



PATIENT'S LEADING THE DISCUSSION

Eibhlín Mulroe, MBA
CEO, IPPOSI

IPPOSI Strategy 2012-2014

Our Mission

We expedite development of and patient access to innovative therapies through a unique partnership of Patient Groups, Industry and Science

To deliver on our Vision

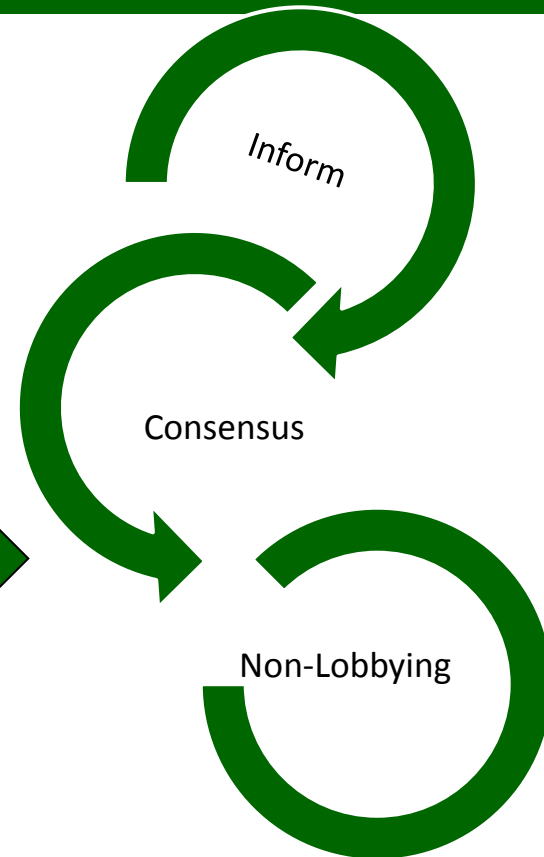
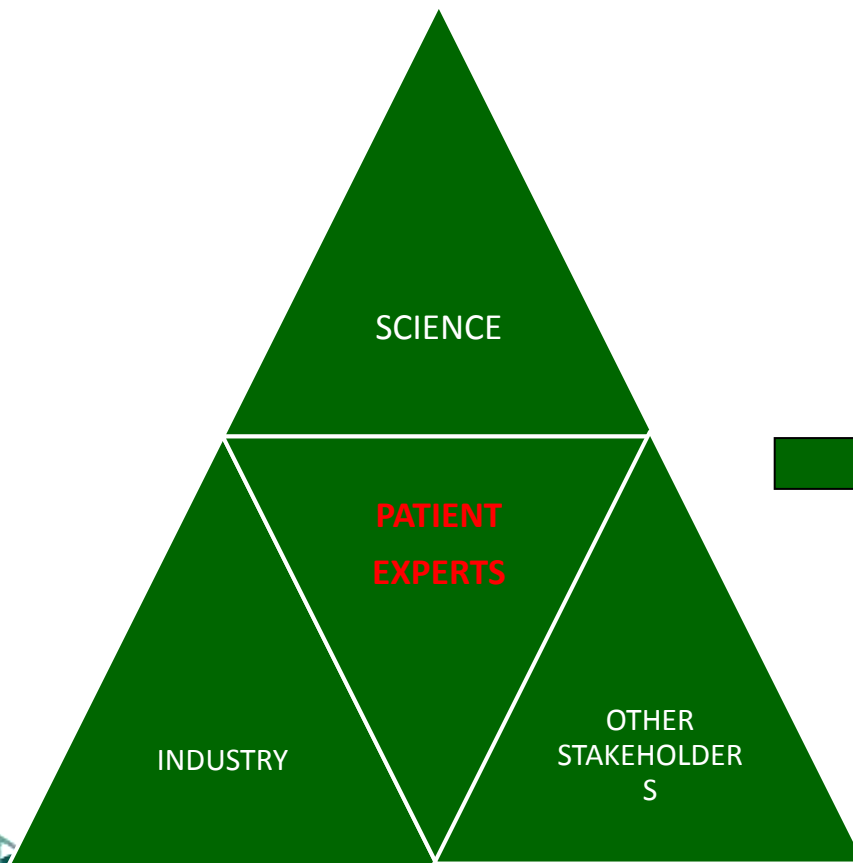
Our Strategic Priorities

- Bring a patient perspective to clinical research in Ireland
- **Actively influence policy that impacts on research and access to innovative therapies**
- Increase understanding of the work done by IPPOSI
- Source funding to ensure IPPOSI's sustainability

Vision

Patients in Ireland have prompt access to new and developing innovative therapies

Leadership



IPPOSI Funding

- **Membership subscriptions**
- **The Health Research Board**

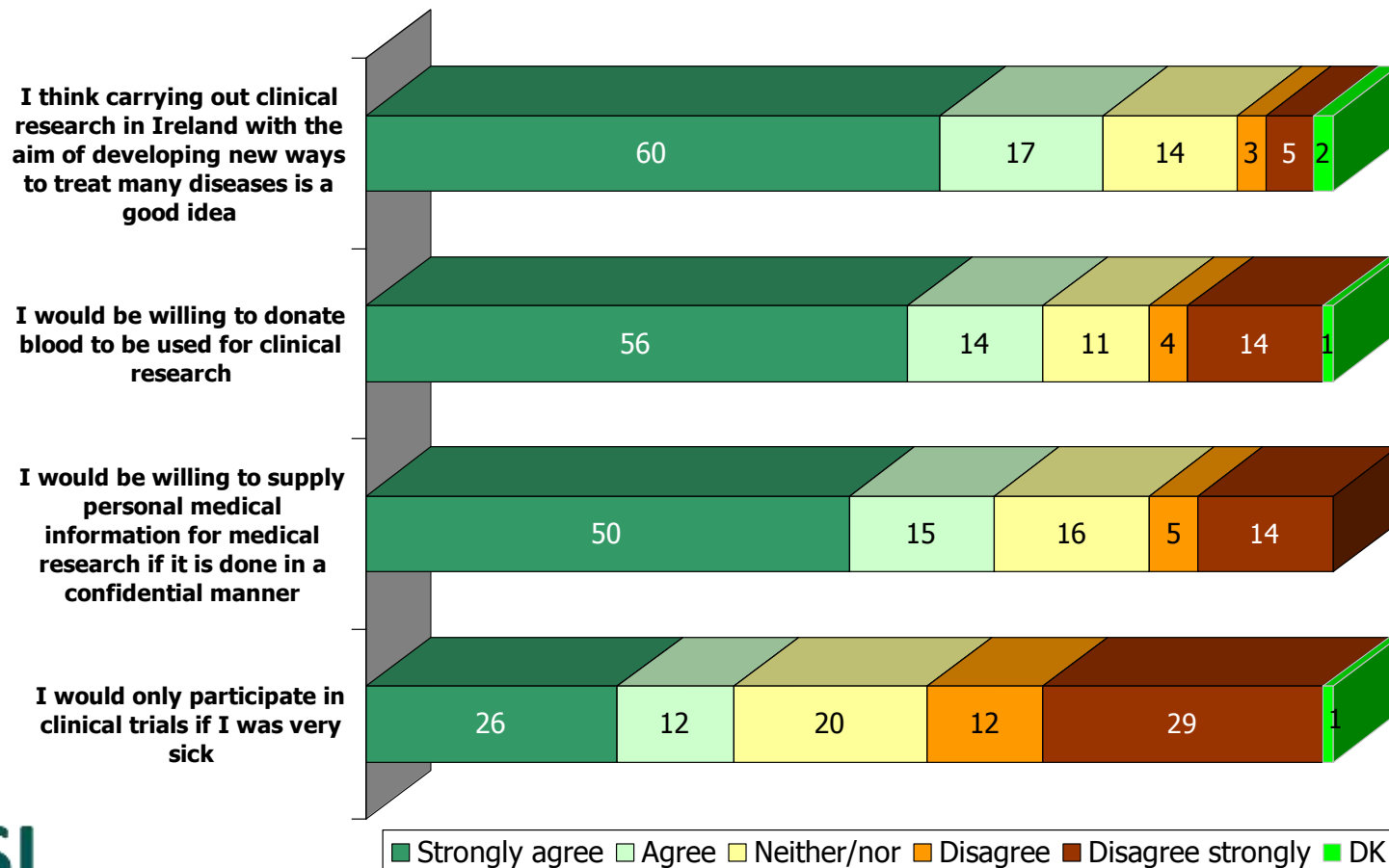


REPORT INTO THE GENERAL PUBLIC'S ATTITUDES TOWARDS CLINICAL RESEARCH

Prepared for IPPOSI
by
Drury Research
November 2009



Agree v Disagree with Attitudinal Statements RANKING 1 - 5



{Base: n=1000, All adults}

clinicaltrials.ie





2013 EXPANDING OUR CLINICAL TRIALS INFORMATION CAMPAIGN



NCRC

Developing written content based on EGAN & Patient Partner's material and NCRC knowledge

IPPOSI

Providing editorial assistance from a regulatory perspective and are developing a communications campaign

The project will produce age appropriate Information for children and parents who have been asked to participate in a clinical trial

Public and Patients want information ipposi.ie



“EPF has been a guiding light to work of IPPOSI in the context of new EU legislation and Initiatives eg Transparency Directive, Clinical Trials Regulation, Patient Involvement in HTA, Patient Compliance and Adherence, the Cross Border Directive etc.. Thank you!” Eibhlin Mulroe, 23rd May 2013



IPPOSI and the European Patients' Academy on Therapeutic Innovation


<http://www.patientsacademy.eu> – info@patientsacademy.eu



Medical landscape is transforming at a fast pace

Innovation transforms the lives of patients with serious, lifelong conditions:

- Molecular targets/pathways
- Genome sequencing,
- Translational research
- Personalized medicine
 - Small trial populations
 - Biomarkers, companion diagnostics
- Need for post-marketing data
- Health Technology Assessment, QoL, endpoints, comparators
- BUT long term pressure on health budgets – here to stay

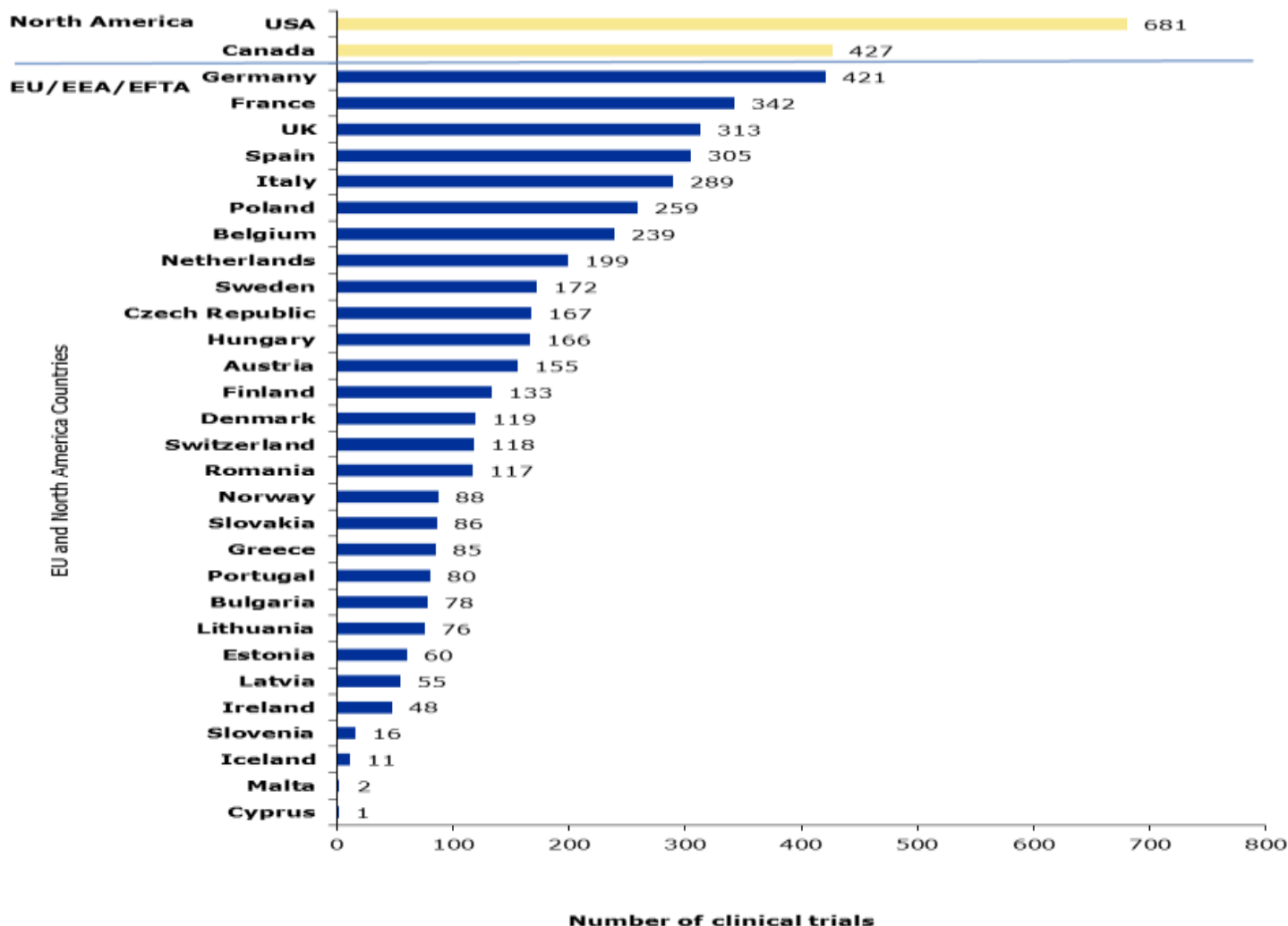


Window of opportunity

- trial design
- relationship between researchers, regulators, industry, **patients**

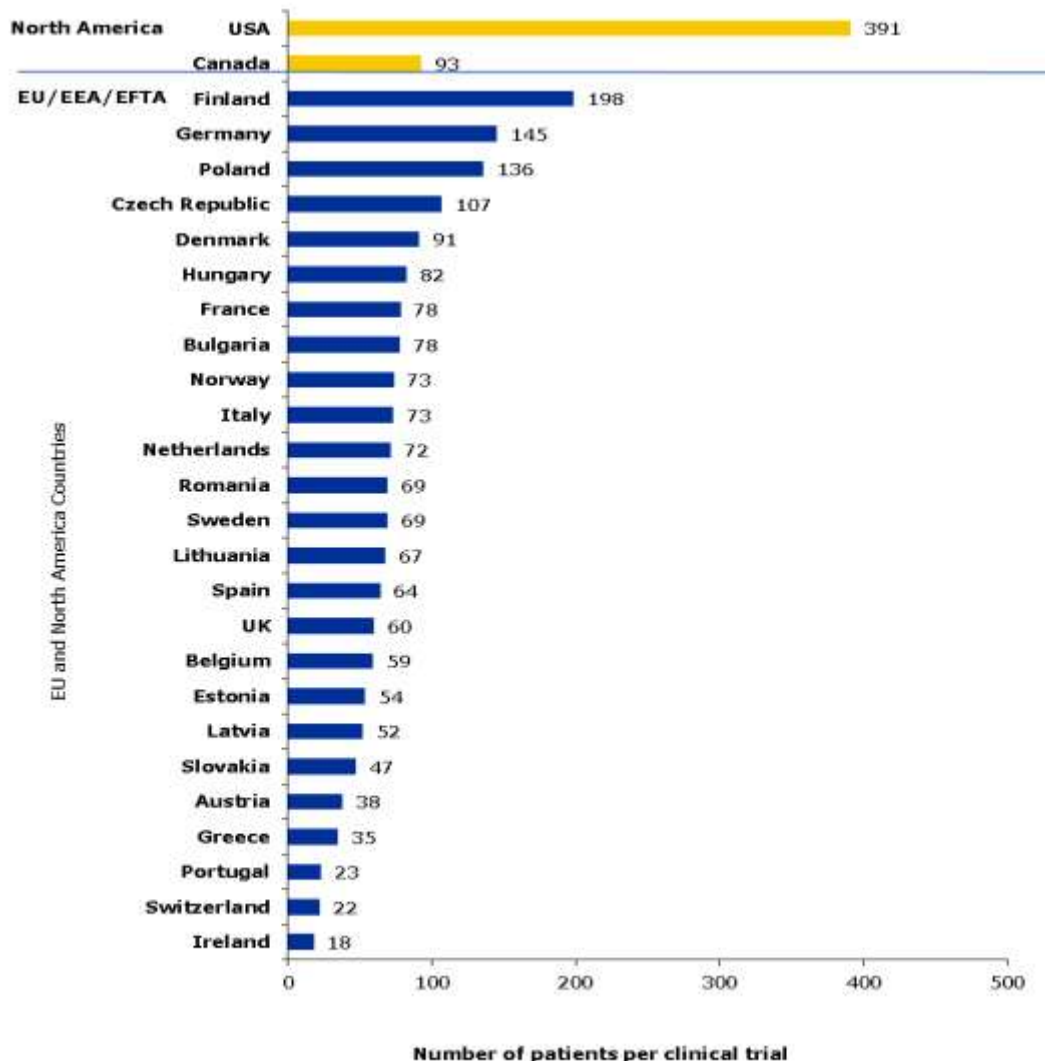
The number of pivotal clinical trials in MAA submitted to the Agency performed in each country of the North America and EU/EEA/EFTA regions in the 2005-2011

Clinical trials
submitted in
marketing-authori-
sation
applications to the
European
Medicines Agency
EMA/INS/GCP/676
319/2012



The average number of patients recruited per pivotal clinical trial per country in MAAs submitted to the Agency in the 2005-2011 period

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Patients as partners of research: More needs to be done!



“ *Rare cancers will never be a priority unless the patients make it one. **Patients themselves must therefore play a larger role in driving forward the search for therapies.** They are able to see connections that have eluded scientists.* ”

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JOURNAL OF CLINICAL ONCOLOGY

PERSPECTIVES IN ONCOLOGY

To Make Progress in Rare Cancers, Patients Must Lead the Way

Amy Dockser Marcus

Submitted January 9, 2009; accepted February 3, 2009; published online ahead of print at www.jco.org on May 4, 2009.

In January, 2004, I flew to New Orleans, LA, to meet Andy Martin. He took me to the laboratory where he was working. A third-year medical stu-

dent. They recognized that when it came to SNUC, Andy was in many ways the expert on the disease. These physicians learned from the research he did,

Patient advocates have a key role in building new environment for R&D

- Patient organisations have unique insights into „real life“ and „real needs“ of patients:
 - Gaps → research priorities
 - Clinical trial design
 - Quality of Life measurement
 - Real-world access to therapies
 - „Value“
 - Patient-centered research policy

Training essential to get experts to contribute to medicines research & development (R&D)

Patient Partner



With more than 200 cancers and >4000 rare diseases, we need many qualified patient experts!

Addressing public scrutiny and distrust of research...

- Only 6-12% of cancer patients participate in clinical studies
- 75% of Phase II-IV studies delayed due to slow patient recruitment
- Bad image one reason for delayed generation of meaningful clinical data

Europe has a lot of safeguards in medicines R&D – but public image lagging behind

MEDICINE AND THE MEDIA

“We saw human guinea pigs explode”

L Stobbert and colleagues examine newspaper coverage of adverse events in the TGN1412 trial



with death and disfigurement. Science fiction or cinematic imagery is often used to add potency to detailed and gruesome descriptions—although no pictures were printed of the victims' deformities, references such as “his face now resembled that of the Elephant Man” (*Daily Star*, 16 March) were used with effect.



Patient advocates working with regulators...



EMA track record since 2005...

- Patients' and Consumers' Working Party (PCWP, 34 POs)
- Full members of MA Management Board, COMP (rare diseases), PDCO (pediatric), CAT (advanced therapies)
- Assessment of EPARs, Package leaflets, safety information
- Ad-hoc support in CHMP: Product assessment, guidelines, Pharmacovigilance WG, protocol assistance
- Speakers and participants at EMA conferences/workshops
- AND AT THE NATIONAL LEVEL ... Patient involvement, by accident



Patient organisations need more qualified advocates to engage with regulators

Patient advocates through IPPOSI in Ireland working with...



Patient organisations need more qualified advocates to engage with regulators

- Irish Medicines Board
- Health Information and Quality Authority
- Department of Health Steering Group on Rare Diseases
- Health Service Executive Patient Forum
- National Centre for Pharmacoeconomics (HTA)



Having a patient (advocate) in every Research Ethics Committee...



Country	Inhabitants in 1,000 ^a	Number of ethics committees	Number of ethics committees (including local ethics committees)	Ethics committees per million inhabitants
Austria	8,356.7	27		3.23
Belgium	10,741.0	35	215	3.26
Bulgaria	7,602.1	103		13.55
Czech Republic	10,474.6	9	>100	0.86
Cyprus	801.6	1		1.25
Denmark	5,519.3	8		1.44
Estonia	1,340.3	2		1.49
Finland	5,325.1	25		1.49
France	64,105.5	29		0.45
Germany	82,225.3	29		0.35
Greece	10,029.9	1		0.09
Ireland	4,152.5	40		9.63
Italy	59,226.1	264		4.39
Latvia	2,261.1	5		2.21
Lithuania	3,350.0	1		0.60
Luxembourg	4,426.0	1		2.03
Malta	412.6	1		2.42
Netherlands	16,482.0	25		1.88
Poland	38,500.0	25		1.44
Portugal	10,620.0	1		0.09
Romania	21,496.7	1		0.05
Slovakia	5,411.1	9	89	1.66
Slovenia	2,053.4	1		0.49
Spain	45,853.0	136		2.97
Sweden	9,259.0	8		0.86
UK	61,612.3	126		2.05

If patients are to be involved in RECs on a broad level, we need more patients who understand how trials work

- 9.400 EU applications for clinical studies/year
- 5.000 clinical studies initiated in EU/year
 - 25% multinational = ~1250 studies/year
 - 4.5 Member States on average per multinational study
 - Single opinion per country assumed
- **For 1250 multinational studies, more than 5.000 ethics panels with 35.000 panelists needed**

Sources: Impact on Clinical Research of European Legislation (ICREL), Final Report, Feb. 2009, and Rokus de Zeeuw 2010

Having a patient's voice in pharmaceutical policy at both European and national level



– needs qualified patients

EPF survey on HTA agencies, decision makers and patients



- Patient involvement in HTA has the most impact in putting forward patients' needs in terms of QoL and providing a real-life context to the use of health technologies; this is acknowledged by all
- To facilitate patient involvement, HTA agencies and decision-makers provide access to
 - HTA reports/guides/protocols,
 - easy-to-read HTA summaries
 - – but no training support for patients

Patient organisations need more capacity to engage on HTA





**Patients want a seat at the table.
Currently, there are many empty seats.**

**This is why we have
established the
European Patients' Academy (EUPATI).**

Key stakeholders of the Patients' Academy



Audiences: advocacy leaders and the public at large



EUPATI Certificate Training Programme

- Academic Modular Certificate Programme
- Patient Ambassadors in committees, R&D teams, ...
- Patient Journalists raising awareness
- Patient Trainers for patient communities & networks

100
patient
advocates



EUPATI Educational Toolbox

- Educational tools for patient advocates
- Variety of distributable formats: Paper-based booklets, presentations, eLearning, webinars, videos etc.

12.000
patient
advocates



EUPATI Internet Library

- Patients & lay public at large, e.g. on specific aspects of the development process of medicines for patients with low (health) literacy.
- Wiki, YouTube, films and/or cartoons

100.000
individuals

Patients' Academy: up, running and real. Workshop, 5 Sept 2012 and National Liaisons Workshop in March 2013



- ~100 participants from 24 countries
- Majority patient advocates
- 14 countries building national platforms



EUPATI: A paradigm shift in empowering patients on medicines R&D



- ▶ Launched Feb '12, runs for 5 years, 30 consortium members, PPP of EU Commission and EFPIA
- ▶ will **develop and disseminate objective, credible, correct knowledge about medicines R&D**
- ▶ will **build competencies & expert capacity** among patients & public
- ▶ will **facilitate patient involvement in R&D** to support industry, academia, authorities and ethics committees



EUPATI by 2017: Where we want to be.



- ▶ EUPATI platform complete with training courses, education, information material in multiple languages
- ▶ Good practice guidelines on patient involvement available and in use
- ▶ Public conferences and regional workshops will lead to an extensive expert network established.
- ▶ 12 National Platforms established in 12 countries
- ▶ Robust strategy on sustainability and political buy-in



**EUPATI can make the difference.
creating the tipping point for patient
engagement in medicines R&D**

It's for all of us to make it happen.

Get to know us!



Web:
www.patientsacademy.eu

Twitter: @eupatients
 as well as:





More Information

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[@ipposi](https://twitter.com/ipposi)