PML – Balancing risks and benefits. The patients' view

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emsp
european multiple sclerosis platform aisbl

Structure of the presentation

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is the umbrella organisation of currently 38 national MS-Societies in 34 European countries and represents more than half a million people with MS and more then 1,5 million people daily affected by MS (such as family and other care givers)

Contrary to other disease areas, people with Multiple Sclerosis in Europe are represented by only ***one*** *European MS umbrella organisation.*

*…was **founded by 21 national MS Societies** (= patient groups) in 1989 as European umbrella organisation.*

*…**main purpose** was (and is until today) to complement on European level the work of its national member organisations*

*- through advocacy work towards **European Institutions**,*

*- through **collecting and disseminating relevant information** &*

*- through **European projects** within the **EU and***

Europe

EMSP Members

- Austria
- Belarus
- Belgium
- Bosnia-Herzegovina
- Bulgaria
- Croatia
- Czech Republic
- Denmark
- Estonia
- Finland
- France
- Germany
- Greece
- Hungary
- Iceland
- Ireland
- Italy
- Latvia
- Lithuania
- Luxembourg
- Malta
- Netherlands
- Norway
- Poland
- Portugal
- Romania
- Russia
- Serbia
- Slovakia
- Slovenia
- Spain
- Sweden
- Switzerland
- UK

The patient is no longer passive

British Medical Journal 1999;319(18 September):762.

Why is it important that patients and caregivers play a role in the health care debate in civil society?

- *Knowledge and expertise through their own personal and collective experience*

- *Drive, legitimacy and authority of a representative and independent "Patients' Movement", as both a watchdog and critical friend*

- *The mutual value of partnerships - with the medical community, in political dialogue, and with the corporate world to progress health outcomes and patients' quality of life*

Cross EU standards & consensus papers

As requested by European Parliament's Resolution on Multiple Sclerosis in 2003, EMSP facilitated – with the help of key experts - the development of the

"European Code of Good Practice in MS"

including the European Consensus Papers for

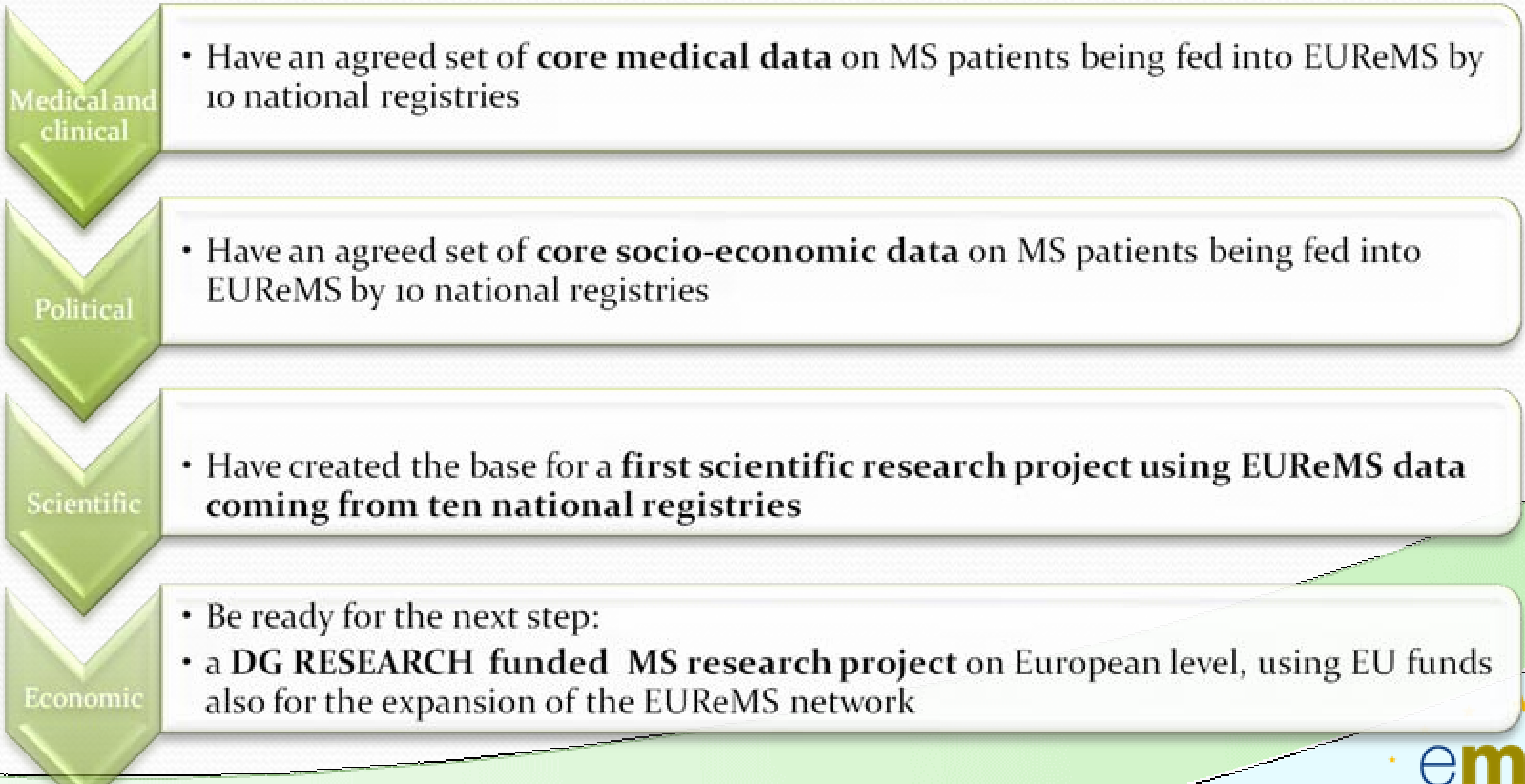
Rehabilitation in MS, Symptomatic Treatment, Use of DMDs Palliative Care and (in preparation) Paediatric MS

amended by MSIF's "Principles for Quality of Life" plus – allowing evidence-based comparative lobbying across Europe – the

*www.europeanmapofms.org & www.**ms**-barometer 2009.org*

EU & Industry co-funded project European Register for Multiple Sclerosis (EUReMS) 1, 5 Million Euro 2011-2013

- By mid 2014, EUReMS will ...



Medical and clinical

- Have an agreed set of **core medical data** on MS patients being fed into EUReMS by 10 national registries

Political

- Have an agreed set of **core socio-economic data** on MS patients being fed into EUReMS by 10 national registries

Scientific

- Have created the base for a **first scientific research project using EUReMS data coming from ten national registries**

Economic

- Be ready for the next step:
- a **DG RESEARCH funded MS research project** on European level, using EU funds also for the expansion of the EUReMS network

The patient's perspective within regulatory work

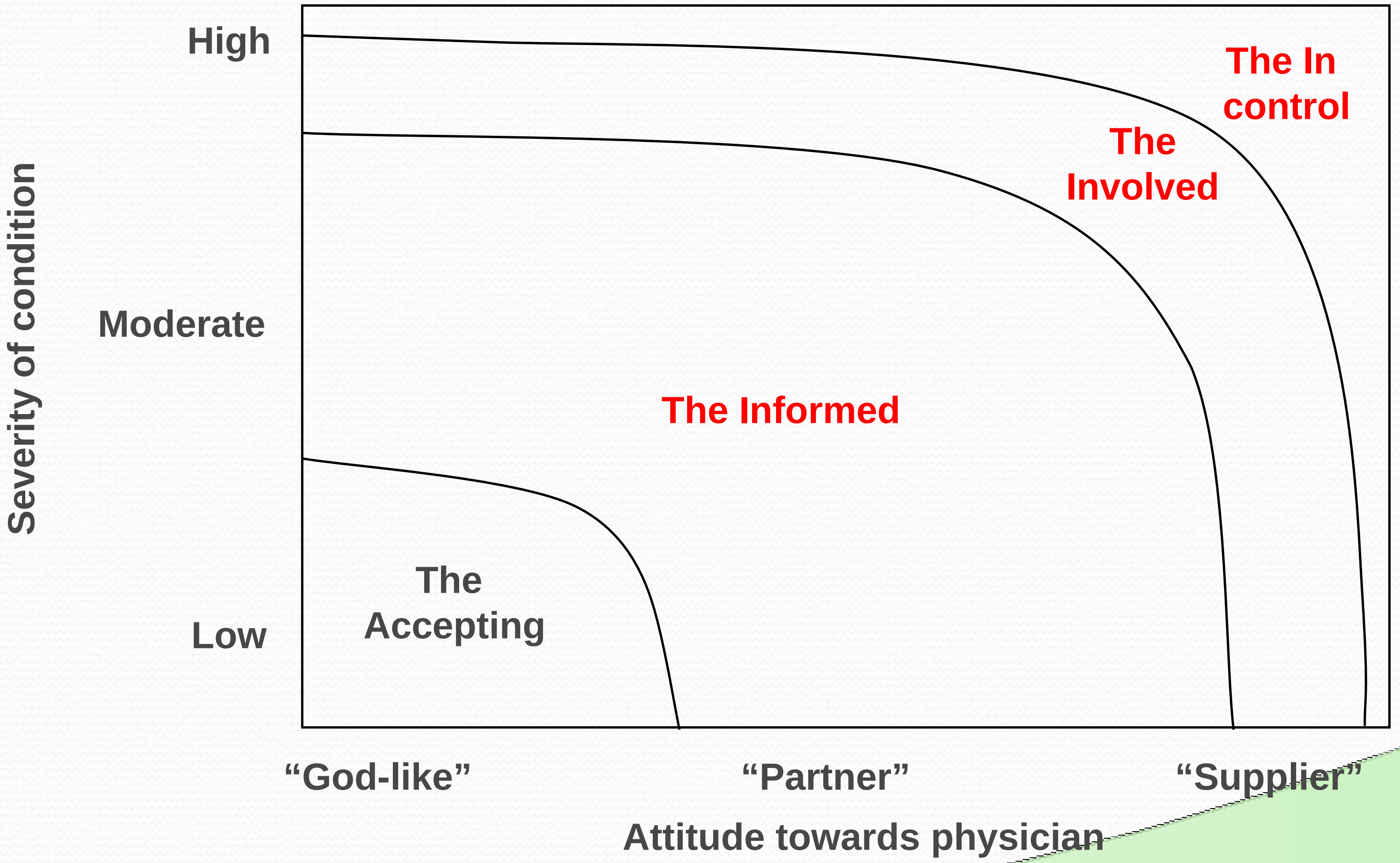
Where are patients involved in the discussion already?

EMA Working Party with Patient Organisations (PCWP)

- *on "Pharmacovigilance"   (e.g. direct reporting)*
- *on "Information":*
  - *e.g. Package Leaflet (as shown on EMSP website)*
  - *e.g. EPAR summary (as shown on EMSP website)*

SAG meetings (input to benefit –risk questions and other issues)

Patients are getting more involved



Severity of condition

High

Moderate

Low

The In control

The Involved

The Informed

The Accepting

"God-like"

"Partner"

"Supplier"

Attitude towards physician

Note: Weighted to population on online chronically ill, if weighted to overall chronically ill population (accepting, 12% ; informed, 59%; involved 22%; in control 7%)

Source: Harris Interactive 10,000 patient survey (Oct-99) and BCG analysis

EMA Scientific Committees Working Party with Patients' and Consumers' Organisations

emsp
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IPOPI

EATG
EUROPEAN AIDS

europeanpatients'forum

EPHA

EUROPEAN CANCER PATIENT COALITION

beuc

EURORDIS
European Organisation for Rare Diseases

International Diabetes Federation

European Federation of Neurological Associations EFNA

International Alliance of Patients' Organizations

- *PCWP Members: 15/25 Eligible Organisations + representatives from Agency's Scientific Committees (CHMP, COMP, HMPC, PDCO and CAT)*
- *Co-Chair: Isabelle Moulon (EMA)/ Lise Murphy(EURORDIS)*
- *4 meetings per year (one joint with Healthcare professionals)*

Tysabri – Opening a new door to hope for MS - Patients

What MS patients know today:

+ ***Highly effective drug improving QoL considerably****, but mostly reserved for patients that stop responding to other MS drugs or have an aggressive form of the debilitating disease*

+ *Seems to help many of the ca. 60.000 MS patients currently worldwide being on Tysabri, for some of them it not only stops worsening symptoms, but even improves existing symptoms*

+ *Study* shows that Tysabri reduces the relative risk of disability progression by almost half*

**AFFIRM trial*

PML – A price to high to be paid for QoL of MS – Patients?

What MS patients know today:

- *Progressive Multifocal Leukoencephalopathy (PML) is a extremely debilitating, often fatal viral disease, happening to MS patients on Tysabri and to other patients being on similar drugs*
- *MS patients on Tysabri are recommended to stop there treatment after 2 years to reduce the risk of developing PML*
- ***Globally, there are 145 MS patients who developed PML, 29 of which died until July 5th , despite the strict access plan that regularly monitors MS patients on Tysabri***
- *A blood test for antibodies to the PML causing JC virus may better determine the risk of patients contracting*

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!

Open letter to EMA by PwMS

(…) A decision to proceed with any medical treatment for any person not just a person with MS, should be made by the person themselves, after having a consultation with the doctor (Neurologists etc) where the following should be discussed in detail:

- *1.An explanation of the treatment and procedure involved*
- *2.The alternative choices, if any, available to the person, including “do nothing”.*
- *3.The likely benefits supported by the statistical information relating to outcome, successful, neutral, negative or even fatal.*
- *4.The side effects, short-term and long-term, that may result from a physical, mental and every-day living point of view*

I accept that the National and European licensing authorities have an important role to play in the development of any and all new MS treatments, HOWEVER, I too, would like to have a say, and I would not be too happy if any of these bodies denied me the opportunity of accessing any reasonably-safe product or procedure that could have a beneficial effect on my quality of life.(…)

Yours faithfully,

Allen O’Connor, Person with Multiple Sclerosis

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 are a <u>must</u>*

Patient decision: PML risk versus QoL

Lauren Parrott

PML Patients view – Angela Cleary

‘Please, stop taking Tysabri!!!!!!!!!!!! It almost killed me and it put me through the most horrible and painful periods of my life.. We shouldn't be suffering this much so please, stop taking that poison.

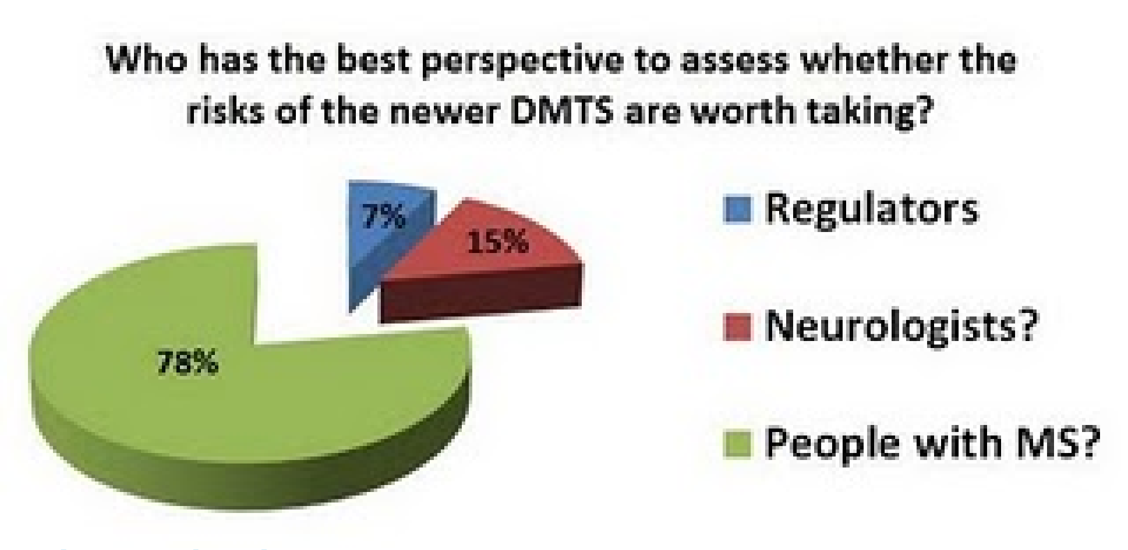
Every day, I live my life in horrible emotional and physical pain because of that awful drug that didn't make me better... it made me worse!

I am sorry that I am so honest and so direct to you but I CARE about people and I don't want this to happen to you! so please, talk to your doctor and ask about stopping this bad drug... stopping has its own risks so ask about that too...

Angela Cleary, PML Patient, CT USA

A random piece of public opinion- a blog survey done by Prof. 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
I was appointed to the Chair of Neurology, Blizard Institute, Barts and The London School of Medicine and Dentistry, in September 2008

DEFERNO Trust – a private PML website www.defernotrust.org

- Private, non for profit , website and trust
- Established and currently maintained by Mr. Declan Walsh, Sligo, Ireland in January 2011, on behalf of his wife, Dr. Natalie Murphy, who was diagnosed with PML in November 2009.
- The only independent worldwide patient and family forum support group currently available for those affected by Tysabri related PML.

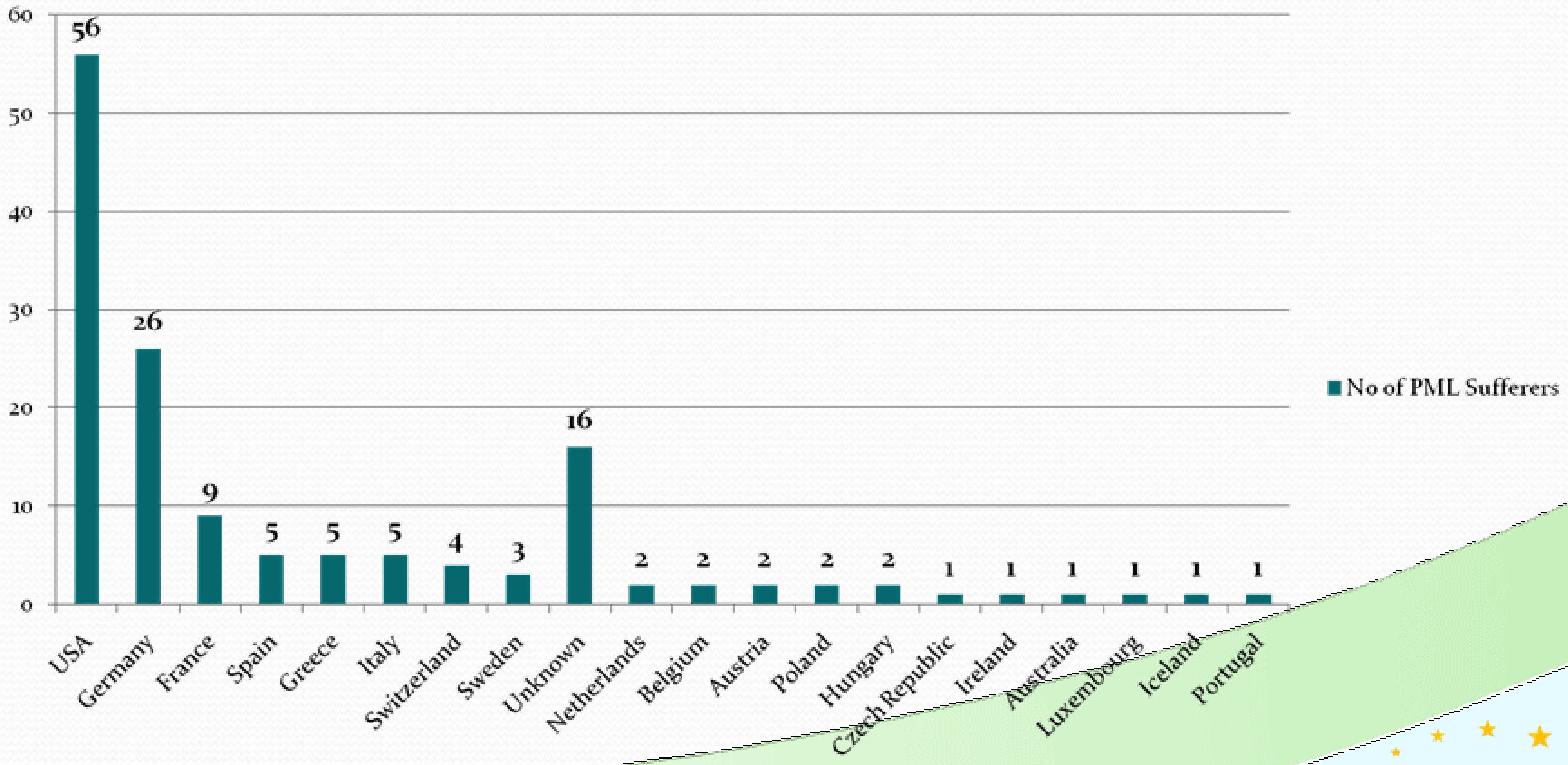
Tysabri related PML – trending

- *The following slides represent the increase incidence of Tysabri related PML*
- *They are unconfirmed as there is no central and accurate database available directly to patients to understand the impact and spread of the this disease.*
- *The numbers have been compiled from trawling the financial and medical press each month.*
- *This represents a major frustration for patients and families faced with Tysabri related PML*

Geographical Analysis of PML Sufferers

(Unconfirmed)

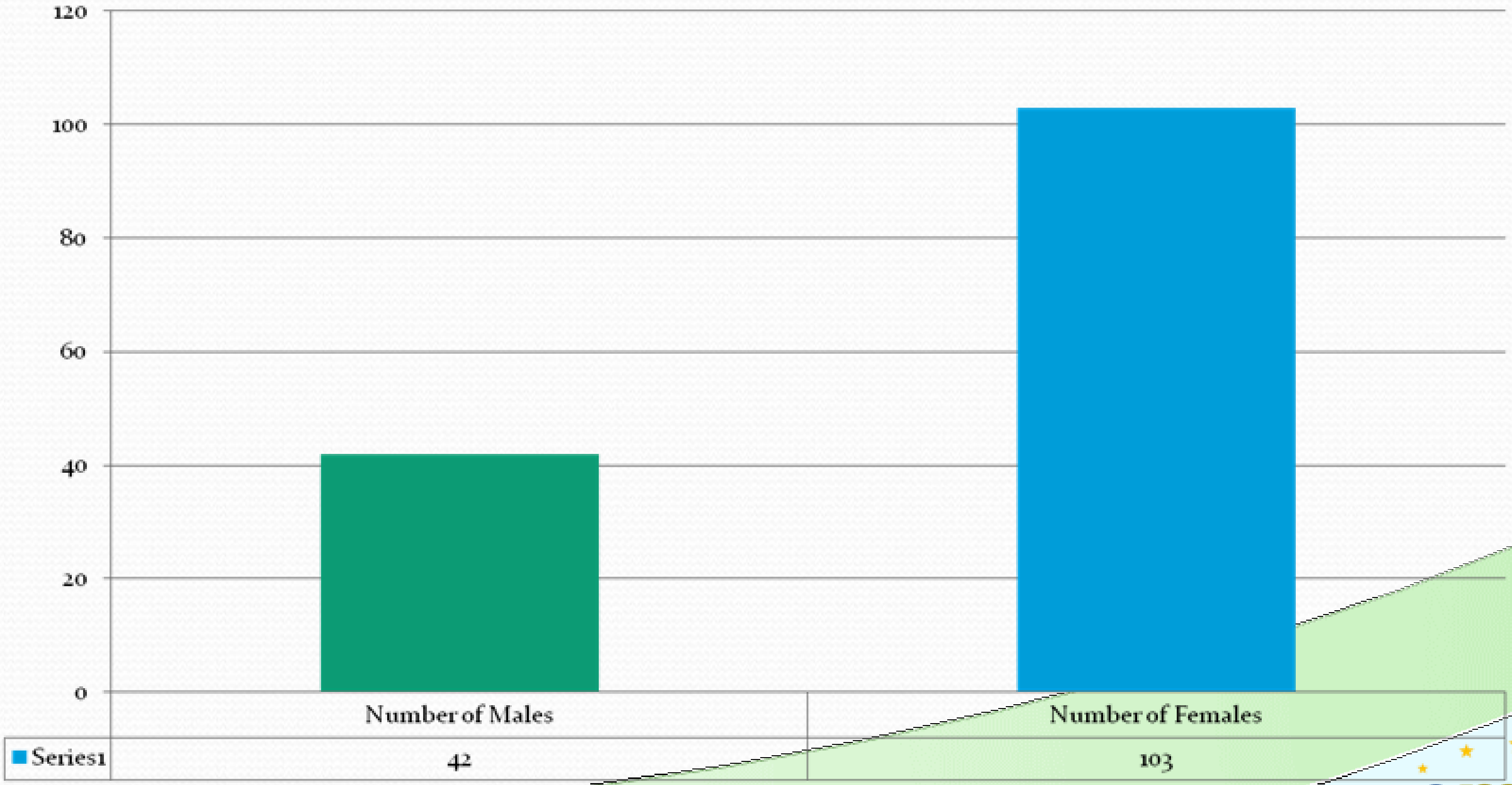
(145 patients - July 2011)



PML Sufferers - Gender Analysis

(Unconfirmed)

(145 patients - July 2011)

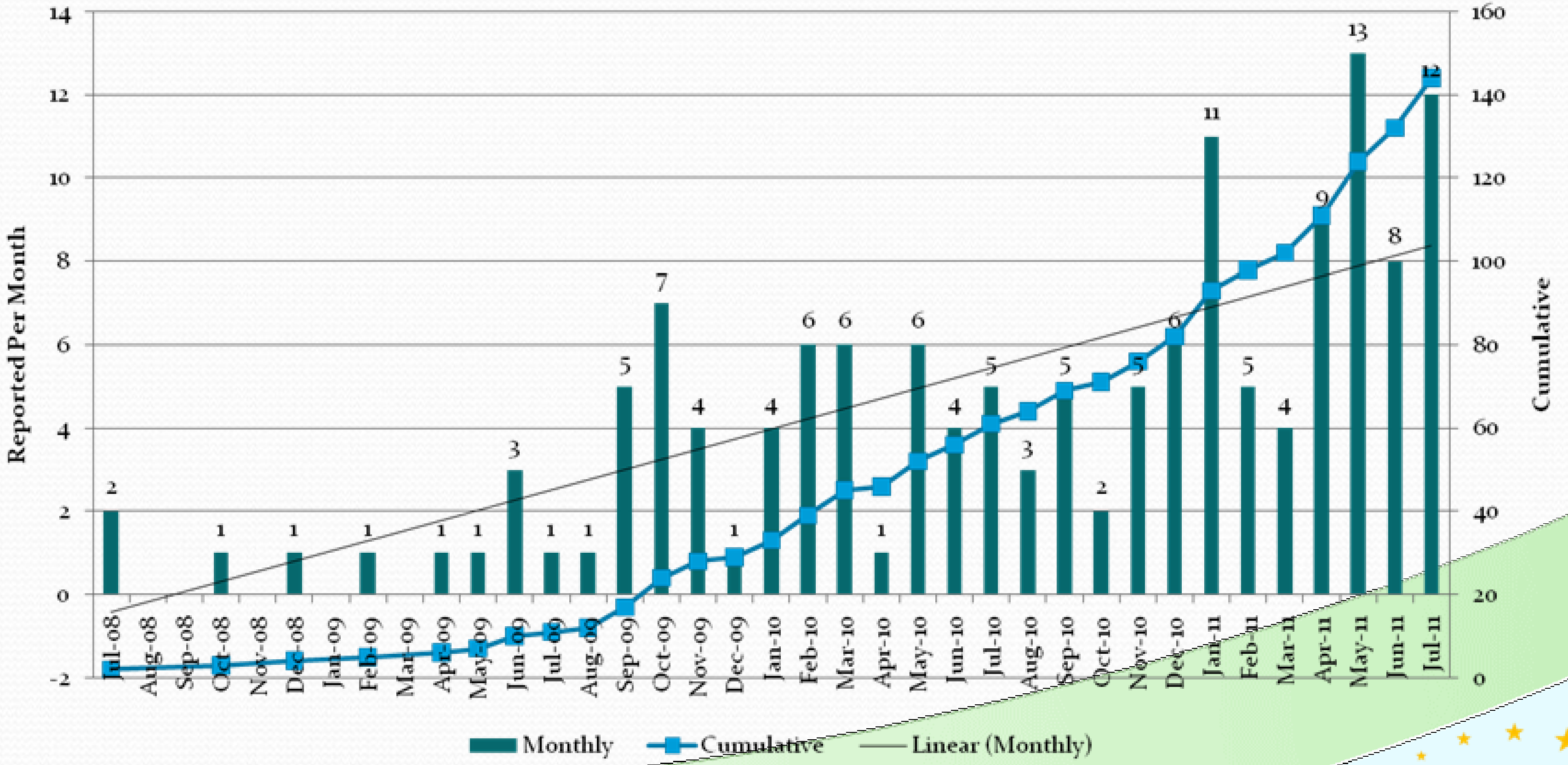


	Number of Males	Number of Females
■ Series1	42	103

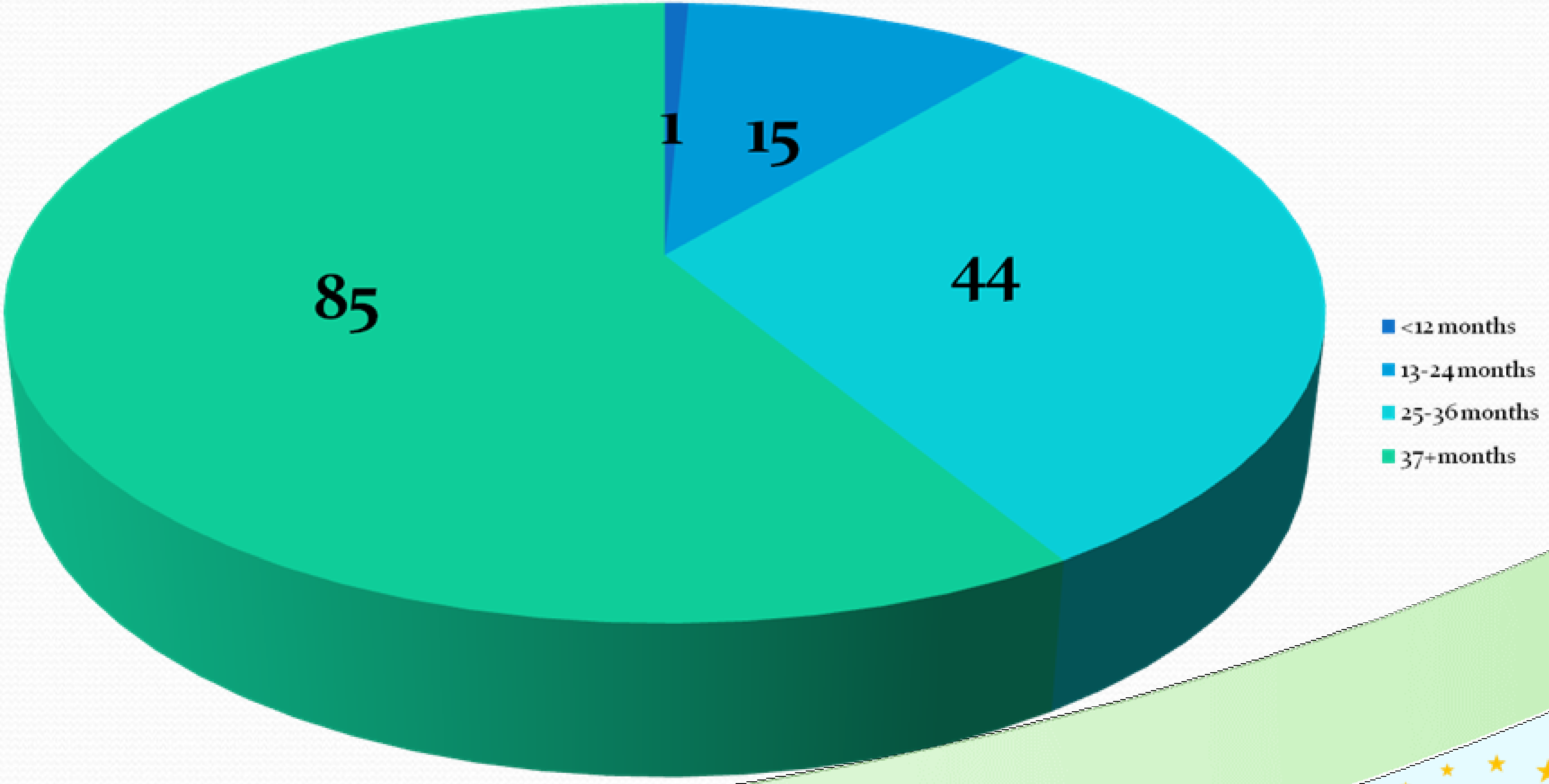
Timeline of reporting of Tysabri related PML

(unconfirmed)

(145 patients - July 2011)



No of Infusions taken when PML Sufferers received Diagnosis (145 patients - July 2011- Unconfirmed)



What is on the agenda/wish list of EMSP?

- ***European MS Register EUReMS – the Online Patient & Research Database being in development (chance for data collection to support PML research?)***

- *More* ***comparative studies****/ -information between drug options*

- ***Concrete figures*** *of clinical trial results rather than percentage (11 out of 2536 patients showed…)*

- ***Common approach*** *of all (MS) pharma industry plus regulators on how to measure & describe benefit & risk in lay language*
  (involving much more “patient reported outcome”)

Summary I

- *Many MS patients demand monoclonal antibodies as powerful agents for treating agressive MS, even if safety profiles of these new agents are uncertain and stringent risks management plans are required*
- *MS Patients want to be regarded as equal partner within optimized shared-decision making between patients and neurologists - objectivity and full transparency by the medical expert are basic requirements for this process*
- *Risk acceptability increases with disease severity – MS patients - being often severely debilitated - want to add "life to their years, not necessarily years to their life"*

Summary II

- *Safety concerns of medical experts and regulators are not necessarily identical with scaling of benefit-risk ratio by patients*
- *Caregivers of MS Patients with PML have a specifically strong need for information and re-confirmation on latest research developments, to be provided by*

  - the medical expert (neurologist or MS nurse)?

  - the regulatory bodies?

  - who else?
- *Caregivers of MS Patients with PML wish to get the feeling that they are not alone – internet based networking to be supported by EMA/FDA?*

Thank you!

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