

(Why should +) How can patient advocates be more involved in clinical research?

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Some Of The Challenges Of Rare Cancers...

Prevention and screening
mostly irrelevant

Late or incorrect diagnosis

Lack of information and
patient groups

Widespread, small
populations of patients

Shortage of local
medical expertise

Incomplete registries
and tissue banks

Methodological barriers
(in traditionally-designed
clinical trials)

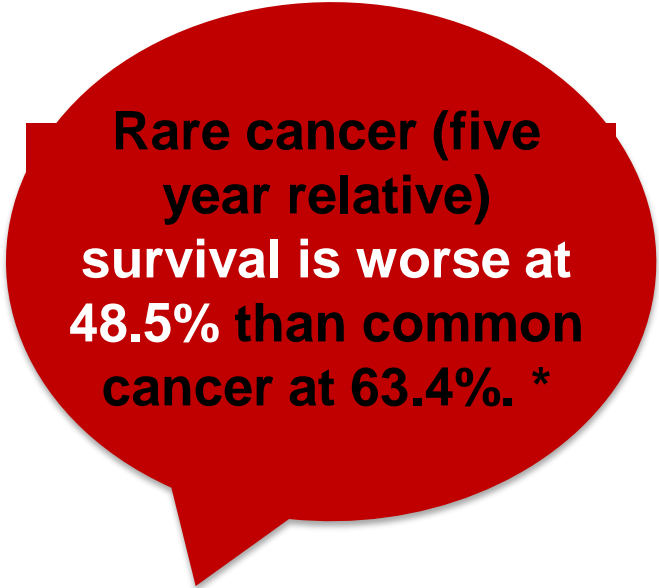
Not enough clinical trials
for rare cancers

Regulatory barriers

Reimbursement
challenges for treatments

Inequitable access to
therapies and care

Stigma



**Rare cancer (five
year relative)
survival is worse at
48.5% than common
cancer at 63.4%. ***

* Lancet Oncol. 2017 Aug; 18 (8) 1022-1039

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Several of these
challenges have
directly or indirectly
to do with deficits in
cancer research!

Why “Patient Involvement In Clinical Research”?

And why should patient advocates be interested in?



The meaning of medicine & health care

>>> to support / to benefit people!

**Patients (relatives) must be the focus –
they are the real "customers"**

- Everyone can be a patient / relative tomorrow (including doctors, researchers, politicians!)
- Patients = are a cross section through our society
- Patients are citizens, voters, taxpayers, contributors!!!
They/We have rights to be involved, to be listened to!

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Patients bear the ultimate risk in clinical trials:
The "risk for their lives" – through e.g.

- new drugs in clinical trials
- wrong research questions
- inappropriate study designs
- missing / insufficient information
- delays in research & development
- lack of access to therapies due to cost...
- etc.

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Patients know best – what it means to have a disease, to live with a special condition!

- Patients are the real experts!
- The views of doctors and patients can differ
- Rare Cancers: Patients often know more than their doctors
- Patient Groups have access to the very first source of information – to the patients / relatives
- New field: Personalised medicine / precision oncology...

Increasing Criticism Of The “Old” Study System...

- Focus on the patient: often ignored / neglected
- Results of Clinical Trials: often far away from real life and real needs
- Over 1 million studies published worldwide: Some experts complain: 85% are useless, waste of money
- A lot of resources are spent into: Doing things right – instead of – doing the right things
- Challenges with Clinical Trials in Rare Cancers: Clinical evidence - is more difficult to build...
- Difficult: Gaps between FDA/EMA approvals and HTA
- It is evident, that cancer is a very individual disease. Personalized oncology (precision oncology) is the future. We need better concepts to do target-oriented research...

IS THE CLINICAL TRIAL SYSTEM BROKEN?

[PLoS Med.](#) 2016 Jun; 13(6): e1002049.

Published online 2016 Jun 21. doi: [\[10.1371/journal.pmed.1002049\]](#)

Why Most Clinical Research Is Not Useful

[John P. A. Ioannidis](#)^{1, 2,*}

Urologic oncology survey

Laboratory research

Why most published research findings are false: Ioannidis JP, *Department of Hygiene and Epidemiology, University of Ioannina School of Medicine, Ioannina, Greece*

September 21, 2017

'Clinical Trials System is Broken,' FDA Drug Chief Says



Annals of Oncology
Volume 26, Issue 2, February 2015, Pages 300-306



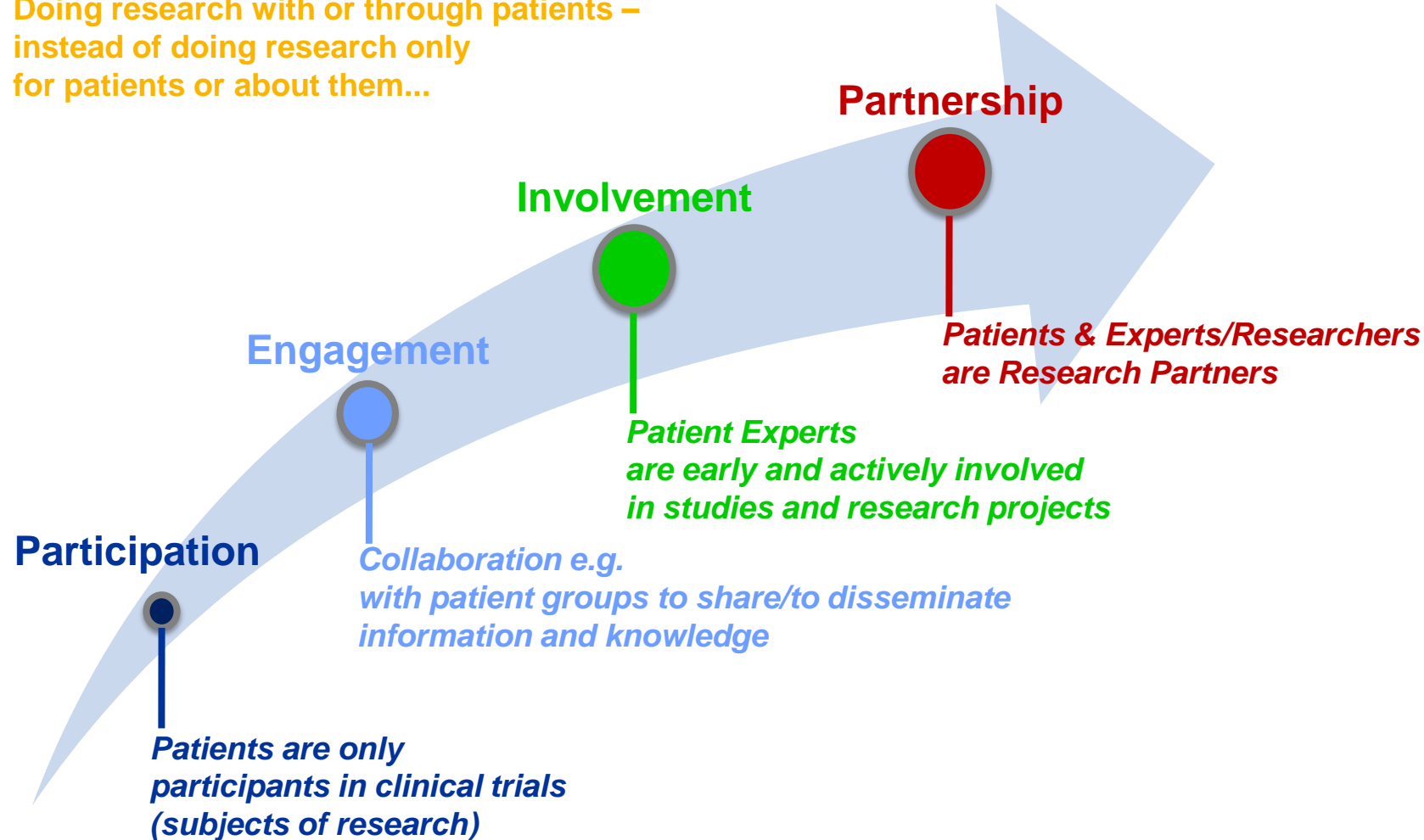
reviews

Rare Cancers Europe (RCE) methodological recommendations for clinical studies in rare cancers: a European consensus position paper

Patient Involvement In Clinical Research...

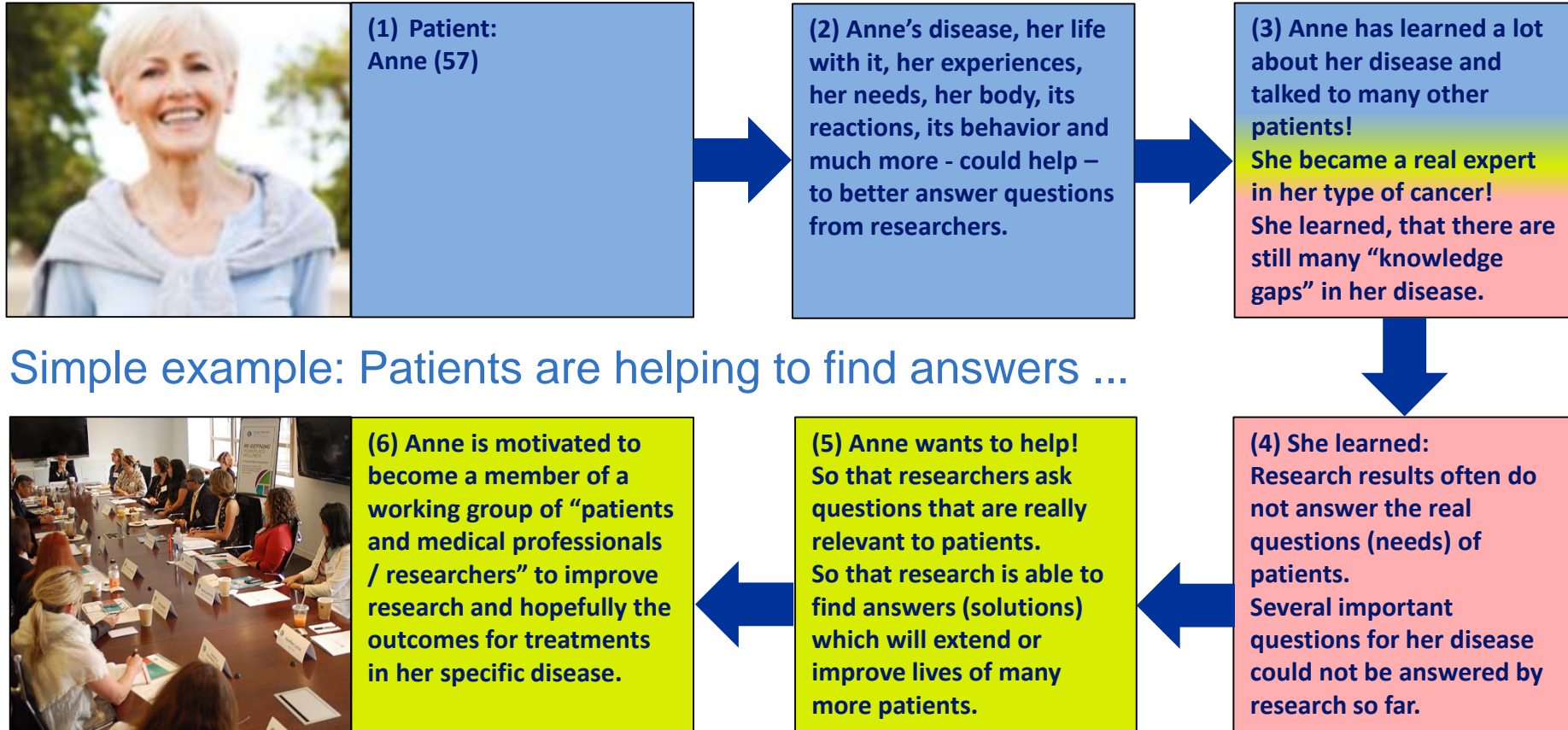
In the future:

Doing research with or through patients –
instead of doing research only
for patients or about them...

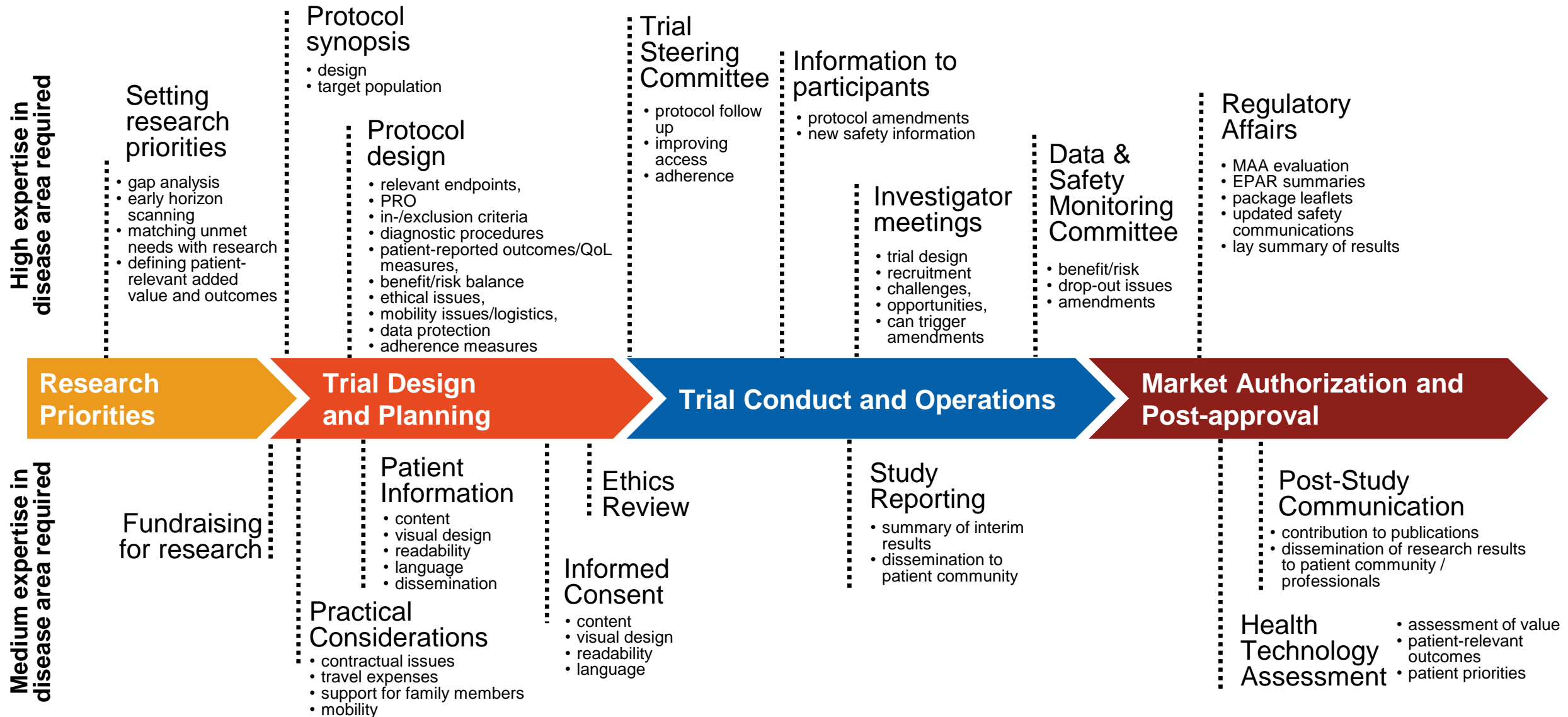


- *Research = better - faster – more efficient and more meaningful = closer to real life / to real needs*
- *studies = part of cancer treatment and part of the patient experience*
- *better recruitment*
- *change of scientific culture*
- *more public confidence in research*

Patient Involvement In Clinical Research...



Patient Involvement In Clinical Research (Drug Development In Practice)



Some Aspects For “Rare Cancer Patient Organisations” ...

“Patient Involvement in Clinical Research” ...means...

...enormous opportunities for change...

...to understand how research works and which language is spoken =
Basic Research Training!

...to understand how research in your country and your disease is organised...

...close peer to peer cooperation with experts, researching companies and other stakeholders...

...to prepare your organisation to be part of the research process...

...to analyse and define where in your disease the needs/gaps are and the research priorities should be...

...a general changing mindset, that this topic is a **core success factor** for research and not just a nice PR idea...

...to understand that countries are differently developed on this topic. E.g. UK has a history in cancer for more than 20 years...

...(maybe) to unite with other patient organisations to make general progress on the topic...