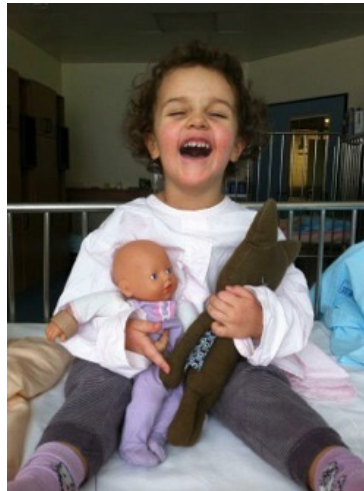




Training patient representatives to work with NF researchers

Ing. **Claas Röhl** Bakk phil.

NF Kinder/ GENTURIS/ Pro Rare Austria/ EUPATI Austria







Patienteninvolvement means to include patients/ patients representatives as equal partners in the whole decision making process

- medical/ psychological care
- health care politics
- R&D

Why?

Motivation

Patients/ their relatives want NF research to be successful more than anyone else.

Fairness

Nothing about us without us

Why?

Motivation

Fairness

Experience

Patients/ Relatives have unique every day experience with NF

Patients can define:

- factors that affect QoL / social factors
- unmet needs
- patient reported outcomes, patient relevant outcomes, endpoints

Why?

Motivation

Fairness

Experience

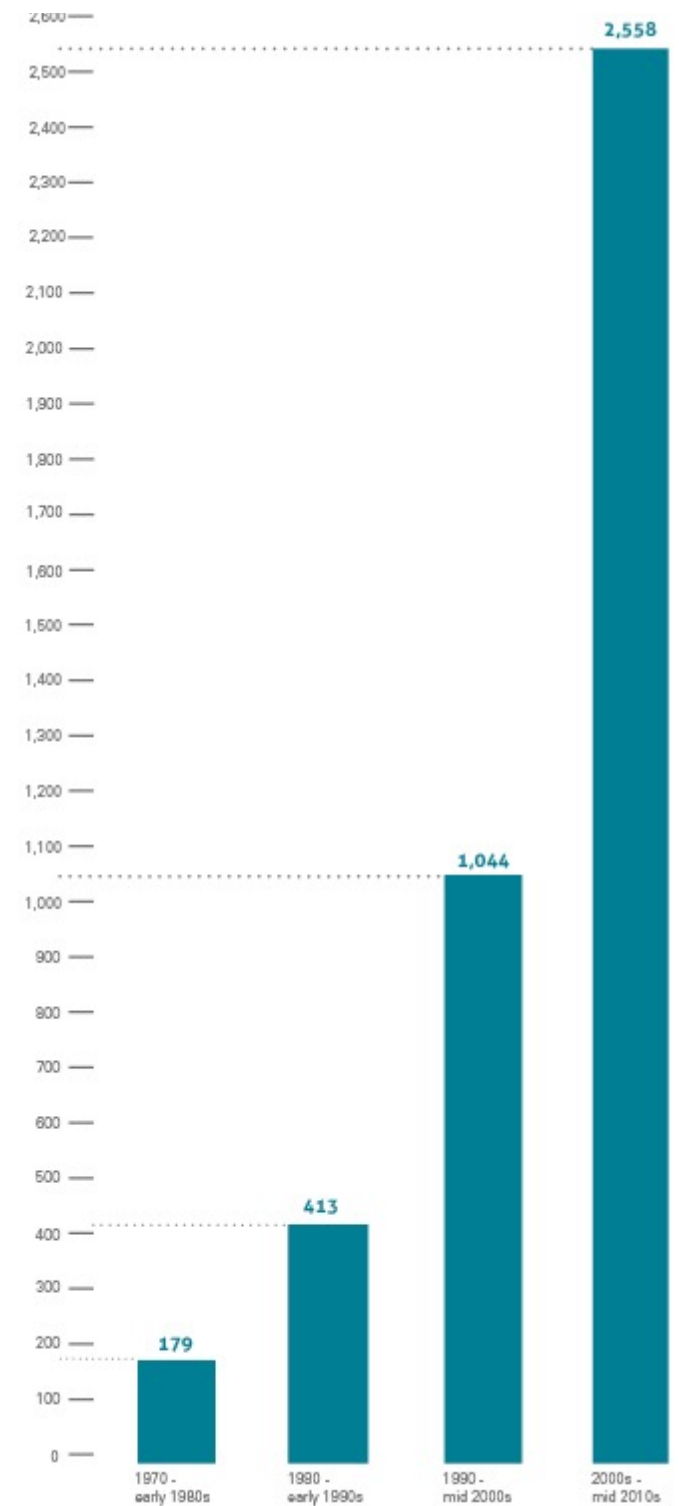
Change of course needed

- R&D gets more and more costly
- strategies to improve efficacy and efficiency are needed



cost development in R&D

source: Joseph A. DiMasi, Henry G. Grabowski, Ronald W. Hansen, Innovation in the pharmaceutical industry: New estimates of R&D costs, Journal of Health Economics, 47 (2016), 20-33



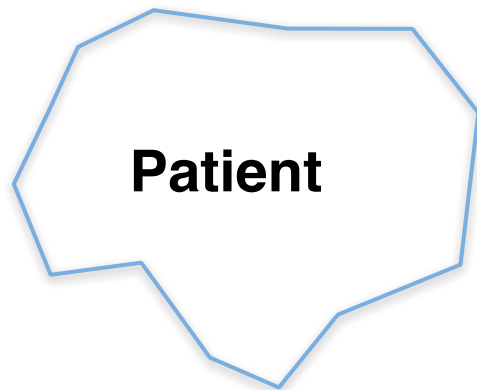


Motivation

Fairness

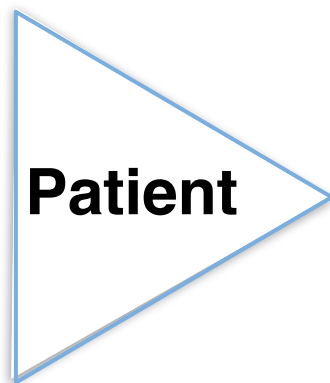
Experience

Change of course needed





Motivation
Fairness
Experience
Change of course needed
Knowhow



<- language ->





Snapshots



Translation: "You have a bruised rib."



goal: patients are equal partners

Motivation

Fairness

Experience

Change of course needed

Knowhow

Patients

Clinicians/

Researchers

Industry

Regulatory bodies

Health Care Politicians

Patients as partners: needs cultural change and training for patients



role of patients in R&D (Academia + Industry)

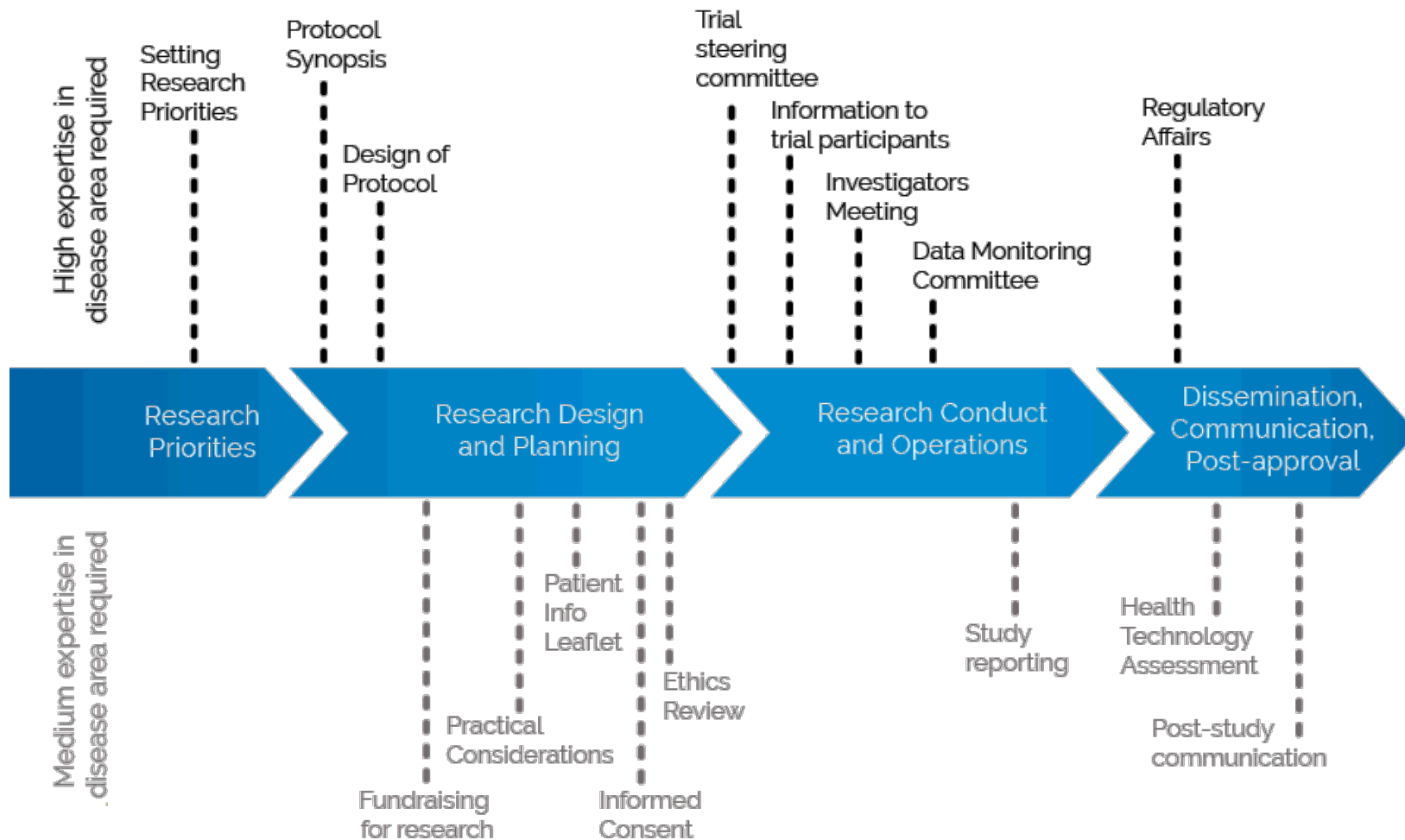


Source: PatientPartner
FP7 Project (2010)

Patient involvement in practice within the R&D life cycle

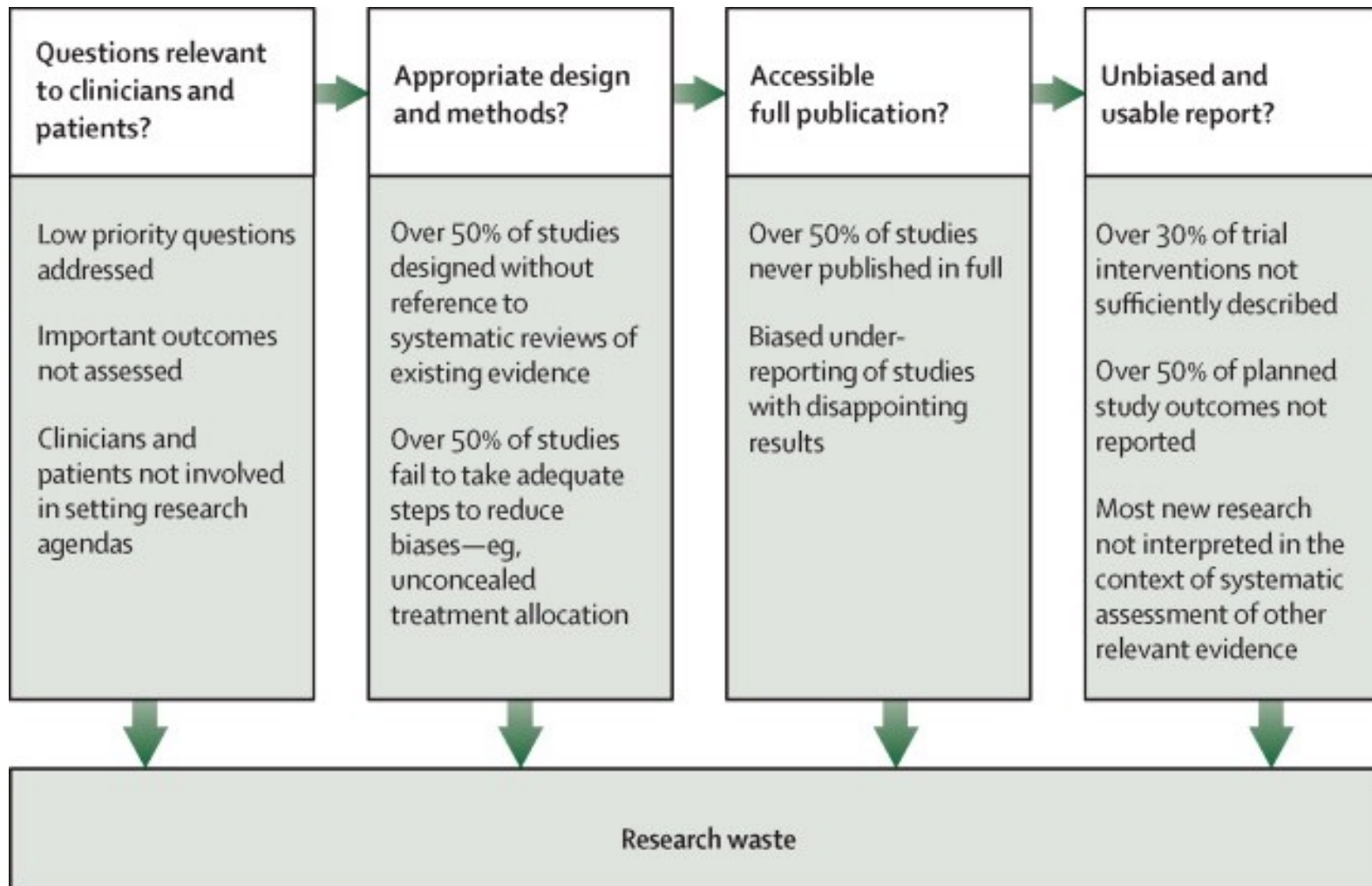


Source: Geissler, Ryll, Leto, Uhlenhopp – www.eupati.eu



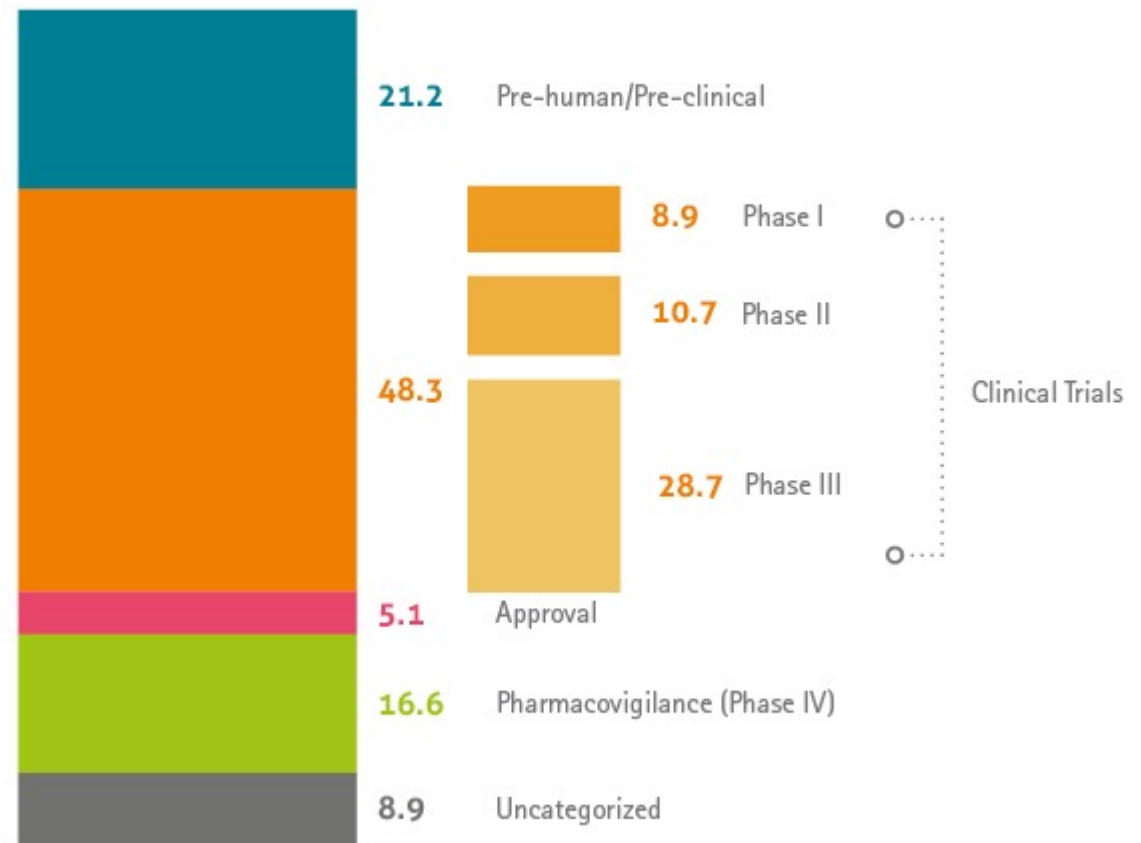
Source: Geissler, Ryll, Uhlenhopp, Leto (2016): www.eupati.eu

Faktor Forschungsprioritäten





cost distribution in R&D



PhRMA, Annual Membership Survey 2016
(percentages calculated from 2014 data; total values may be affected by rounding)



Patient Recruitment for clinical trials

Patient recruitment costs constitute about 1/3 of the costs for clinical trials

about 400 billion USD



Patient Recruitment for clinical trials

Study at Kings College in London showed:

Patient involvement in study design and implementation contributes to reaching the recruitment targets.

When patients and public partners were involved, achieving recruitment target was improved by 4,12

could translate to save 100 billion USD



summary

Patient involvement improves:

- quality and relevancy of research.
- chance of a drug getting approval.
- efficacy and efficiency - saves costs



Training for NF patient representatives



NF Kinder

Neurofibromatose durch Forschung besiegen

My trainings so far:

- Fundraising-College 2013/2014
- Fundraising summer school 2015
- EURORDIS Summerschool 2015
- EUPATI patient expert training course 2015/2016





NF Kinder

Neurofibromatose durch Forschung besiegen

Topics

1. Discovery of Medicines
2. Pre-clinical Development
3. Clinical Development
4. Clinical Trials
5. Regulatory Affairs, Drug Safety, Pharmaco-vigilance
6. Health Technology Assessment



my experience - my advice

1. learn about the whole life cycle of drug development

- patient input is valuable in every phase of R&D
- early decisions have an impact on all following steps
- patient input in FDA and HTA discussions may decide about the final decisions

approval?

reimbursement?



my experience - my advice

2. interact

- inspire each other
- learn from each other
- discuss what you learned

discussion forums (reflective questions), face to face sessions, webinars, workshops, role plays,.....



my experience - my advice

3. represent the whole NF population

- bring in personal experience
- try to represent the whole patient community
- chance to set the right priorities
- chance to compare between different subpopulations countries

patient surveys, qualitative interviews, patient registries,....



my experience - my advice

4. don't be afraid to ask questions

- it's no shame if you aren't sure about a topic
- it is absolutely ok to question the point of a doctor/ researcher and start a discussion
- ask if you feel that a change of the format would improve your ability to give input (another time table, another way of communication,...)



my experience - my advice

5. cross-working group interaction

- enable exchange between the patients of the different working groups
- online-plattform (f.e. facebook workspace)

Patient education!

The A to Z of medicines development

Search the Toolbox by keyword

Browse the Toolbox by category

Basics of Medicine Development

General description of the basic concepts and processes of R&D in medicines.

Types of Medicines

Explanation of traditional and innovative medicine types and perspectives of use.

Drug Discovery

From the discovery of molecules to the explanation of how diseases affect humans.

Safety of Medicines

Concise, comprehensive description of the complex mechanisms to keep medicines safe.

Pharmaceutical Development

Explanation of the various methodologies of how medicines are and can be developed.

Clinical Development and Trials

Introduction to research methods and the conceptual description of trial phases.

Personalised Medicine

Benefits and challenges in developing, delivering medicines tailored to individuals.

Benefit and Risk Assessment

Risks and benefits of medicines, and the elaborate system of pharmacovigilance.

Regulatory Affairs

Regulatory systems for medicines from EMA to prescription and off-label use.

Health Technology Assessment

Explanation of HTA methods and processes, and patient involvement mechanisms in HTA.

Non-Clinical Studies

Medicines R&D involves more than clinical trials: translational medicine explained.

Pharmacoepidemiology

Exploration of various epidemiological aspects relevant for medicine development.



NF Kinder

first Austrian NF department

co-run by Medical University of Vienna and NF Kinder

- 1 doctor, 1 psychologist, 1 study nurse





NF Kinder

first Austrian NF meeting

educational event

50 doctors from Austria and abroad

networking dinner





NF Kinder

first Austrian NF meeting

2 patient days

work shops, panel discussions, talks
side program, dinner





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Thank you!





NF Kinder

Neurofibromatose durch Forschung besiegen

Results:

- listed patient expert for NF at EMA
- involved in setting up ERN GENTURIS (genetic tumor risk syndromes)
- patient representative on the executive board of GENTURIS
- founder of European NF umbrella organization (NF Patients United)
- patient representative in REiNS working group for cutaneous neurofibromas