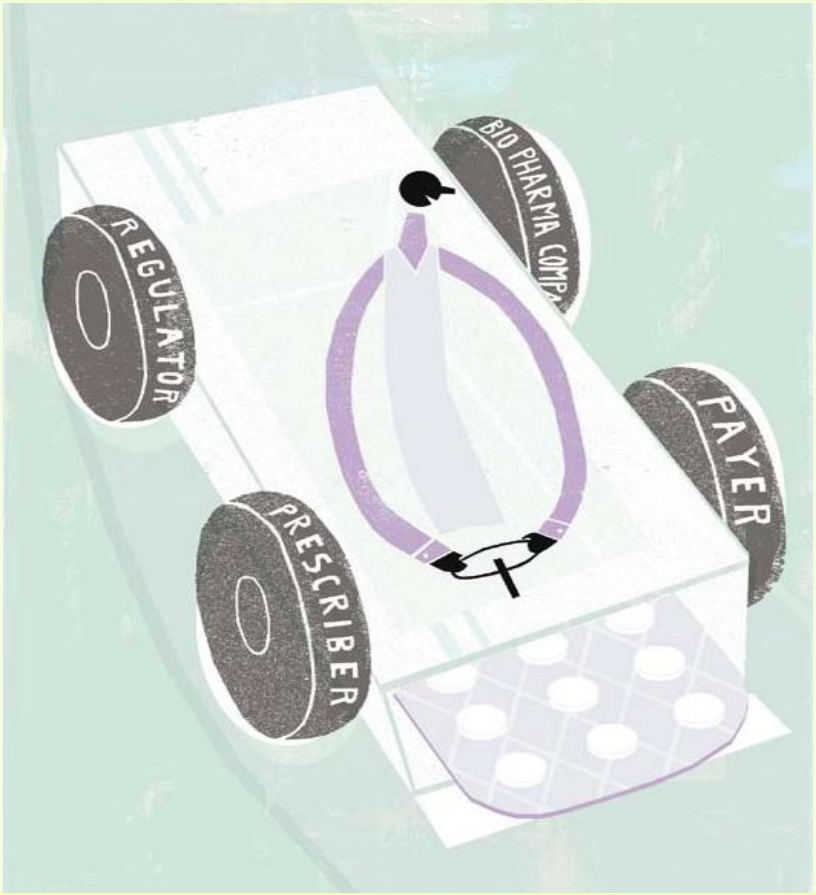
EMSP 2014
Spring Conference

Driving improved access to treatment via Europe and through national MS Societies

Christoph Thalheim – *EMSP*
Luiza Wieczynska – *PTSR*

EUROPEAN
MULTIPLE SCLEROSIS
PLATFORM

The pharmaceutical industry is suffering

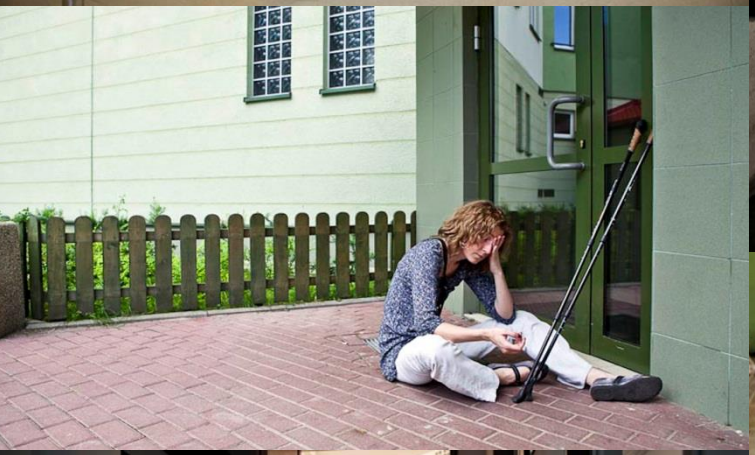


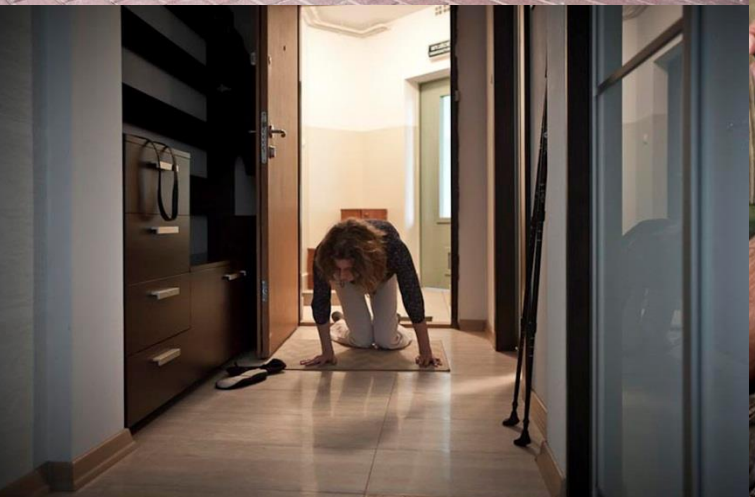
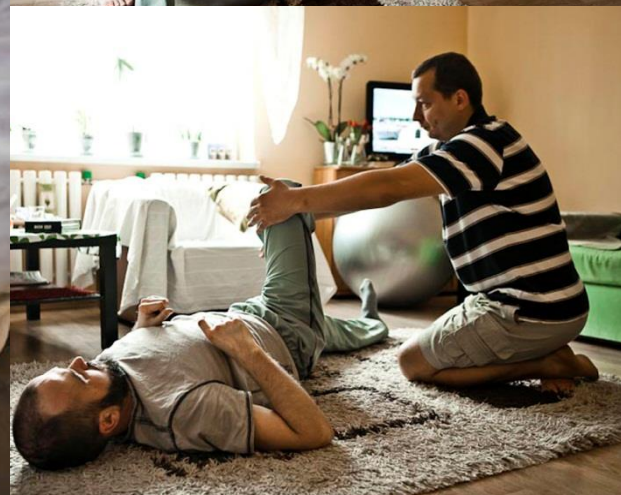
Expiring patents, increasing demands from regulators and decreasing healthcare budgets are putting companies under pressure, and the industry has to walk a narrow tightrope between keeping profitability up and quickly developing attractive medications.

Source: Prism / 2 / 2009 Arthur D Little

EUROPEAN MULTIPLE SCLEROSIS PLATFORM

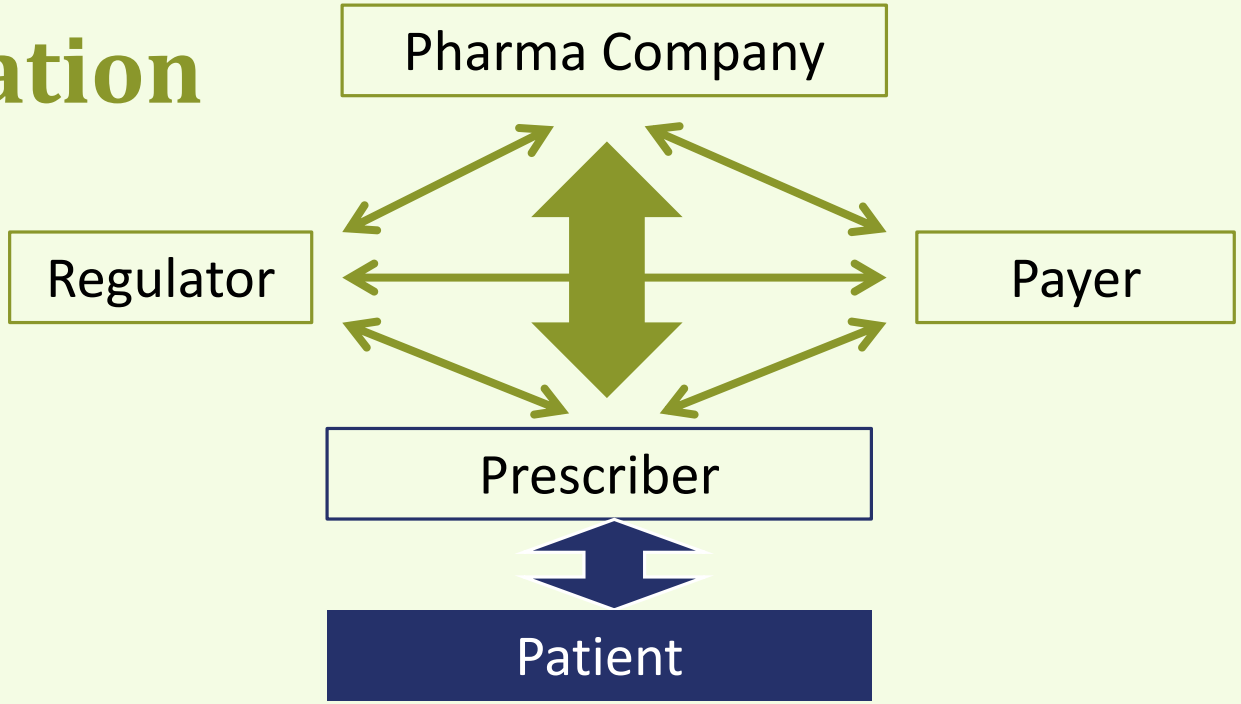


The position of the patient in healthcare transactions [1]

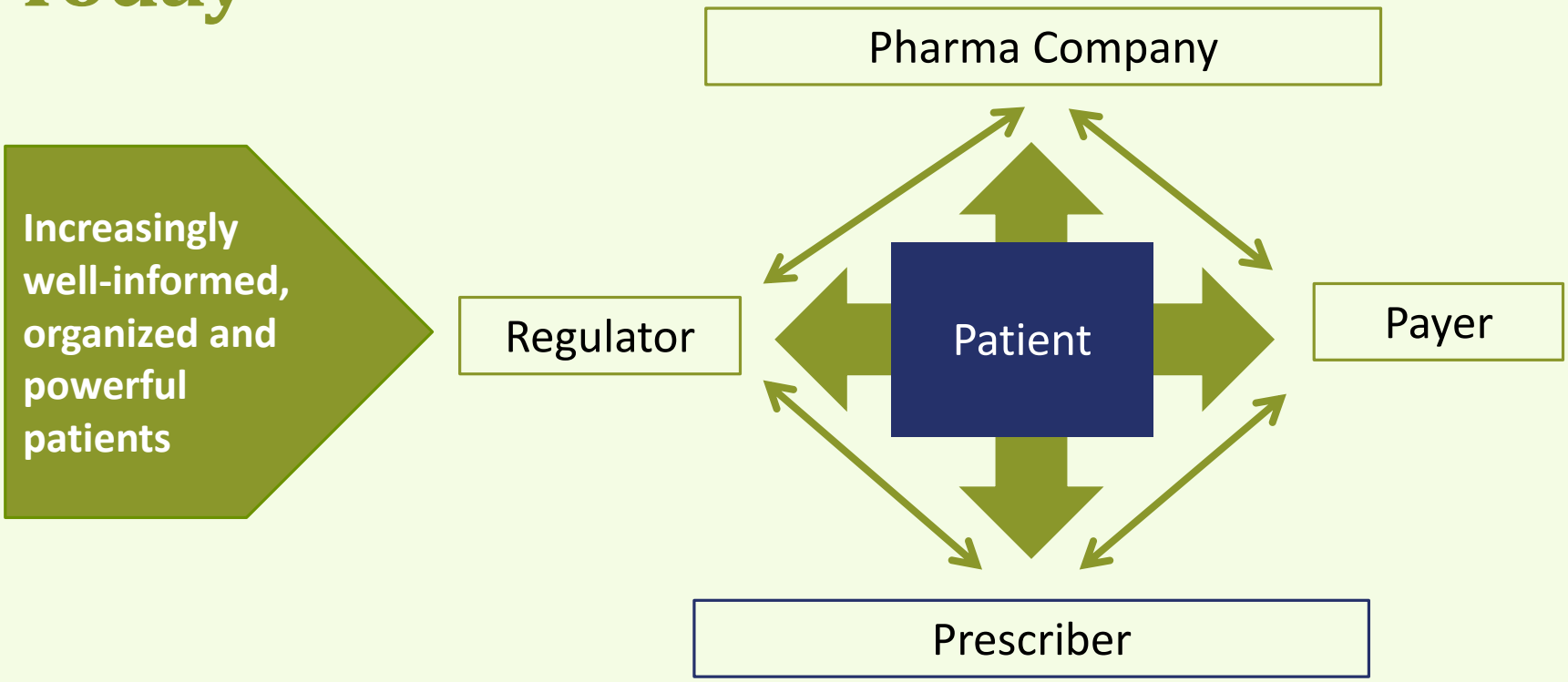
Historical Situation



EUROPEAN MULTIPLE SCLEROSIS PLATFORM

The position of the patient in healthcare transactions [2]

Today



EUROPEAN MULTIPLE SCLEROSIS PLATFORM

“First EU Report on MS in EP”

18th December 2003, EU Parliament Strasbourg

- Approval of the MS Report by 240 MEP‘s in the European Parliament
- (Petition 842/ 2001) concerning the effects of discriminatory treatment towards persons with MS, within the European Union (2003/2173 (INI)):
- “…persons with Multiple Sclerosis, and many other chronic long-term illnesses, are subject to varying levels of medical and therapeutic care depending on their place of residence and … insufficient priority has been accorded by Member States of the Union… to remedying this fact”

Late Uma Aaltonen,
Finnish MEP and PwMS

EUROPEAN MULTIPLE SCLEROSIS PLATFORM

High Level Pharmaceutical Forum 2005 - 2008

Was a milestone for patient involvement + emporwerment because EPF was invited to send their representatives to all working groups of the Forum

EPF European Patients Forum

emsp EUROPEAN MULTIPLE SCLEROSIS PLATFORM

Which is the added value of involving patients in the scientific process?

In general, patients bring real-life experience of the disease and its current therapeutic environment; as a consequence:

- it enriches regulatory outcome by complementing it with the views of those directly affected by regulatory decisions,
- it increases confidence and trust in the regulatory process
- it incurs in higher level of transparency

8

Patient involvement in the Agency's activities [1]:

Patients are full members of:

- Management Board
- Committee for Orphan Medicinal Products (COMP)
- Paediatric Committee (PDCO)
- Committee for Advance Therapies (CAT)

9

Patient involvement in the Agency's activities (2):

- **Patients and Consumers Working Party (PCWP)**
- **Review of Product Information**:
  - EPAR summaries, Package Leaflets, safety information–Q&A
- **CHMP (Ad-hoc collaboration):**
  - Input on assessment of products (e.g. thalidomide, **tysabri**, etc)
  - Experts in scientific advice/protocol assistance
  - Input in guideline preparation
  - Observers in Pharmacovigilance working party (pilot phase)
- **Regular participation in Agency's workshops & conferences**

10

Tysabri was a Watershed!

- Natalizumab (Tysabri) is a great drug, but!
- Efficacy was about double that of IFN-ß…
- …but a few patients contracted PML during clinical trials (about 1 in 1,000) and 2 died!
- At this stage, FDA or EMA would normally have rejected the drug as presenting an unacceptable risk/benefit ratio, but
- Patient advocacy groups were decisive in demanding regulatory agencies approve the drug

Open letter to EMA by PwMS

Allen O'Connor, Irish person with MS (August 2005)

Dear Mr. Lönngren,

I am a person with Multiple Sclerosis, a board member and past Chairman of the Multiple Sclerosis Society of Ireland (2002 to 2005), Honorary Treasurer of the European Multiple Sclerosis Platform (EMSP) and a board member of the Multiple Sclerosis International Federation (MSIF).

In relation to EMEA's role in the licensing or otherwise of any and all future treatments relating to multiple sclerosis, I would like to make the following personal comments.

I am more interested in the quality of my life than in the length of my life and all decisions that I make in relation to medical and non-medical interventions are made with this in mind.

I want to add life to my years, not necessarily years to my life!

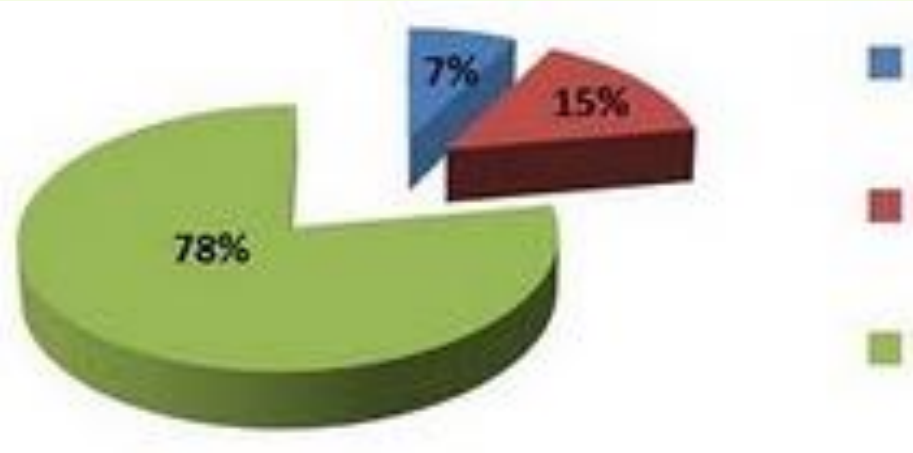
EUROPEAN MULTIPLE SCLEROSIS PLATFORM

Lessons to be learned from the early Tysabri handling?

- Safety concerns of medical experts and regulators not necessarily identical with scaling of benefit-risk ratio by patients
- MS patients accept a much higher risk then medical experts think – if
  - the benefit is clearly described and likely /sure to happen
  - the risk information provides at least an “educated guess” on worst case scenario and likeliness for this to happen
- Patients’ and Consumer Organisations can act as efficient information multiplier - if informed asap in laymen’s language
- Full transparency and pro-active information policy by the industry are a **must**

A random piece of public opinion
a blog survey done by Prof. Gavin Giovannoni

Thursday, 2 June 2011
Who has the best perspective to assess whether the risks of the newer DMTS are worth taking



Professor Gavin Giovannoni MBBCh, PhD, FCP (S.A., Neurol.), FRCP, FRCPath www.ms-res.org Chair of Neurology, Blizard Institute, Barts and The London School of Medicine and Dentistry

The case of FAMPYRA (fampridine)

Patients do have a say (if they wish)!

On January 22, 2010 Fampridine (meant to improve walking abilities of PwMS) was approved by FDA under the trade name FAMPYRA

EMA says NO!

On January 21, 2011 the European Medicines Agency issued a negative opinion (uncertain benefits, many non-responders)

PwMS react

Request by Austrian patients and neurologist to EMSP to act on their behalf

EMSP reacts

Submission of collected patients ans neurologists opinion coming from Belgium, Estonia, France, Germany, Iceland, Ireland, Norway, Portugal, Romania and Sweden

EMA says YES!

On May 19, 2011, the European Medicines Agency issued a positive opinion on a (conditional) marketing authorisation

Bi-annual MS Barometer

European benchmarking tool as driver of change

2008 – First version of MS Barometer data used at HLNRT in Slovenia

2009 – Development of improved version of MS Barometer – widely used in

2011 – and also in

2013 – results to be published in:

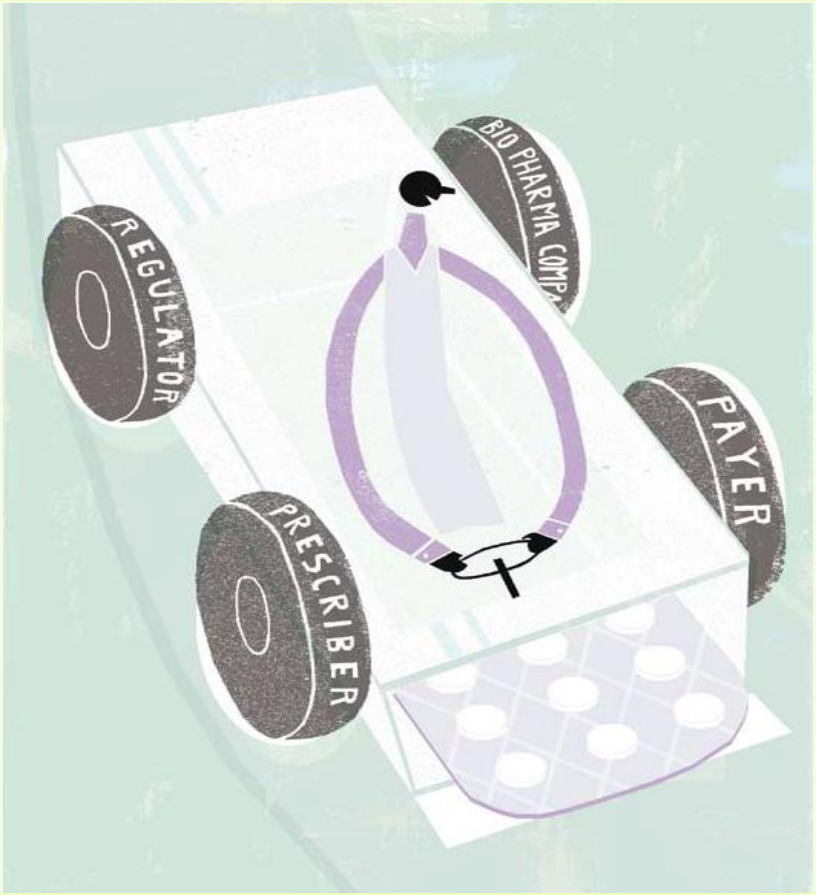
2014 – May 28th **"World MS Day"**

2015 – Further improved version No. 3 available?

… hand over to Luiza presenting the Polish experiences with our Barometer …

EUROPEAN MULTIPLE SCLEROSIS PLATFORM

The pharmaceutical industry is suffering



Expiring patents, increasing demands from regulators and decreasing healthcare budgets are putting companies under pressure, and the industry has to walk a narrow tightrope between keeping profitability up and quickly developing attractive medications.

Source: Prism / 2 / 2009 Arthur D Little

EUROPEAN MULTIPLE SCLEROSIS PLATFORM