a SWOT Analysis of Hemophilia Patients' Perspective

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My own history

- Born with severe haemophilia A (1951)
- Disease where clotting factor (VIII/IX) is missing
- Treatment for haemophilia with plasma products (J. Pool/B. Soulier) since 1965 - 1970
- Followed by prophylaxis, home-treatment
- Mid-nineties: rDNA products (biologicals)
- Future: gene therapy

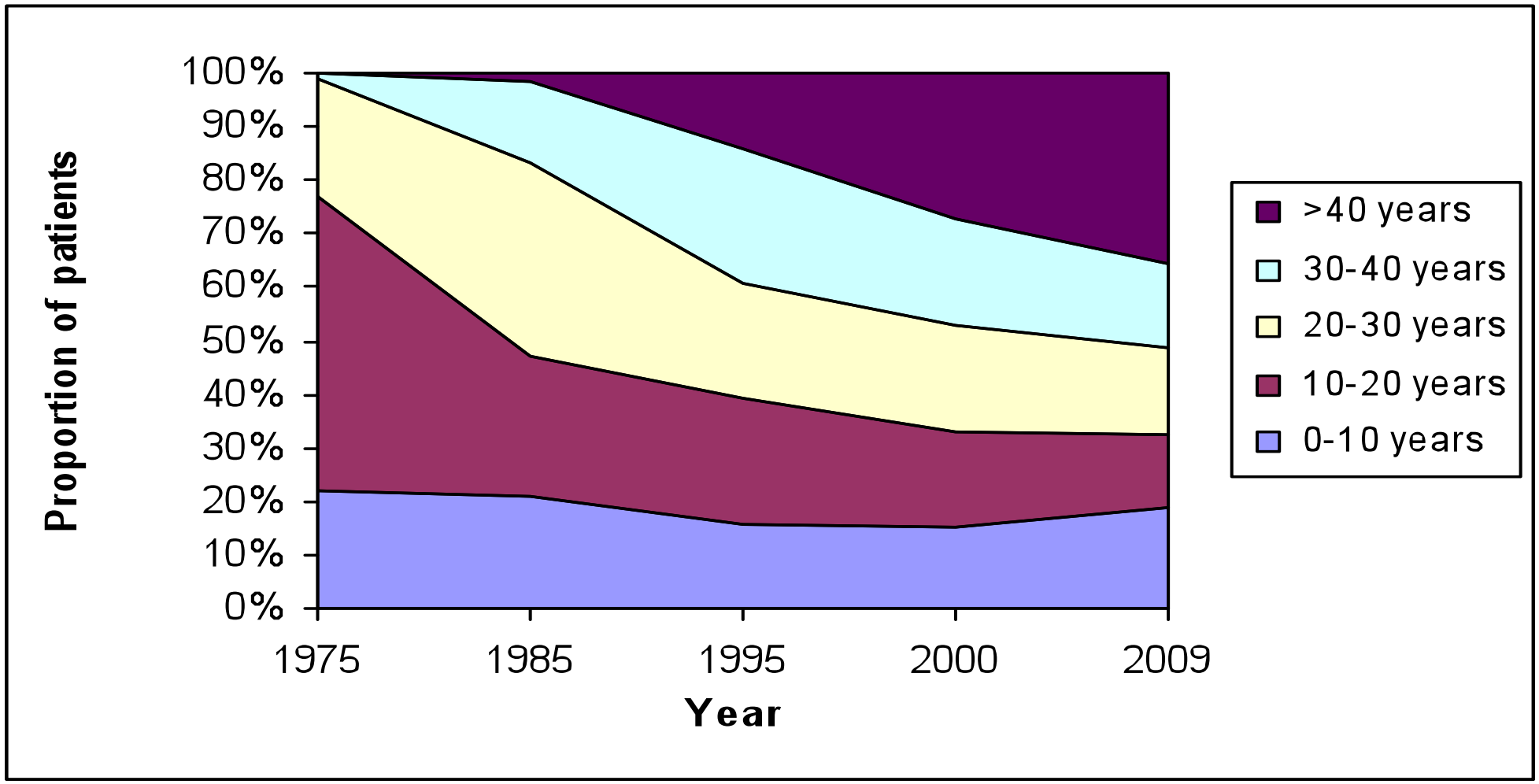
Unmet medical needs

Unmet medical needs
are the driving force
for patient groups
to become active in medical research





Age-distribution severe haemophilia (n=338)



Creveldclinic, Utrecht, 2010, personal communication

Aging with haemophilia

Medical and psychosocial impact

E.P. MAUSER-BUNSCHOTEN

A. DE KNECHT-VAN EEKELEN

C. SMIT

USA, 1975

'The disease we have fought is hemophilia, every family with a handicapped or chronically ill child shares the same problems: lack of money, isolation from the community of the healthy, prejudice, misunderstanding in school, loneliness, boredom, depression'

Robert & Suzanne Massie*, Journey,* 1975, p.XI

USA, 2012

'My hemophilia, the one thing that I had thought would define my life from birth to death, had been utterly, totally, and permanently cured'

Robert IV Massie*, A song in the night: a memoir of resilience,* 2012, p. 269



FRONTISPIECE.

A TALE OF TWO CITIES.

BY

CHARLES DICKENS.



LONDON:
CHAPMAN & HALL, 186, PICCADILLY.

A tale of two cities, Charles Dickens, 1859

'It was the best of times, it was the worst of times, it was the age of wisdom, it was the age of foolishness, it was the epoch of belief, it was the epoch of incredulity, it was the season of Light, it was the season of Darkness, it was the spring of hope, it was the winter of despair…'

Setbacks of hemophilia treatment

- ± 20.000 hiv-infections, early eighties
- ± 90 % hcv-infections, early eighties
- High cost of treatment, ± 150.000 $ / year
- ± 75 % patients worldwide still untreated
- rDNA products, more inhibitors in discussion
- Inhibitors, more expensive treatment
- No final cure within reach in near future

19 October 1987: Chicago

- **Alternatives for plasma products hemophilia**

- **Recombinant DNA**
- **Transgenic animals (Bull ‘Herman’, rabbits)**
- **Gene therapy**

- **Conclusion: Changes go very slow !**

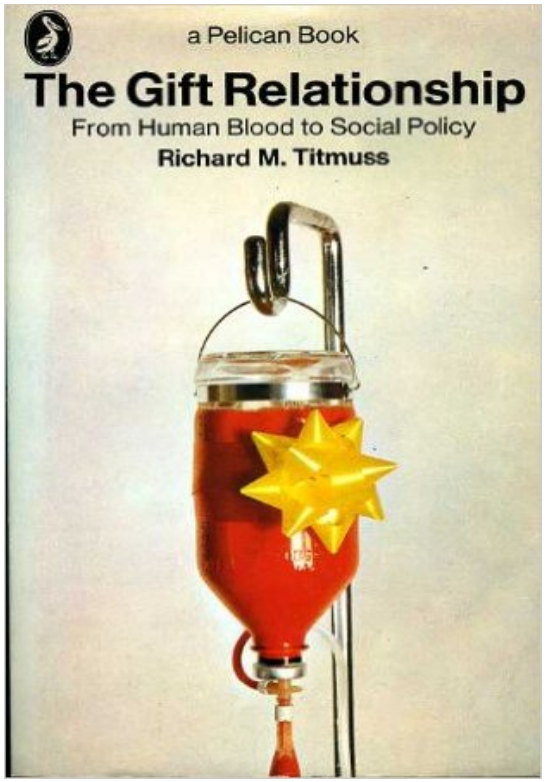
Farewell Symposium Pim van Aken, 2001

- *'There is a future, but it will be complex, different and demanding'*
- (J. Mc. Cullough, USA)

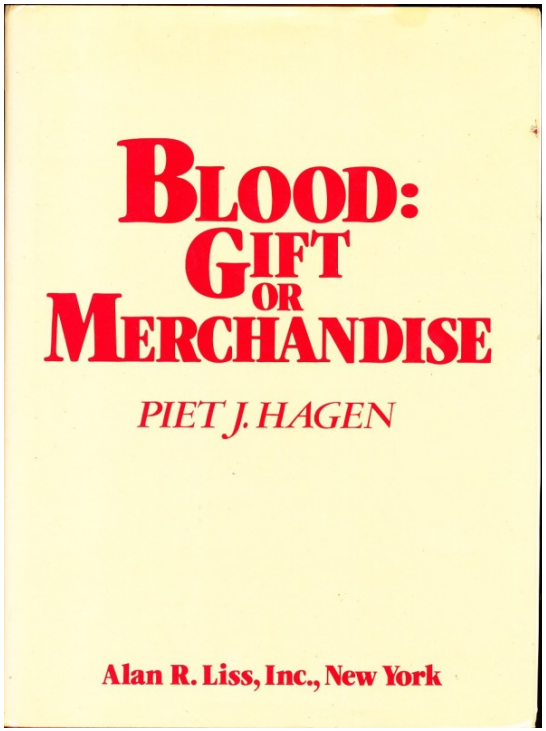
- *'The 'globalization' of the plasma products industry leads to a call for not only harmonization within the EU but also across continents'*
- (Richard Walker, UK)

Paid Plasma safety warnings before Aids/hiv became a problem

1970 : Richard Titmuss

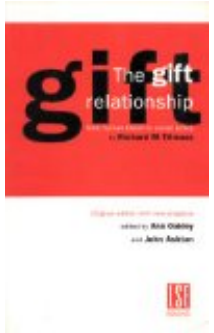


1982: Piet J. Hagen

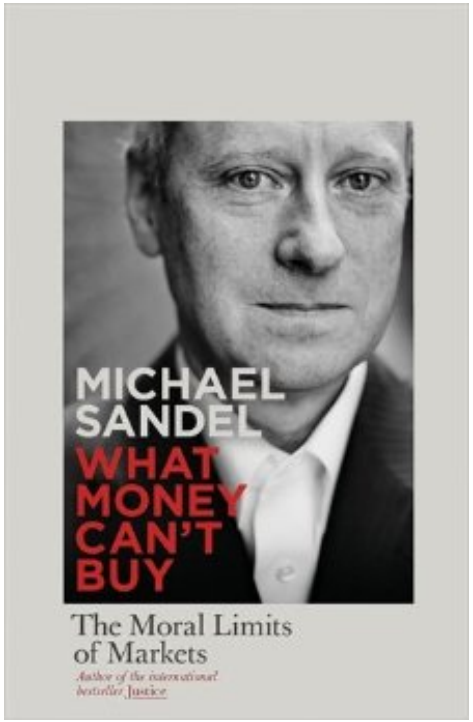


Paid Plasma safety warnings after Aids/hiv became a problem

1997



2013



Titmuss, Oakley & Ashton (1997)

- *'One of the striking features of the blood system highlighted by AIDS in the UK, in France, and elsewhere in Europe, was that a 'volunteer image' fronted systems which had become highly dependent on commercial sources'*

- *'In an era of marketised health and welfare services, the arguments of 'The gift relationship' are even more pertinent than when the book first appeared'*

What money can't buy (2013)

- *'Altruism, generosity, solidarity, and civic spirit are not like commodities that are depleted with use. They are more like muscles that develop and grow stronger with excercise.*
- *One of the defects of a market-driven society is that it lets these virtuses languish.*
- *To renew our public life we need to excercise them more strenously'*

The long shadows of past mistakes

- *'The ideal blood supply is from volunteer donors'*

- *'Around the world, nations without adequate safety assurances for blood products must strive to attain blood security while increasing the number of voluntary donations .. – failure to do so will cast shadows long into the future'*

- The Lancet, editorial, Vol 2 February 2015

Solutions

- A collective international call for voluntary donations & an increase of plasma collection from voluntary donations
- A gradual decrease of paid ‘forced’ donations
- Cooperation with industry in fractionation
- A better use of plasma FVIII/IX from countries that switched to recombinant FVIII/IX
- International harmonization of regulations

Conclusion

‘There is an urgency to meet unmet medical needs of hemophiliacs and other plasma products dependent patients all over the world’

Cees Smit, Forthworth, September 29, 2015

For more information

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