

Giuseppe Recchia, Direttore Medico & Scientifico



Terapie Avanzate

Un Successo Made in Italy

**Terapia Genica
ex vivo**

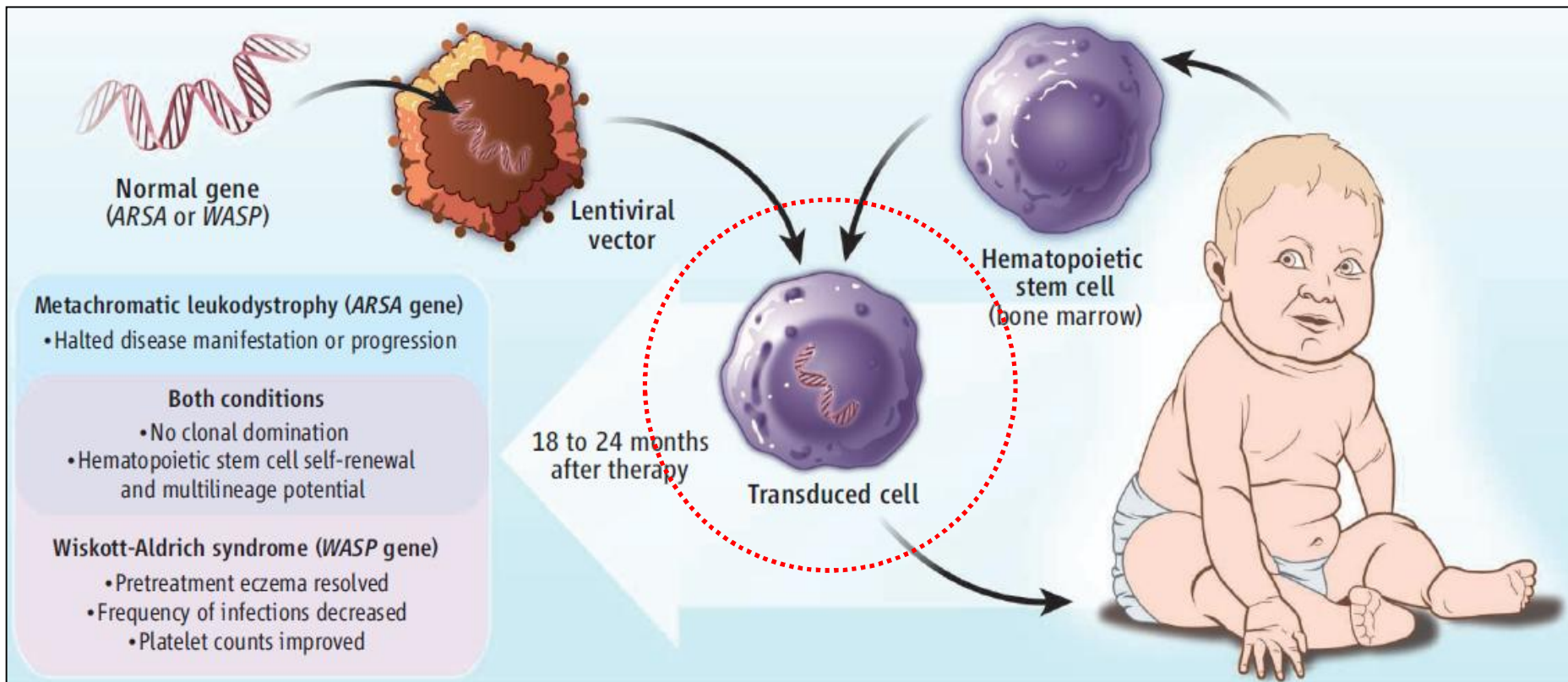
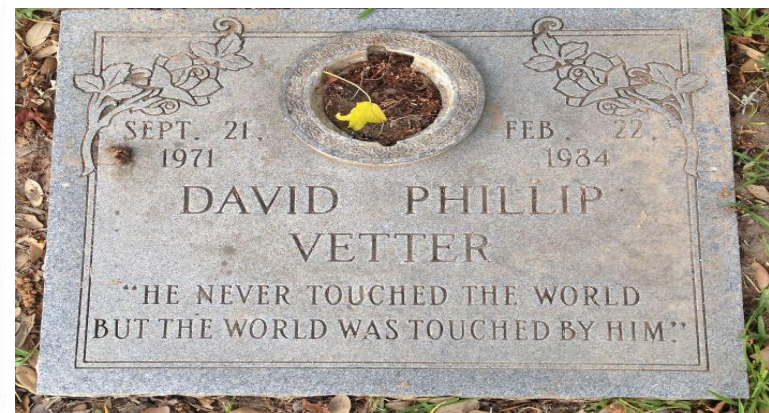


Roma, 25 ottobre 2016

27 maggio 2016



BAYLOR COLLEGE OF MEDICINE ARCHIVES



Pharma e Terapie Avanzate

Pharma Invests in Gene Therapy

Pharmaceutical companies have stepped up gene therapy development in the wake of the approval of Glybera in 2012.

BioPharm International
Volume 26, Issue 8

When the European Medicines Agency granted approval for Glybera (alipogene tiparvovec)—the first gene therapy—
—in November 2012, it was a milestone for gene therapy. It
encouraged investment and development across the industry.
Elemer Piros, managing director and senior advisor at Burrill
Securities, in a May 16, 2013 podcast, "Why is the pharmaceutical industry
<http://www.burrillreport.com/>

In recent months, pharmaceutical companies have stepped up gene therapy development on their respective websites.

Perché?

Come?

The Next Big Thing !

Why is the pharmaceutical industry investing in Cell and Gene Therapy?

Dr Laurent Jespers 17th October 2016

Over the years scientific discoveries have spurred the pharmaceutical industry to look at new and innovative ways to treat disease. Our understanding of disciplines such as molecular and cell biology, medicinal chemistry and recombinant DNA technologies have led to the discovery and development of small molecules and biopharmaceutical medicines that are widely used today.

Now, outstanding advances in genetics, regulatory pathways and system biology are playing a key role in fundamentally changing our understanding of human biology. With up to three new genetic causes of rare diseases being identified every week and the emergence of novel technologies, such as cell and gene therapy, we are moving to a period where one-time durable treatments addressing the cause of the disease (at gene level) rather than the consequences (symptoms) are becoming a reality for severely affected patients.

Come? 2010, da Closed ad Open Innovation ...



1

2.2010

Discovery Partnership with Academia - DPAc

11.2010

4



10.2010

3

nature publishing group

PERSPECTIVES

POINT/COUNTERPOINT

The Future Is Much Closer Collaboration Between the Pharmaceutical Industry and Academic Medical Centers

P Vallance¹, P Williams¹ and C Dollery¹

A reader of newspaper articles and editorials in some medical journals might conclude that relationships between pharmaceutical companies and academic clinical investigators are dominated by mistrust and the desire of academics to keep industry at a distance from the high moral ground of academia. Fortunately, that is not a correct analysis of a complex situation, but even the perception is an impediment to the pressing need for the parties to work very closely together with a shared desire to improve human health by discovery of new medicines for which the unmet needs, particularly in an aging population, are obvious.

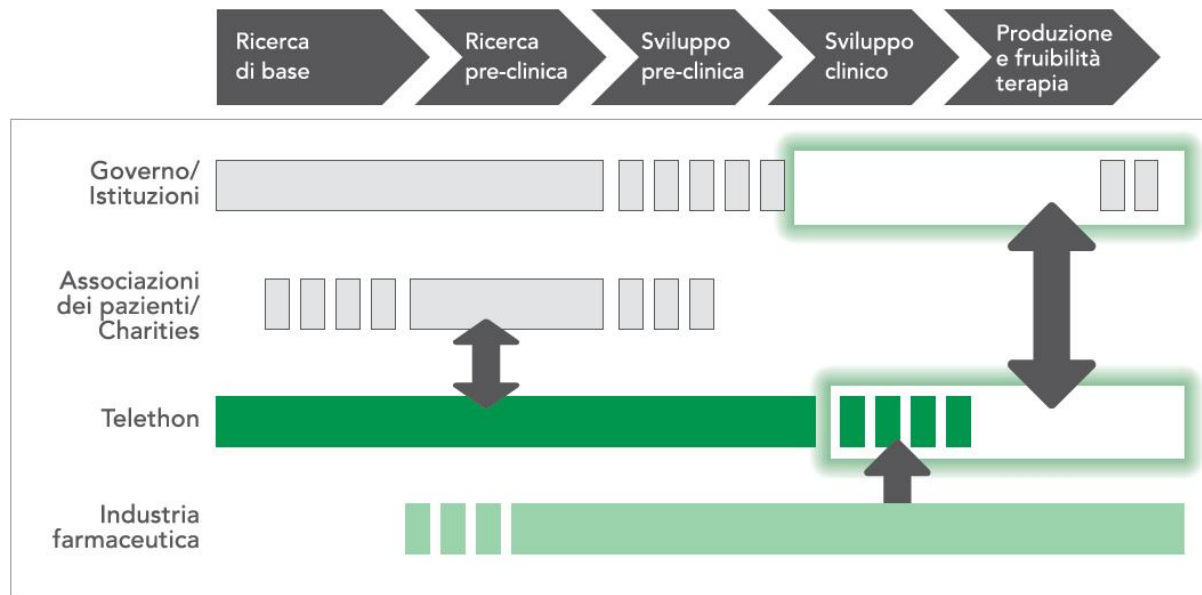
physiological control mechanisms in living animals and humans, and this in turn led to the emergence of flourishing academic departments of pharmacology and clinical pharmacology. James Black developed β-adrenergic blocking drugs with the treatment of angina in mind and the opportunity provided by these drugs found applications in clinical situations varied as tremor and heart failure. Many academic scientists became advisers to the pharmaceutical companies, but the information about new approaches to treatment of disease, promising chemical leads, and early clinical results was regarded as highly confidential for commercial reasons ranging from patent pro-

among the first to discover information did not readily exploit useful new medicines. A biological lack of knowledge of the new targets and their mechanisms of action. In many, to the etiology of a disease process. A company might have a biologist working on a new target. The information was in the public domain, it was likely to be exploited by hundreds of academic scientists, resulting in much more rapid expansion of knowledge. However, the academic scientific leaders in a new area often patented their discoveries, and not a few established small biotechnology companies to exploit them. Early drug dis-

2

5.2010

Come? Nuovo modello di R&D collaborativi



Alleanza Strategica



1. ADA – SCID
2. Leucodistrofia Metacrom.
3. Sindrome Wiskott Aldrich
4. Beta Talassemia
5. Leucodistrofia globoide
6. Mucopolisaccaridosi 1
7. Granulomatosi cronica



3



10.2010

Come? Sviluppo & Delivery

Sperimentazione Clinica

Uso Terapeutico
Composto Sperimentale

Valutazione Post
Autorizzativa - Registro

Produzione

Supply Chain

Processo Regolatorio

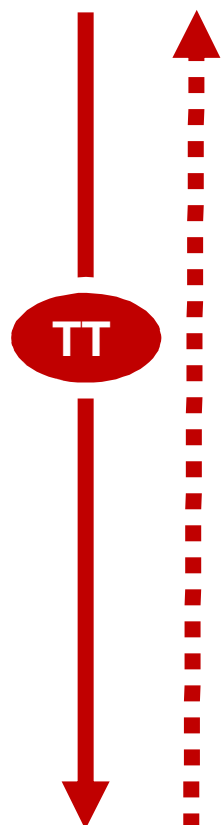
Registrazione

Prezzo & Rimborso

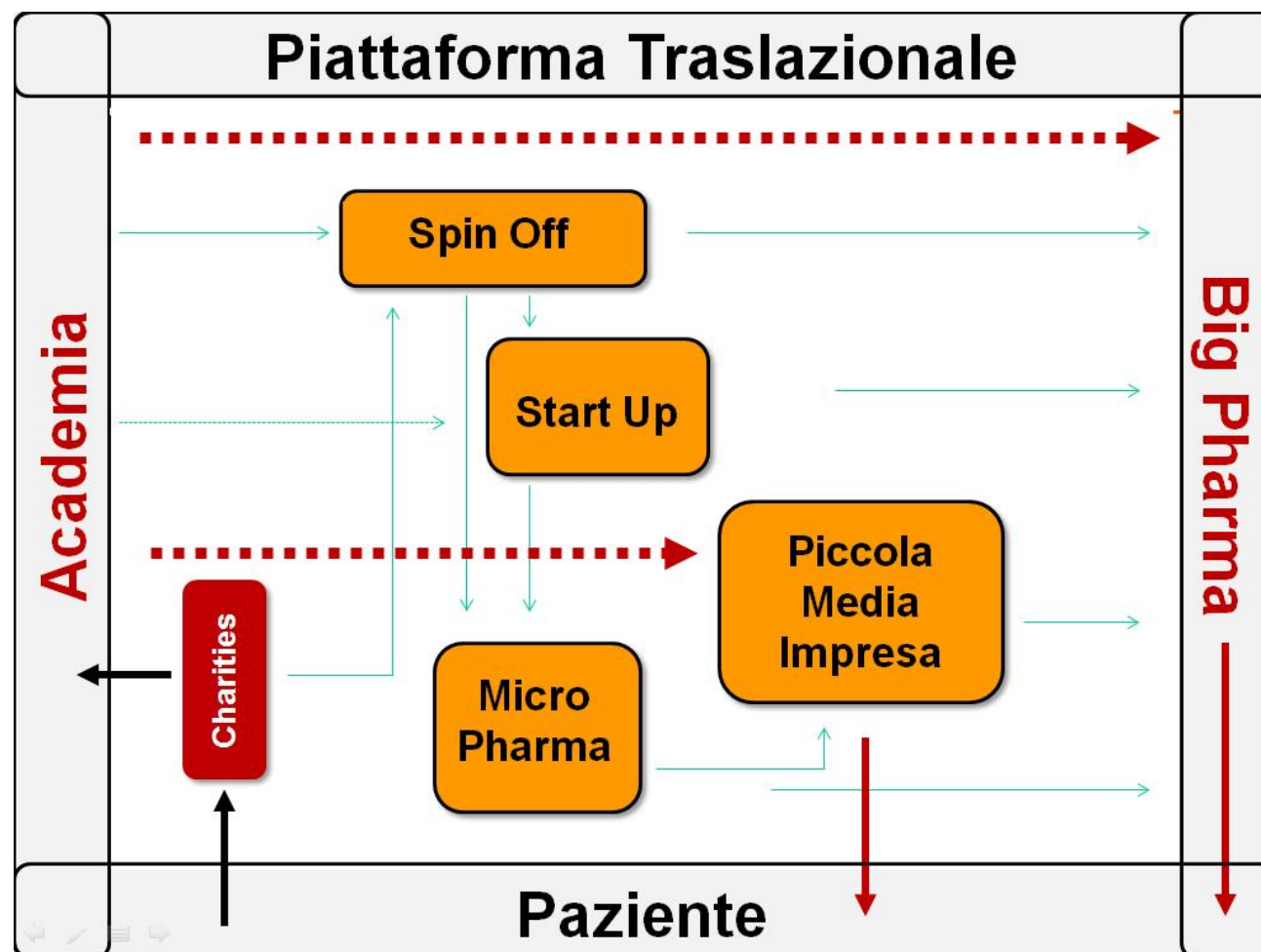
- **01.5.2015 – EMA file**
- **01.4.2016 – EMA Opinion**
- **27.5.2016 – EU Commission**
- **01.8.2016 – AIFA P&R cl.H**

2016 ed oltre - Nuove Terapie Avanzate in Italia...

- Accademia



- Impresa



SUN, UniFederico II, UniSA → 25 - 27.10.2016

UniPA, UniCT, UniME → 23 - 25.11.2016

Conclusione

1. Nuova tecnologia per la salute
2. Nuovo modello R&D
3. Ricerca Italia
4. Pharma e Terapie Avanzate

Formiche @formicheneews · Jun 27

Oggi parliamo di #Strimvelis, la prima #TerapiaGenica per i pazienti affetti da ADA-SCID #CronacaDiUnPrimatItaliano

Paolo Messa, GSK, Fondazione Telethon and M.Di Domenico



Giuseppe Recchia @G__Recchia · Jun 27

Conference @formicheneews. Sir A. Witty: with #GeneTherapy, Italy has not just discovered a new therapy, Italy has discovered a new industry



13



16

