



Forum Risk Management

obiettivo sanità & salute

18

21-24 NOVEMBRE 2023
AREZZO FIERE E CONGRESSI

Lorenzo Giovanni Mantovani

Direttore Centro Studi Sanità Pubblica, Università di Milano

Quali terapie avanzate nel prossimo futuro

Lorenzo G Mantovani
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IRCCS Istituto Auxologico Italiano

- Contesto
 - Pazienti
 - Rari e gravi
 - Fragili
 - Tecnologie
 - Cura
 - Assistenza
- Valore
 - Sanitario
 - Sociale

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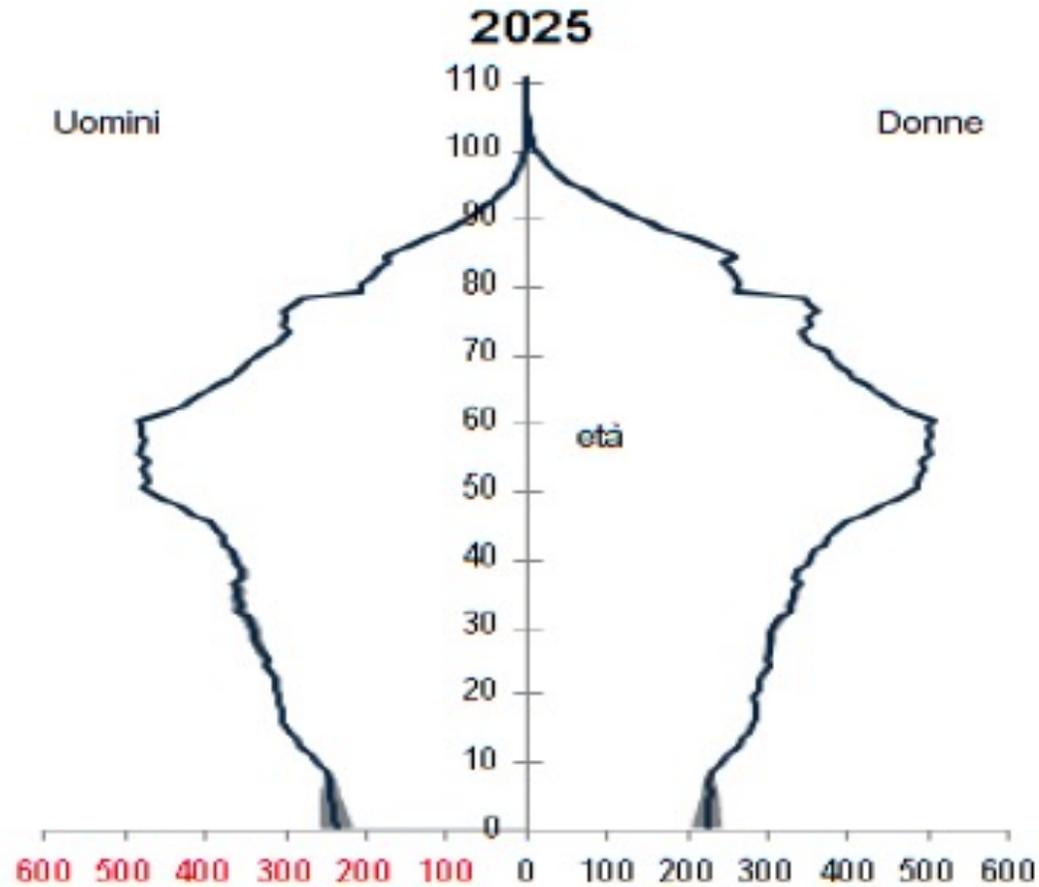
ADA-SCID - 'bubble boy'

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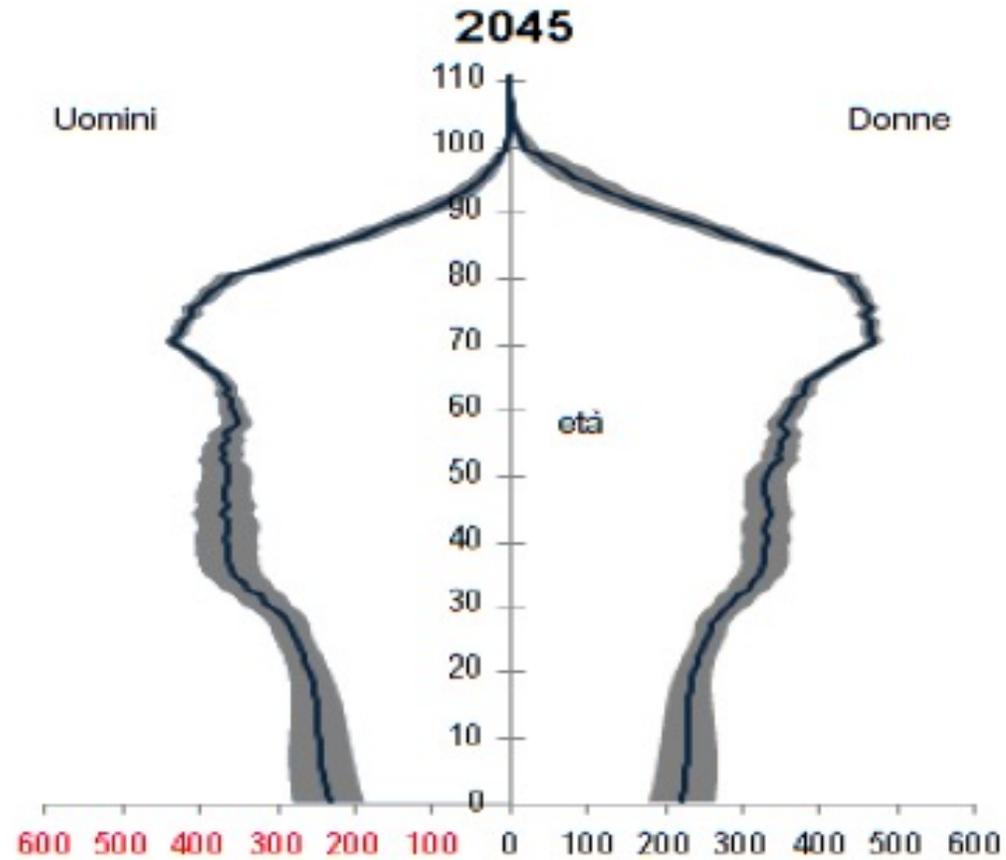


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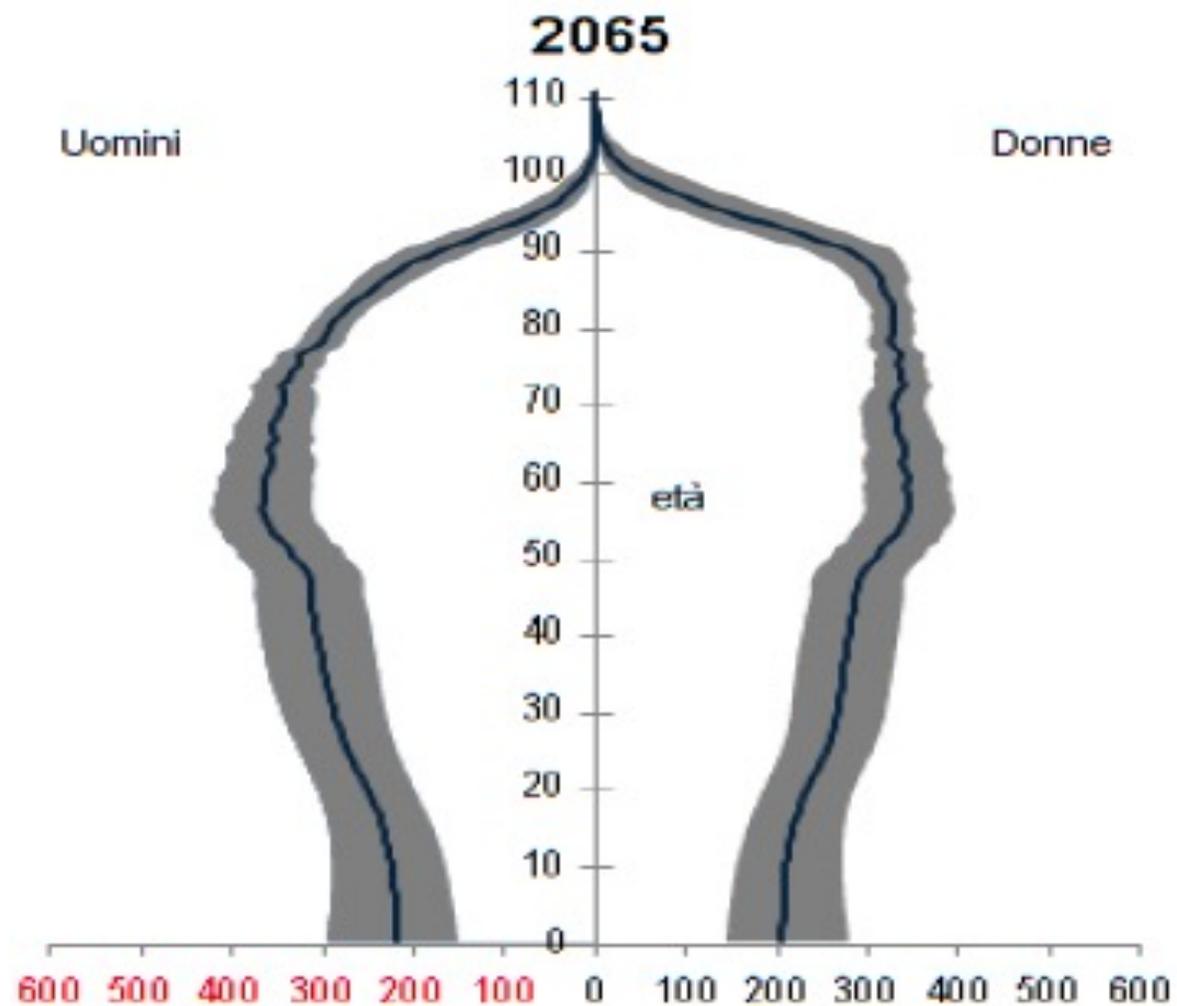
Istat, Il Futuro Demografico del Paese



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Global, regional, and national disability-adjusted life-years (DALYs) for 359 diseases and injuries and healthy life expectancy (HALE) for 195 countries and territories, 1990–2017: a systematic analysis for the Global Burden of Disease Study 2017



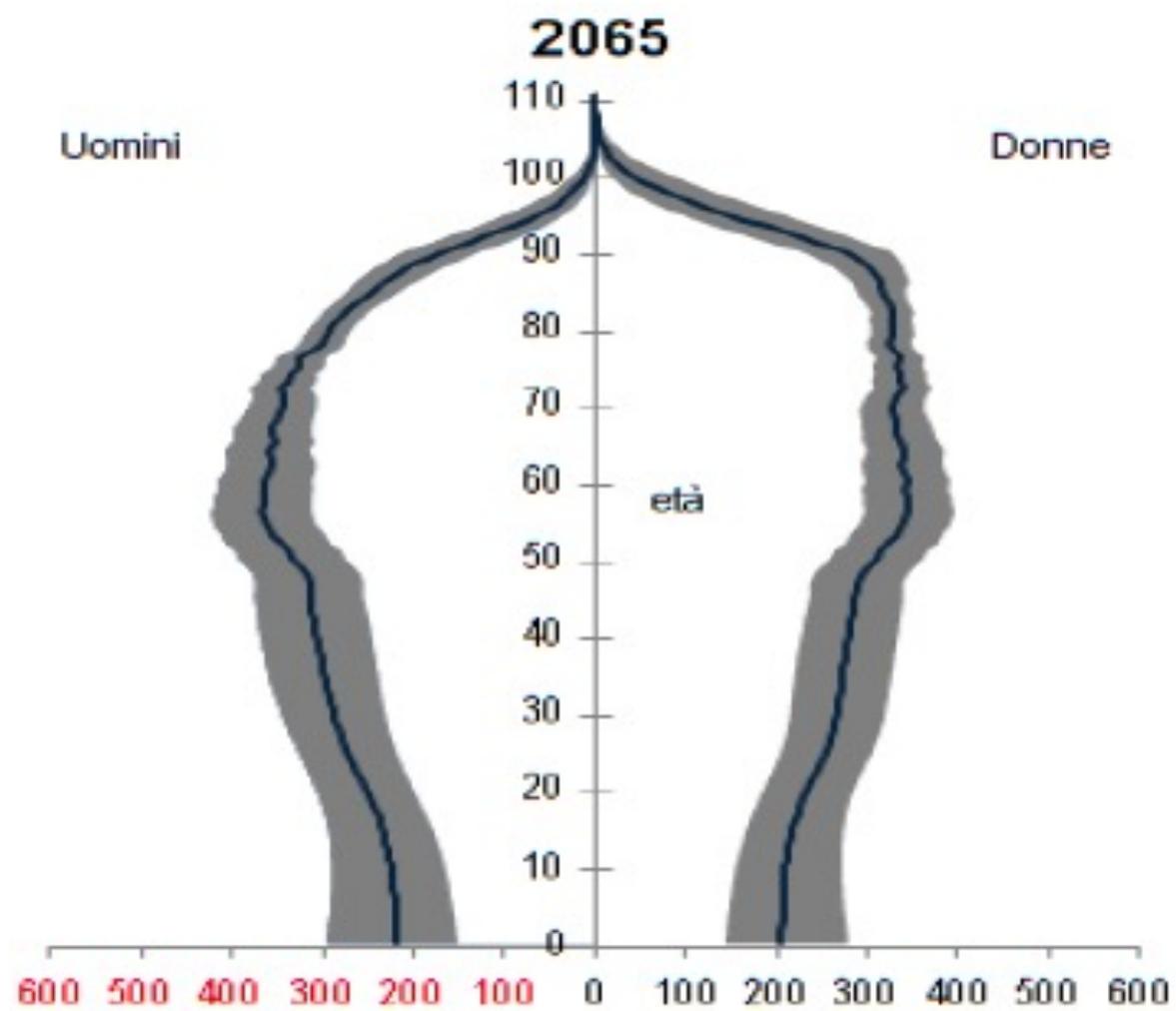
GBD 2017 DALYs and HALE Collaborators*



Summary

Background How long one lives, how many years of life are spent in good and poor health, and how the population's *Lancet* 2018; 392: 1859–922

Interpretation With increasing life expectancy in most countries, the question of whether the additional years of life gained are spent in good health or poor health has been increasingly relevant because of the potential policy implications, such as health-care provisions and extending retirement ages. In some locations, a large proportion of those additional years are spent in poor health. Large inequalities in HALE and disease burden exist across countries in different SDI quintiles and between sexes. The burden of disabling conditions has serious implications for health system planning and health-related expenditures. Despite the progress made in reducing the burden of communicable



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- Effetto Baumol

There's a possible cure for 'bubble boy' disease. It will cost \$665,000



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Klaus Miesenberger
Deborah Fels
Dominique Archambault
Petr Peňáz
Wolfgang Zagler (Eds.)

LNCS 8548

Computers Helping People with Special Needs

14th International Conference, ICCHP 2014
Paris, France, July 9–11, 2014
Proceedings, Part II

Assistive Device

- *"... qualsiasi prodotto esterno (dispositivo, apparecchiatura, strumento o software), di produzione specializzata o di comune commercio, il cui scopo primario è mantenere o migliorare il funzionamento e l'indipendenza della persona e in tal modo favorire il suo benessere...»
(def EASTIN)*

... customized



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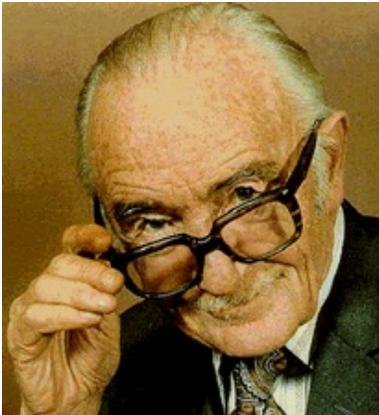
Principles

“All effective treatments should be given free”

Archie Cochrane 1971

“All cost effective treatments should be given free”

Alan Williams, 1997



Cost of care and quality of life for patients with hemophilia complicated by inhibitors: the COCIS Study Group

Alessandro Gringeri, Lorenzo G. Mantovani, Luciana Scalone, and Pier Mannuccio Mannucci, for the COCIS Study Group

2358

BLOOD, 1 OCTOBER 2003 • VOLUME 102, NUMBER 7

Table 4. Health care costs

Resource	Cost per patient per month, in euros	% of total cost
rFVIIa	8491.9	47.3
rFVIII	3174.2	17.7
Human plasma–derived FVIII	3077.1	17.2
Activated prothrombin complex concentrate	2982.0	16.6
Visits, surgeries, and hospitalizations	209.9	1.2
Total	17935.1	100

ORIGINAL ARTICLE

Anti-Inhibitor Coagulant Complex Prophylaxis in Hemophilia with Inhibitors

Cindy Leissinger, M.D., Alessandro Gringeri, M.D., Bülent Antmen, M.D.,
Erik Berntorp, M.D., Chiara Biasoli, M.D., Shannon Carpenter, M.D.,
Paolo Cortesi, M.Sc., Hyejin Jo, M.S., Kaan Kavakli, M.D., Riitta Lassila, M.D.,
Massimo Morfini, M.D., Claude Négrier, M.D., Angiola Rocino, M.D.,
Wolfgang Schramm, M.D., Margit Serban, M.D., Marusia Valentina Uscatescu, M.D.,
Jerzy Windyga, M.D., Bülent Zülfikar, M.D., and Lorenzo Mantovani, D.Sc.

The cost of prophylaxis for patients who have hemophilia without inhibitors is 2.4 to 3.1 times as high as the cost of on-demand therapy.³⁰ Similarly, the cost of AICC prophylaxis in our study was 2.4 times as high as that of on-demand therapy (\$493,633 vs. \$205,549, per patient, based

Cost-Effectiveness and Budget Impact of Emicizumab Prophylaxis in Haemophilia A Patients with Inhibitors

Paolo Angelo Cortesi¹ Giancarlo Castaman² Gianluca Trifirò³ Simona Serao Creazzola⁴
Giovanni Improta⁵ Giampiero Mazzaglia¹ Angelo Claudio Molinari⁶ Lorenzo Giovanni Mantovani¹

What is known about this topic?

- Emicizumab, a bispecific antibody administered subcutaneously once a week, has shown a decrease in the annualised bleeding rate of haemophilia A patients with inhibitors when compared with on-demand or prophylaxis treatment with bypassing agents. However, no economic evaluations assessing the value and sustainability of emicizumab prophylaxis have been performed in Europe.

What does this paper add?

- The current work assesses the lifetime costs and effects of emicizumab prophylaxis and of prophylaxis with bypassing agents, from the Italian National Health Service point of view. The study results identified emicizumab prophylaxis as the cost-saving treatment for haemophilia A patients with inhibitors. Furthermore, the current study estimated a significant reduction of the health care budget associated to the use of emicizumab prophylaxis, making this new treatment a sustainable and convenient health care option.

ORIGINAL ARTICLE

Gene Therapy with Etranacogene Dezaparvovec for Hemophilia B

S.W. Pipe, F.W.G. Leebeek, M. Recht, N.S. Key, G. Castaman, W. Miesbach, S. Lattimore, K. Peerlinck, P. Van der Valk, M. Coppens, P. Kampmann, K. Meijer, N. O'Connell, K.J. Pasi, D.P. Hart, R. Kazmi, J. Astermark, C.R.J.R. Hermans, R. Klamroth, R. Lemons, N. Visweshwar, A. von Drygalski, G. Young, S.E. Crary, M. Escobar, E. Gomez, R. Kruse-Jarres, D.V. Quon, E. Symington, M. Wang, A.P. Wheeler, R. Gut, Y.P. Liu, R.E. Dolmetsch, D.L. Cooper, Y. Li, B. Goldstein, and P.E. Monahan

RESULTS

The annualized bleeding rate decreased from 4.19 (95% confidence interval [CI], 3.22 to 5.45) during the lead-in period to 1.51 (95% CI, 0.81 to 2.82) during months 7 through 18 after treatment, for a rate ratio of 0.36 (95% Wald CI, 0.20 to 0.64; $P < 0.001$), demonstrating noninferiority and superiority of etranacogene dezaparvovec as compared with factor IX prophylaxis. Factor IX activity had increased from baseline by a least-squares mean of 36.2 percentage points (95% CI, 31.4 to 41.0) at 6 months and 34.3 percentage points (95% CI, 29.5 to 39.1) at 18 months after treatment, and usage of factor IX concentrate decreased by a mean of 248,825 IU per year per participant in the post-treatment period ($P < 0.001$ for all three comparisons). Benefits and safety were observed in participants with pre-dose AAV5 neutralizing antibody titers of less than 700. No treatment-related serious adverse events occurred.

Il futuro?

**Stem Cell Therapy BRT-DA01
Demonstrates Positive Safety, Feasibility in
Early-Stage Study of Parkinson Disease**

www-neurologylive.com

Sto diventando vecchio...

- «Il sistema sanitario fallirà per il trattamento del rischio cardiovascolare e per il mal di stomaco»
- «Il sistema sanitario fallirà per questi nuovi farmaci che hanno nomi che sembrano quelli di Re Atztechì» (i mab's, ndr)
- «Il sistema sanitario fallirà per i DES e i defibrillatori»
- «Il sistema sanitario fallirà a causa dei check-point inhibitors»
- «Il sistema sanitario fallirà per le ATMP»

... spero anche saggio

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 - «Il sistema sanitario fallirà per le ATMP»
-
- ... e credo proprio che nemmeno stavolta il sistema sanitario fallirà, almeno non finanziariamente, forse potremo fallire noi organizzativamente, se non saremo capaci di portare i pazienti alle ATMP in tempo

To each according to his need

It is the aim of medical science to increase our capacity to treat effectively the most severe manifestations of disease. It is our expectation that society will reward this pursuit of new medical knowledge by providing the enabling resources to translate it to better care for patients. The report in this

—W. Keith Hoots

University of Texas Houston Health
Science Center and University of Texas
M.D. Anderson Cancer Center

*The title is ascribed to Karl Marx 1875
from Critique of the Gotha Program;
it was in quotes by Marx and may have
originated with Louis Blanc (1811-1882)
or Morelly (1840).*

"... riesce particolarmente pregiudizievole la tendenza a sopravvalutare - spesso, addirittura in modo esclusivo - la ragione che, a mio avviso, e' invece utilissima solo a patto di venir considerata come un complemento atto a perfezionare tutte le altre facoltà istintive intuitive psicologiche (ma non -guai! - a surrogarle)"



Bruno de Finetti, Varenna 1959

Meeting January 14 1965

President's Address

The Environment and Disease: Association or Causation?

by Sir Austin Bradford Hill CBE DSC FRCP(hon) FRS
(Professor Emeritus of Medical Statistics,
University of London)

observed *association* to a verdict of *causation*?
Upon what basis should we proceed to do so?

I have no wish, nor the skill, to embark upon a
philosophical discussion of the meaning of
'causation'. The 'cause' of illness may be imme-



All scientific work is incomplete – whether it be observational or experimental. All scientific work is liable to be upset or modified by advancing knowledge. That does not confer upon us a freedom to ignore the knowledge we already have, or to postpone the action that it appears to demand at a given time.

Who knows, asked Robert Browning, but the world may end tonight? True, but on available evidence most of us make ready to commute on the 8.30 next day.