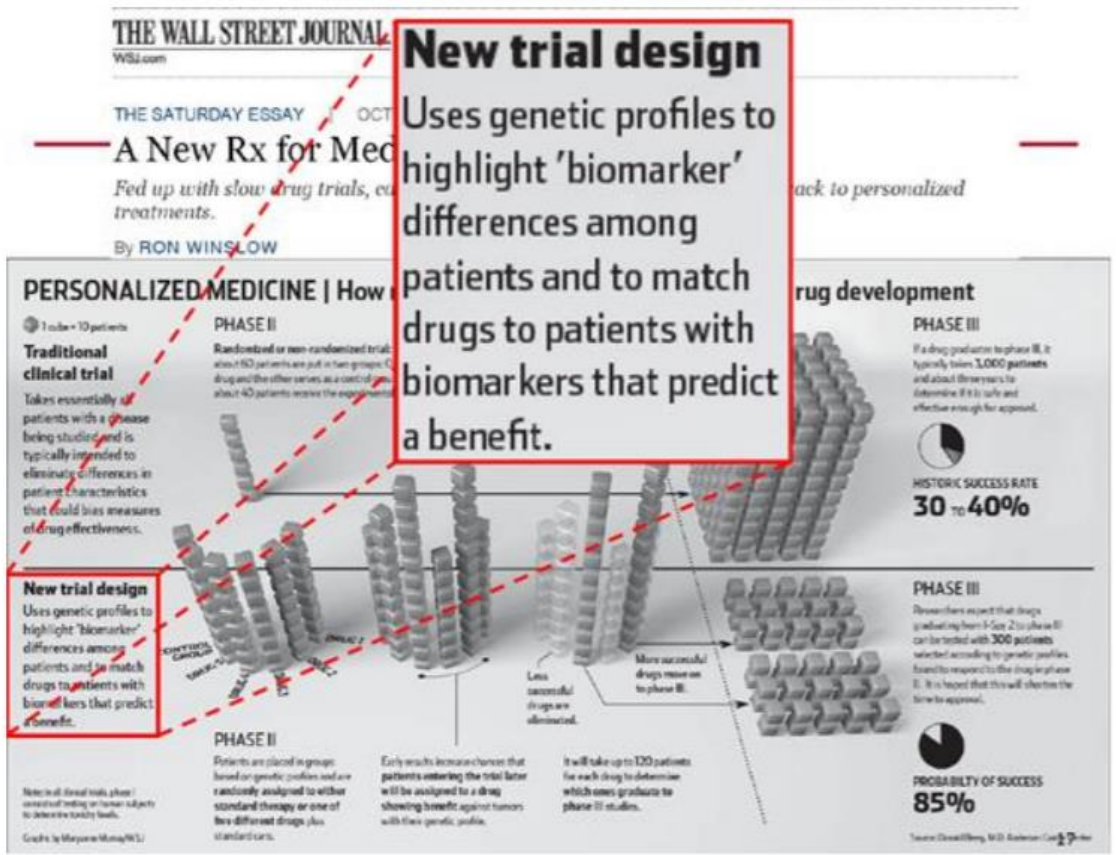
EUROPEAN MEDICINES AGENCY



P T. 2011 July; 36(7): 412-416, 419-422, 450. PMCID: PMC3171815 Pharmacogenomics in Clinical Practice Reality and Expectations, C. Lee Ventola, MS

Biomarkers and stratified medicines: more efficient clinical trials

EUROPEAN MEDICINES AGENCY



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- Understanding genomics
- Data security, privacy and access
- Frequently asked questions

Home › About Genomics England

About Genomics England

Genomics England, with the consent of participants and the support of the public, is creating a lasting legacy for patients, the NHS and the UK economy, through the sequencing of 100,000 genomes.

Useful links

Events
Upcoming talks, debates and seminars, plus videos of past events,

MIRAGGIO O SPERANZA ?

Precision Medicine: From Science to Value

Geoffrey S Ginsburg and
Duke Center for Applied Genomics & Precision Medicine, Duke University, Durham, NC 27708, Ph: 919 668 6210
Kathryn A Phillips
Center for Translational and Policy Research on Personalized Medicine, UCSF School of Pharmacy, San Francisco, CA 94118, Ph: 415 502 8271

Global Efforts to Develop Precision Medicine as a Science and Health Care Strategy

Worldwide, many efforts and initiatives are underway to create national implementation strategies for genomic medicine (Table 2); however, many of these efforts are being carried out in the absence of external collaboration, risking the duplication of efforts and slowing the pace of discovery and translation (31). **Globally, key barriers exist to implementing and integrating precision medicine technologies into health care practice include the absence of supporting IT infrastructure, lack of data standards and interoperability, insufficient decision support technology, and insufficient funding for translational health research. Policies to support progress in these areas will be critical to the adoption and integration of PM technologies into health care worldwide.**

Published in final edited form as: Health Aff (Millwood). 2018 May ; 37(5): 694–701. doi:10.1377/hlthaff.2017.1624.

IL BILANCIO

Medicina di precisione e personalizzata: le nuove sfide del sistema sanitario

Vantaggi e prospettive della medicina personalizzata e di precisione, per migliorare la capacità di praticare l'oncologia (in primis) nel modo più preciso e personalizzato possibile. Gli ostacoli sulla strada, la chiave vincente per indirizzare i trattamenti e quale dovrebbe essere il ruolo delle istituzioni

19 Ott 2018

Ettore Capoluongo

professore di Biochimica clinica e Biologia Molecolare Clinica – Direttore Unità di Diagnostica Molecolare e Genomica, Fondazione Policlinico Gemelli-IRCCS

Francesco Salvatore

Professore Emerito di Biochimica Clinica e Biologia Molecolare Clinica- Università Federico II Napoli, e CEINGE-Biotecnologie Avanzate, Napoli

La nuova frontiera dell'oncologia si chiama medicina di precisione che come dimostrano studi recenti è in grado di migliorare le percentuali di sopravvivenza a fronte di un'ottimizzazione della spesa per i farmaci grazie all'utilizzo delle terapie mirate solo nei pazienti che ne possono beneficiare, evitando inutili tossicità ed i ricoveri in ospedale.

I progressi nel campo della genomica sono resi possibili dal recente sviluppo di nuove piattaforme che consentono di effettuare il sequenziamento di un più ampio numero di geni rispetto ai metodi precedenti, con tempi ridotti per l'analisi e un aumento della sensibilità, con lo scopo di identificare le mutazioni corrispondenti a specifici bersagli molecolari su cui scegliere i farmaci mirati.

i Molecular Tumor Board che grazie alla loro esperienza, permettano una corretta interpretazione dei dati genetici e molecolari e la scelta della strategia terapeutica adeguata.

Le speranze

maggiore efficacia delle terapie

riduzione degli effetti avversi

riduzione dei costi

diagnosi rapida o anticipata

miglioramento nella gestione delle malattie

progettazione migliore degli studi clinici

sequenziamento massivo in parallelo (NGS)

screening di mutazioni “druggable” VERSUS sviluppo me-too o nuove molecole tra tanti tentativi

Lo stato dell'arte

Profilo beneficio rischio ancora incerto

Risorse ancora non sufficienti da destinare alle attività di implementazione tecnologica

Disomogeneo accesso nelle diverse regioni

Assenza tumor board strutturati

Definizione Ruolo in terapia rimane imprescindibile

Scarse risorse di bio-informatici

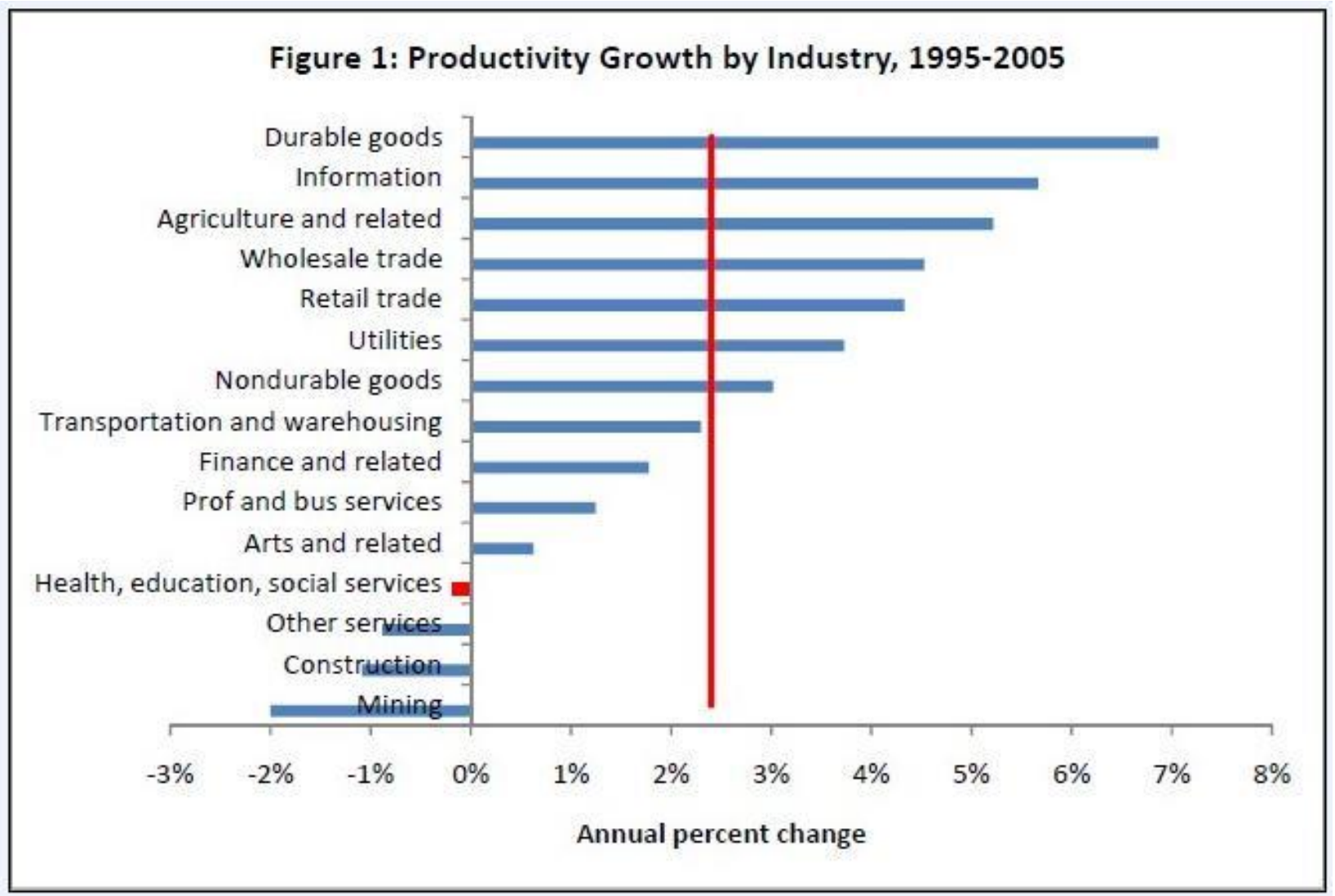
Scarsa integrazione ed evoluzione tecnologie di Intelligenza artificiale e di cartella clinica elettronica

I recenti **farmaci a bersaglio molecolare hanno determinato** crescita esponenziale dei costi

Non accordo su **metodologie di sequenziamento** standard per armonizzare test diagnostici

- *La muldidisciplinarietà da sola non garantisce appropriatezza*
- *Tempestivo accesso all'innovazione* versus **tempestivo accesso alla valutazione del ruolo in terapia dell'innovazione** come strumento per garantire percorsi di cura appropriati a tutti i pazienti che accedono ai servizi sanitari delle strutture pubbliche

La malattia di Baumol



27 lug 2017

TAG

Spesa sanitaria

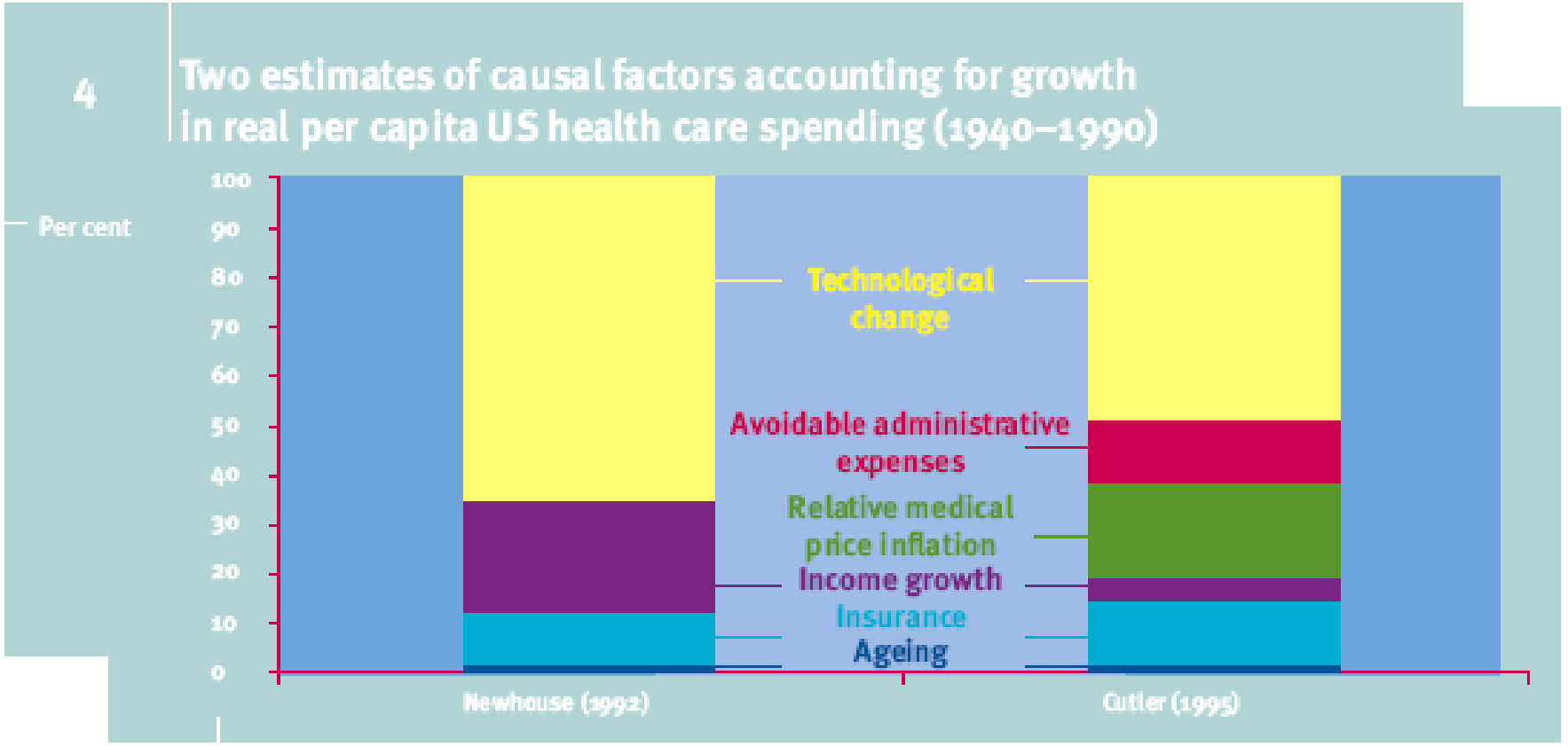
DAL GOVERNO

Sanità, dal 2000 la spesa per i dipendenti è crollata del 22% rispetto alle uscite totali del Ssn. Lo certifica la Ragioneria

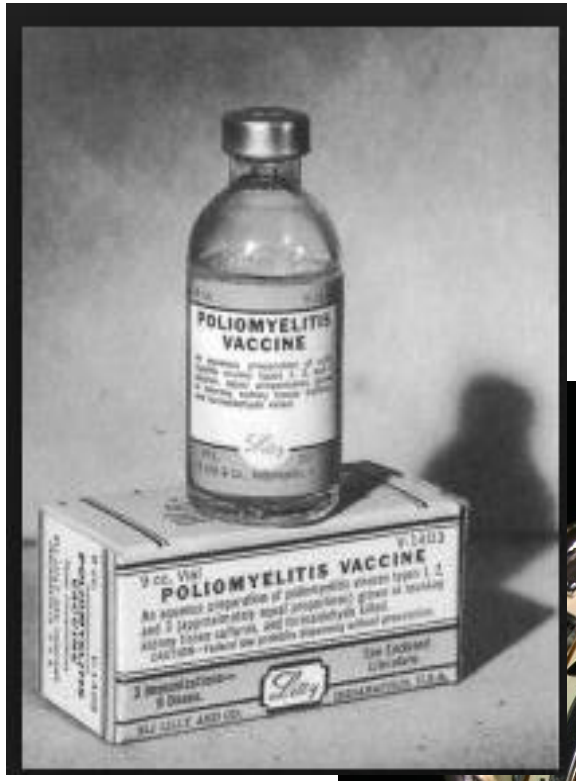
di *r.tu.*

PDF Il rapporto della Ragioneria sulla spesa sanitaria

Technological push



Source: Newhouse (1992) and Cutler (1995)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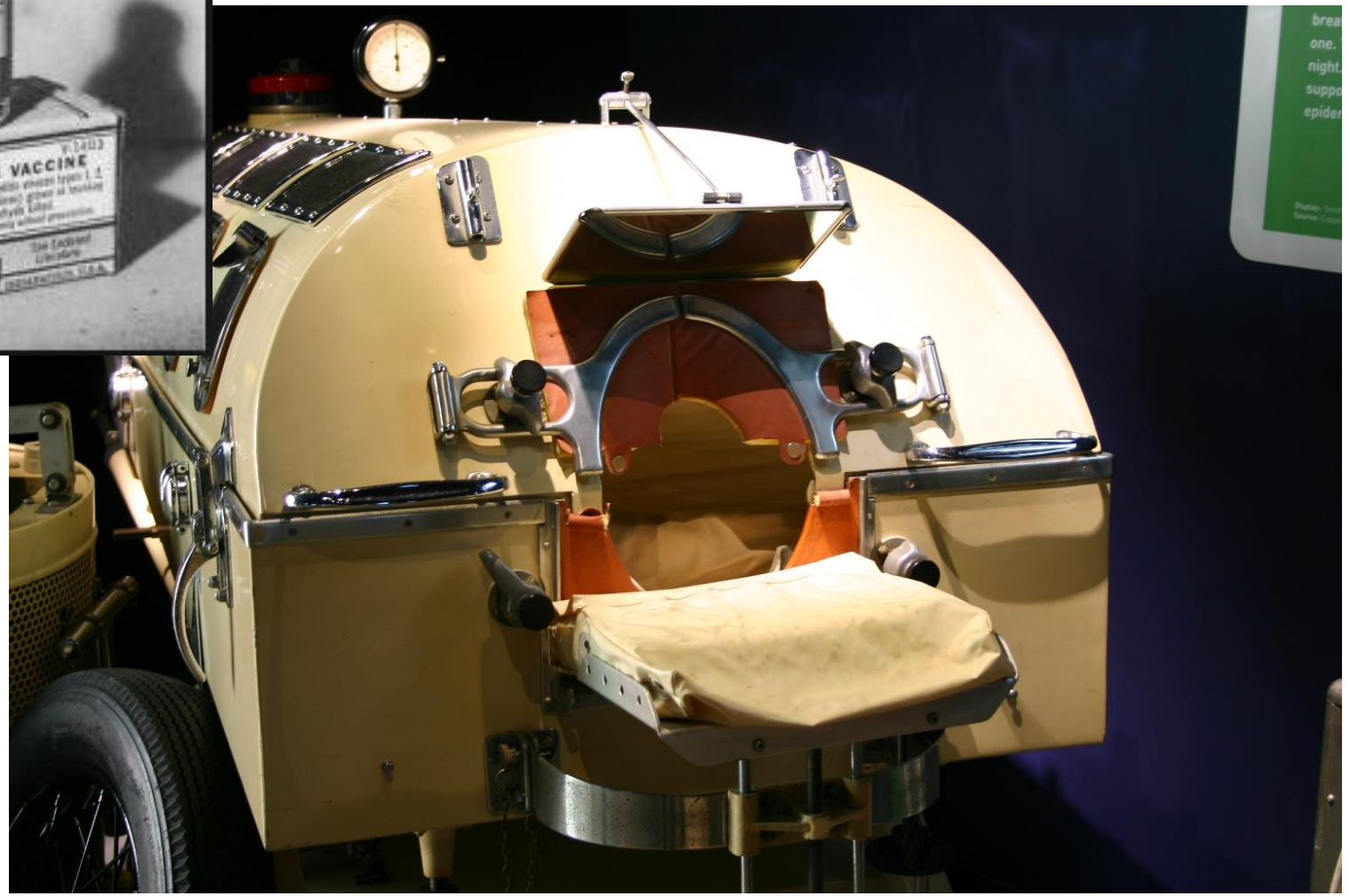
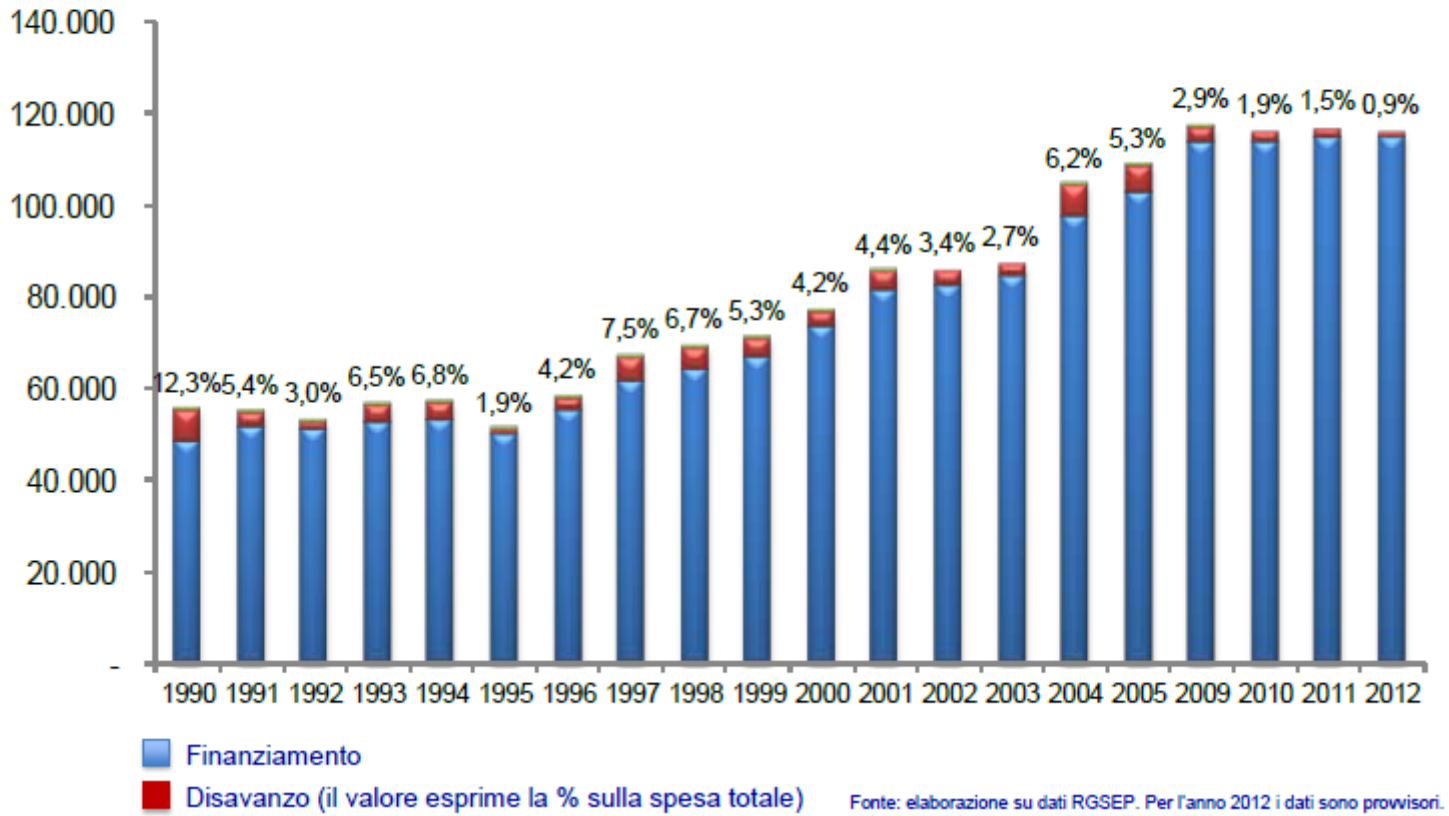


La sostenibilità del SSN

Spesa sanitaria pubblica in Italia (mln €): finanziamento e disavanzo (1990-2012)



Fonte: elaborazione su dati RGSEP. Per l'anno 2012 i dati sono provvisori.


RESEARCH
DEVELOPMENT

Richard Smith: Is precision medicine a fantasy?

July 13, 2018

Was Richard Smith wrong to call precision medicine a fantasy?

Earlier this year I spoke in a debate at the Cambridge Union against the chief executive of AstraZeneca and the motion that "This house needs new drugs," and I threw out the comment that "personalised or precision medicine is a fantasy." Several doctors had said that to me. After the debate Ruth March, head of precision medicine and genomics at AstraZeneca, told me that I was wrong; she invited me to visit her at the company in Cambridge so she and her team could show me that I'm wrong. I went last month. Was I wrong?

The old model of drug development and treatment led to huge numbers of patients being treated with drugs like antihypertensives, statins, and antidepressants, with many patients not benefiting directly, but the right patient means identifying those patients who have a much higher probability of benefiting directly. **This has led to the company spending a lot of resource examining advanced diagnostic methods and partnering with companies that have methods that can identify patients most likely to benefit— methods that are better, cheaper, or quicker than current methods**. AstraZeneca is not itself a diagnostic company and has no plans to become one.

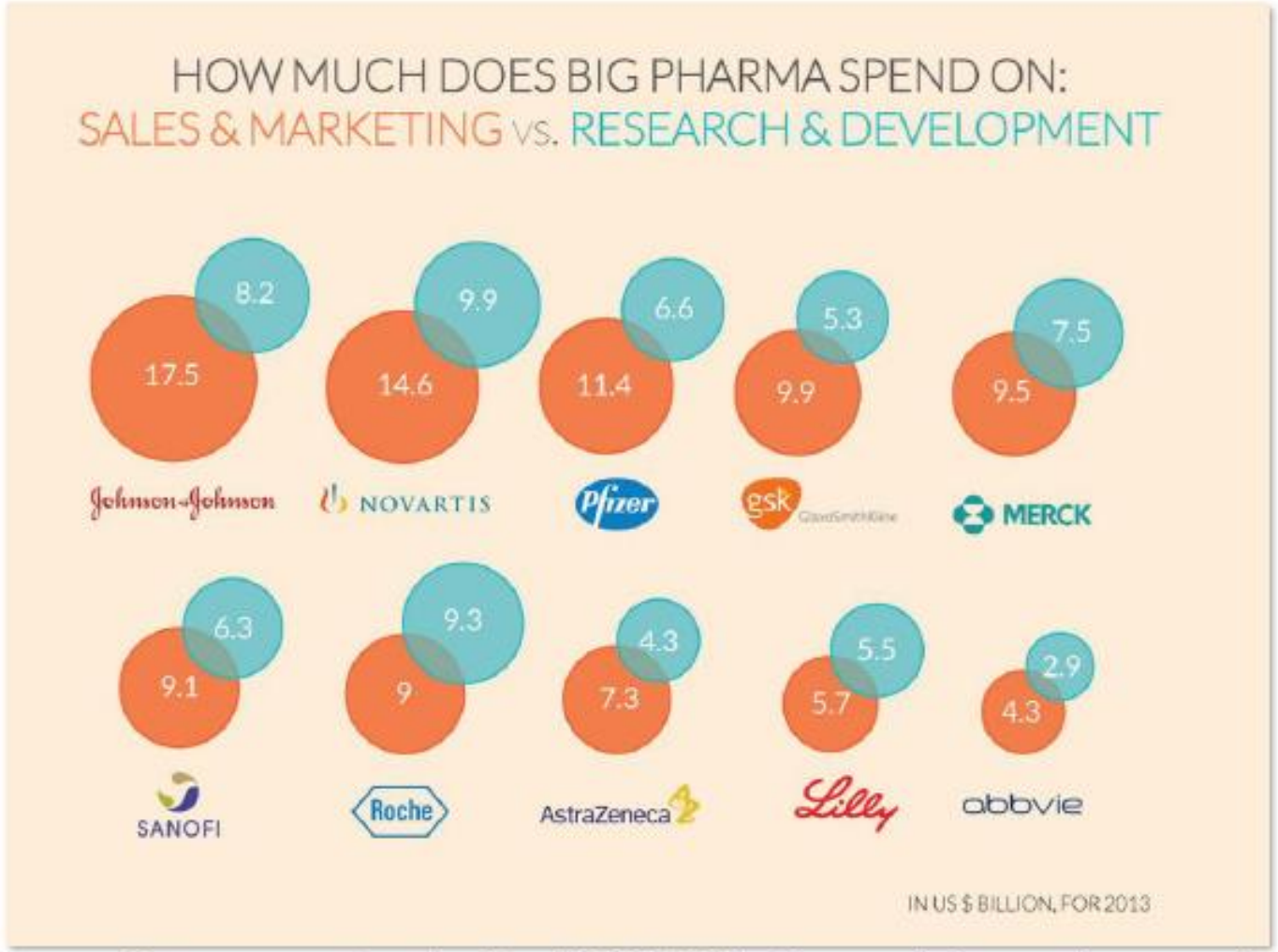
The right commercial potential means thinking of the commercial possibilities from early in drug development, and by the time a drug is considered for a phase III trial there must be "clarity around the patient population, the unmet medical need, differentiation versus standard of care, payer criteria for global reimbursement, competitive environment and sales projections."

Is all this transformation and collaboration working?

One measure of whether this transformation is working is the proportion of molecules moving from preclinical investigation to completion of phase III trials, and for AstraZeneca this has increased from 4% in 2005-10 to 19% in 2012-16. The company was below the industry average of 6% in 2005-2010 but is above the industry average of only 4% from 2003-15. (That is not, I recognise, comparing like with like, but it does show a substantial increase within AstraZeneca).

My response to March at the Cambridge debate was that I feared that even though there might be precise drugs they benefited only small numbers of patients at high cost. March emphasised to me that the cost of testing precise drugs is less than that of traditional drugs in that they didn't need such big trials. Soriot also emphasised in his speech at the Cambridge debate that drugs are relatively quickly off patent, which brings the cost down dramatically.

Le aziende farmaceutiche spendono (in media) più in marketing che in ricerca e sviluppo di nuovi farmaci



https://www.washingtonpost.com/news/wonk/wp/2015/02/11/big-pharmaceutical-companies-are-spending-far-more-on-marketing-than-research/?utm_term=.aafdad32870c – fonte_GLOBALDATA

Cinzia Colombo

I was wrong to argue that precision medicine is a fantasy in that increasing numbers of drugs are being produced using the methods of precision medicine. **At the moment, however, the drugs tend to be very expensive and used only in patients with severe disease.** Perhaps that will change as more drugs come through and some come off patent.

Richard Smith was the editor of The BMJ *until 2004.*

Competing interest: I'm grateful to all the people at AstraZeneca, who were charming, gave me time, and answered all my questions. I paid my own travel expenses and was treated by the company to one cup of coffee, which I'm confident (perhaps wrongly) has not corrupted me.

OSTACOLI SUPERABILI

Outlook | Published: 07 September 2016

Privacy: The myth of anonymity

Neil Savage

Nature **volume 537**, pages S70–S72 (08 September 2016) | Download Citation

It may not be possible to protect the identity of genomic data. But how much of a problem is that?

Image: Andrew Baker

Be clear on consent

Whatever the problem with privacy, the solution is unlikely to be technological, Erlich says. Techniques to encrypt data or disguise it with statistical noise are of limited value, he explains, because the more they protect privacy, the less useful they make the data. He thinks that a better approach is to rethink how privacy and consent are handled, and to treat the people who hand over their DNA with respect and honesty.

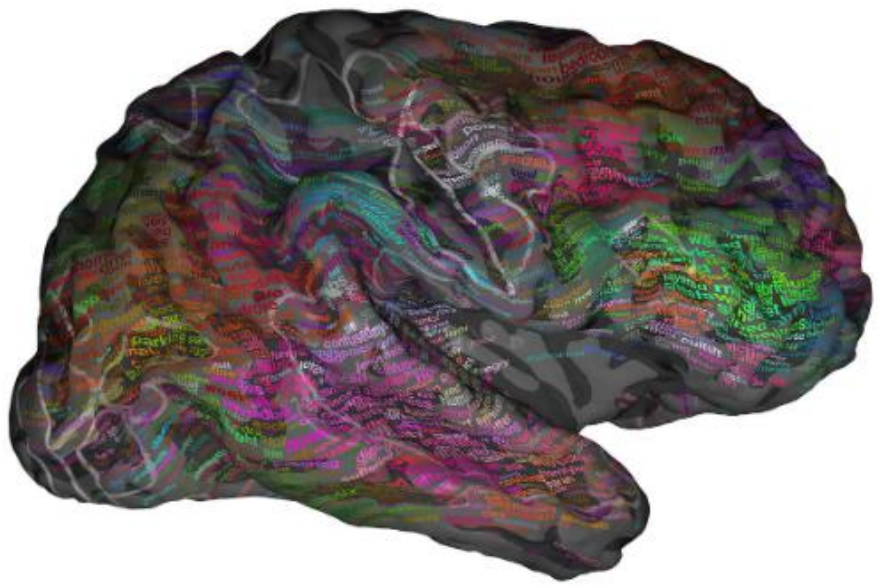
Instead, he argues, engaging with donors and spelling out the risks and benefits can change the privacy equation. “If you talk to people who have children with undiagnosed diseases, they would tell you: 'We would gladly forgo privacy in the interest of accelerated research'.”

Arrivano le macchine per leggere la mente

THE WALL STREET JOURNAL.

Scansioni cerebrali sempre più sofisticate si combinano con l'intelligenza artificiale per produrre strumenti in grado di tra testare la veridicità di quello che dite e, un giorno, forse, rendere le vostre conoscenze ed esperienze disponibili anche

di **Jerry Kaplan**



VOTA 1 VOTO

La risonanza magnetica per immagini (Mri), entrata nell'uso comune negli anni '80, ha reso visibile il cervello umano come mai prima. Per la prima volta, si è potuto vedere il tessuto cerebrale molle di un soggetto vivente, a un