

RESEARCH AND INNOVATION IN ITALY: FUTURE PERSPECTIVES ON POSSIBLE DEVELOPMENT

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Lilly

*140 anni di impegno
per migliorare la vita*



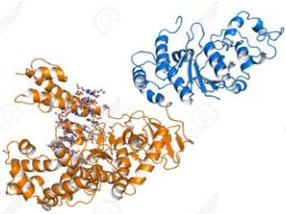
Public Declaration of transparency/interests*

The view and opinions expressed are those of the individual presenter and should not be attributed to AIFA/EMA

Interests in pharmaceutical industry	NO	Current	From 1 to 3 previous years	Over 3 previous years
DIRECT INTERESTS:				
1.1 Employment with a company: pharmaceutical company in an executive role	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> mandatory
1.2 Employment with a company: in a lead role in the development of a medicinal product	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> mandatory
1.3 Employment with a company: other activities	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
2. Consultancy for a company	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
3. Strategic advisory role for a company	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
4. Financial interests	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
5. Ownership of a patent	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
INDIRECT INTERESTS:				
6. Principal investigator	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
7. Investigator	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
8. Grant or other funding	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional
9. Family members interests	<input checked="" type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/> optional

*Luca Pani, in accordance with the Revised Conflict of Interest Regulations approved by AIFA Board of Directors (25.03.2015) and published on the Official Journal of 15.05.2015 according to EMA policy /626261/2014 on the handling of the conflicts of interest for scientific committee members and experts. .

The value of biomedical research



The majority of resources invested in research does not lead to a substantial improvement of public health.

Correctable weaknesses in the design, conduct, and analysis of biomedical and public health research studies can produce misleading results and waste valuable resources.



Innovation



“Innovation is the process of making improvement by introducing something new that should potentially yield a benefit for users”.

“Innovation in the field of medicinal products consists of a completely or partially new active substance or biological entity or combinations acting against a disease, relieving symptoms or preventing a disease, that can improve the quality of patient management and outcomes”.

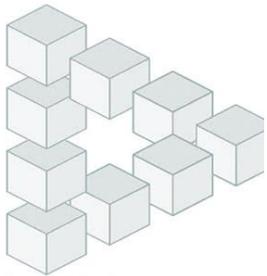
The Innovation Paradox in Health Care

The Costly Paradox of Health Care Technology

In every industry but one, technology makes things better and cheaper. Why is it that innovation increases the cost of health care?

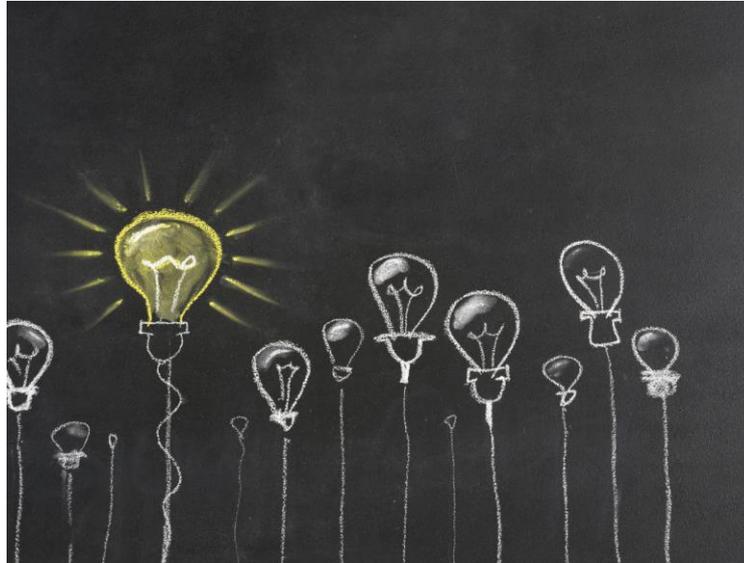
By Jonathan S. Skinner on September 5, 2013

New England Genetics Collaborative



THE ITALIAN WAY

RESEARCH

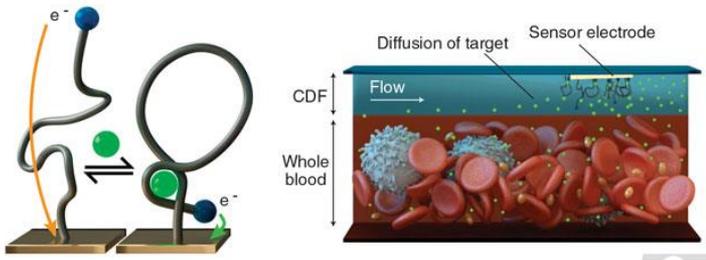


INNOVATION

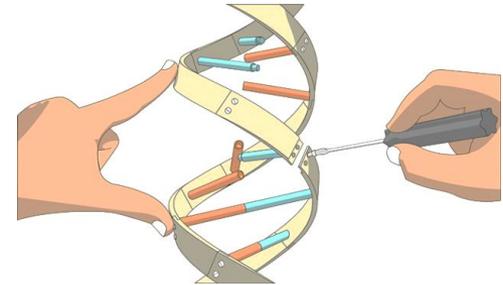


Something innovative?

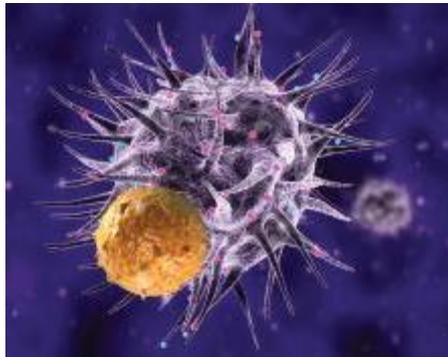
Sensor drugs



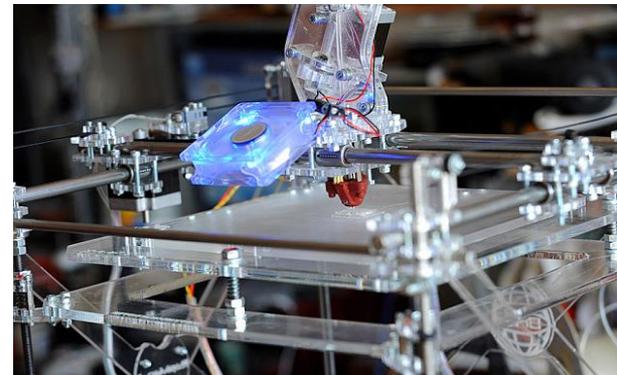
Genetic Editing



Immunotherapy



3D printed drugs



How to value innovation



A drug to be innovative must demonstrate an added therapeutic value (superior in terms of risk-benefit ratio) compared to the available alternative.

In any case, the favorable risk-benefit ratio must also be associated to a sustainable cost for the NHS.

from CTS 12 January 2015

ALGORITMO INNOVATIVITA'

0% 100%

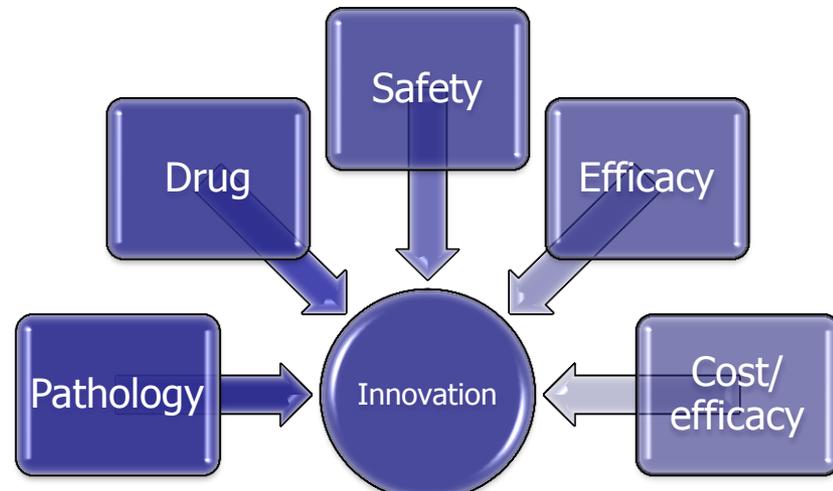
L'obiettivo dell'Algoritmo è quello di guidare, attraverso un percorso sistematico e trasparente, l'attività istruttoria di valutazione dell'innovatività dei farmaci condotta dagli Uffici preposti dell'Agenzia e, successivamente, informare le decisioni delle Commissioni AIFA.

La composizione dell'Algoritmo trae origine ed è in linea con le decisioni della Commissione Tecnico Scientifica (CTS) dell'AIFA (seduta del 12 gennaio 2015), che ha fissato alcune definizioni generali. Un farmaco per potersi veder attribuire l'innovatività deve dimostrare un valore terapeutico aggiunto rispetto alle alternative disponibili per il trattamento di una malattia grave. In particolare si definisce: i) valore terapeutico aggiunto, quando un farmaco dimostra una sostanziale superiorità in termini di rapporto rischio-beneficio rispetto alle alternative terapeutiche disponibili o quando è destinato al trattamento di una malattia o di una condizione clinica priva di alternative terapeutiche; ii) malattia grave, una malattia ad esito mortale oppure che conduca a ospedalizzazioni ripetute o che ponga il paziente in pericolo di vita o che causi disabilità permanente in grado di compromettere la qualità della vita. Resta inteso che, in ogni caso, al valore terapeutico aggiunto dovrà corrispondere anche un costo sostenibile per il Servizio Sanitario Nazionale (SSN).

Pertanto l'Algoritmo prevede una prima fase di valutazione del valore terapeutico aggiunto del medicinale mediante una analisi delle evidenze utili e di tutti gli elementi informativi necessari a supportarlo; al termine, l'Algoritmo infersce l'esito della valutazione di innovatività prendendo in considerazione sia la priorità del trattamento per il SSN, sia l'impatto economico e sociale, che è in grado di determinare. L'innovatività di un medicinale non si esaurisce esclusivamente nel suo valore clinico - terapeutico, che rappresenta una condizione necessaria ma non sufficiente, ma deve includere anche il valore economico - nella prospettiva più ampia di valutazione - che un medicinale può avere per i pazienti, per il SSN e per il sistema Paese nel suo complesso.

In conclusione, il percorso dell'Algoritmo condurrà - sulla base degli elementi istruttori - alla determinazione di tre possibili esiti: i) "farmaco innovativo", in presenza di un valore terapeutico aggiunto consistente, sul piano metodologico e della dimensione dei risultati; ii) "farmaco potenzialmente innovativo", laddove la valutazione del valore terapeutico aggiunto presenta elementi di incertezza legata a fattori che possono inficiare la validità interna ed esterna della sperimentazione clinica; iii) "farmaco non innovativo".

Un percorso di valutazione dell'innovatività così strutturata garantisce non solo la trasparenza del percorso decisionale, ma anche un'analisi critica e condivisa delle informazioni disponibili, utile a rendere il processo dinamico e rivalutabile nel tempo.



European context

NEW

Regulatory Agencies are working on possible collaborations and on valuable solutions to promote innovation in Europe.

The purpose is that of collaborating and exchanging information.

Some examples: The adaptive pathway;



New CTs regulation N. 536/2014;

Compassionate use programs;



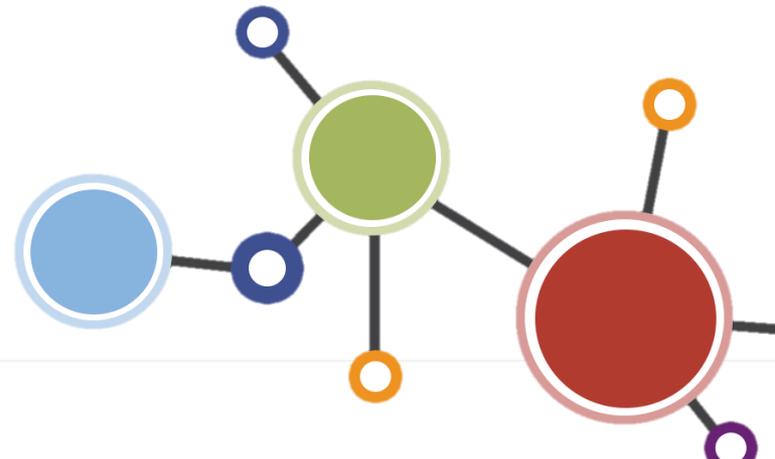
PRIME.



New CTs regulation N. 536/2014: what are the opportunities?

Italy can become the European hub for clinical research and the new Regulation on this subject is a great opportunity.

Ethics Committees rationalization and reformation of the Agency will enable the institution of a unique scientific-economic committee.



Our bet for the future?

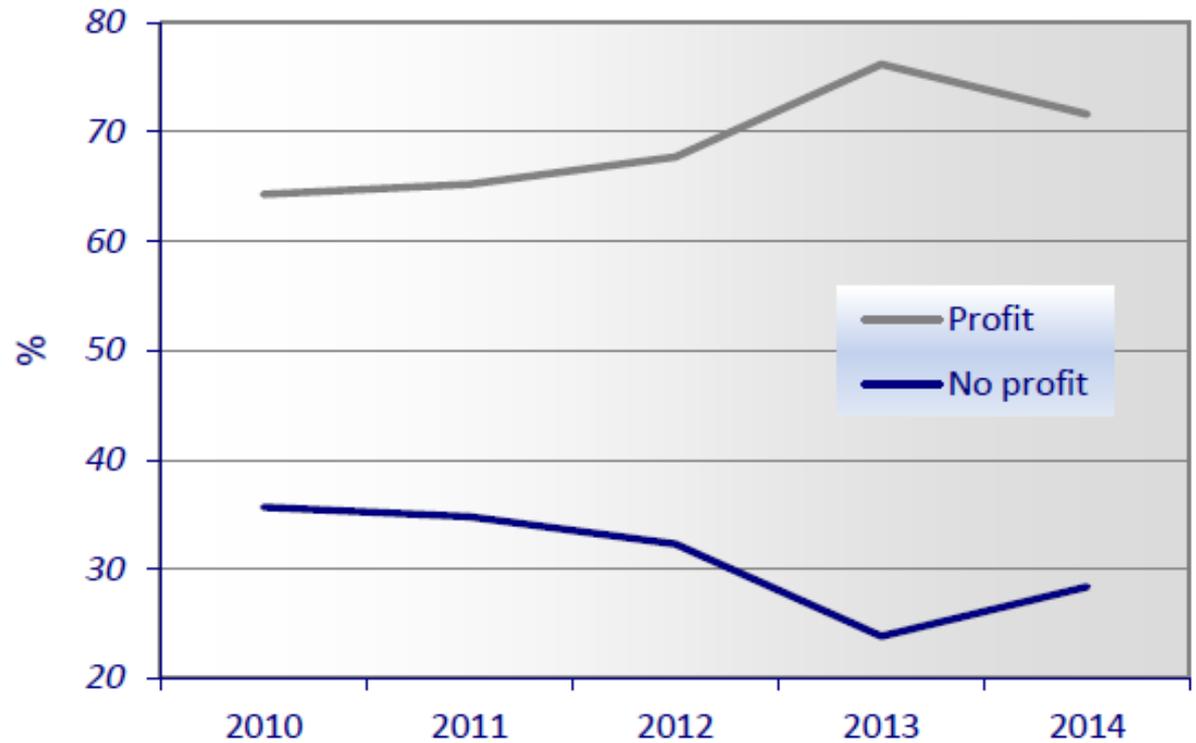


In the new global competitive environment the weight of the emerging economies continues to grow also relating to investments in R&D.

- For new drugs find a “European ” solution;
- “keep” research in our country;
- Studying specific actions to sustain research
- ...also the independent ones;
- ad hoc funds?



Research in Italy

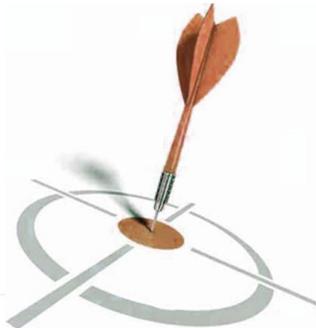




Promoting independent research: why?



- * Studying populations of patients neglected by profit research;
- * Solving doubts regarding on market drugs;
- * Studying the incidence of adverse events on wider populations;
- * Improving prescriptive appropriateness and adherence to therapy.

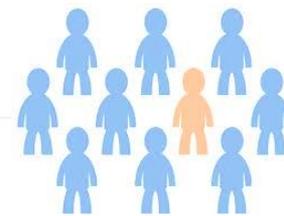


Clinical trials on rare diseases

Clinical trials for rare diseases, considering Promotors profit vs no profit, National and International

CT authorized in 2014: 592 of which 139 (23,5%) on rare diseases

Promotor	National SC (%)	International SC (%)	Total SC (%)
Profit	5 (20,0)	99 (86,8)	104 (74,8)
No profit	20 (80,0)	15 (13,2)	35 (25,2)
Total	25 (18,0)	114 (82,0)	139 (100,0)



Role of the Agency for independent research

The AIFA fund (Art. 48, Legge 326/2003)

- * The promotion of independent research on drugs represents one of the strategic tasks assigned to the Italian Medicines Agency by legislation.
- * It is an *ad hoc* fund, where pharmaceutical companies contribute with 5% of their yearly expenditure devoted to promotional initiatives (e.g., seminars, workshops, etc.) aimed at physicians.



Agenzia Italiana del Farmaco

AIFA

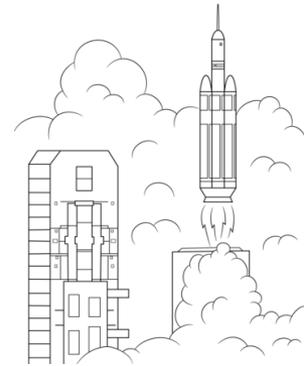
How AIFA uses the 5% fund

(Art. 48, Legge 326/2003)

- ✧ Orphan drugs for the treatment of rare diseases and drugs for non-responders.;
- ✧ Head to head comparison of drugs and therapeutic strategies;
- ✧ Strategies to improve the appropriateness of drug use and pharmacoepidemiology studies.
- ✧ Independent info center on drugs;
- ✧ Programme of active pharmacovigilance.



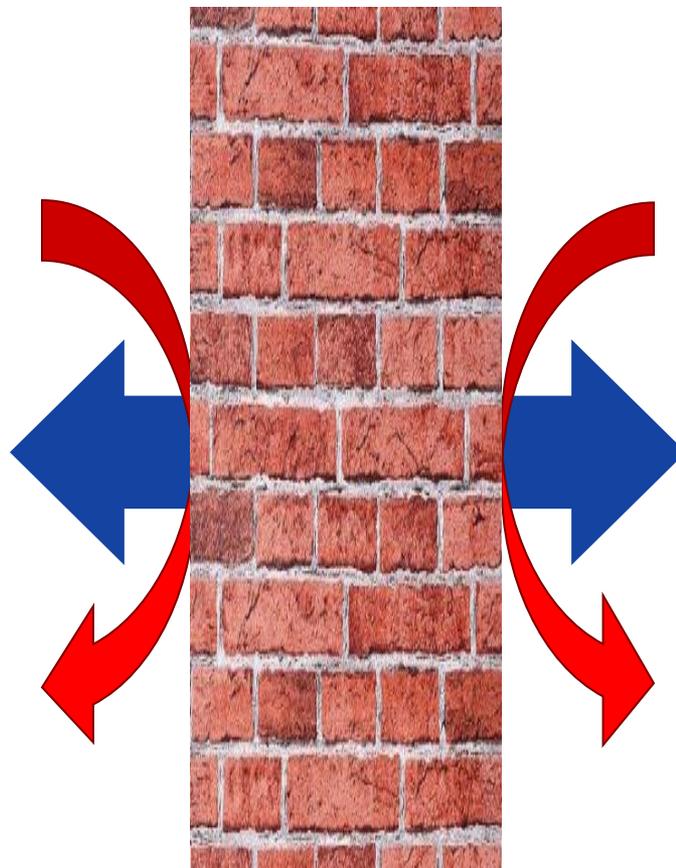
Launching Independent Research



- * Selection process *top down*: one/two areas;
- * One step procedure;
- * Evaluation of the protocols by independent Italian and foreigner experts;
- * Allocation of funding per area.

Public-private partnership

Regulatory
Agency



Pharmaceutical
companies



Sustainability should ensure innovation and balance between investments and costs

53 FDA approved new drugs (2014)

7.000 New drugs under development in the world

12 Potential blockbusters for the next 5 years

1,1 B\$ Italian Innovative Drugs Funds for the year 2015 - 2016

A new ***Governance*** is deemed necessary!

Conclusions



The challenge of Independent clinical research represents, in Italy, a great opportunity for development and a model for Europe.

Access to Innovation:

Universalism?

or

“Selective Universalism?”

