VSOP DUTCH GENETIC ALLIANCE

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Patients Network for Medical Research and Health

Enpr- EMA: a patient / parent perspective

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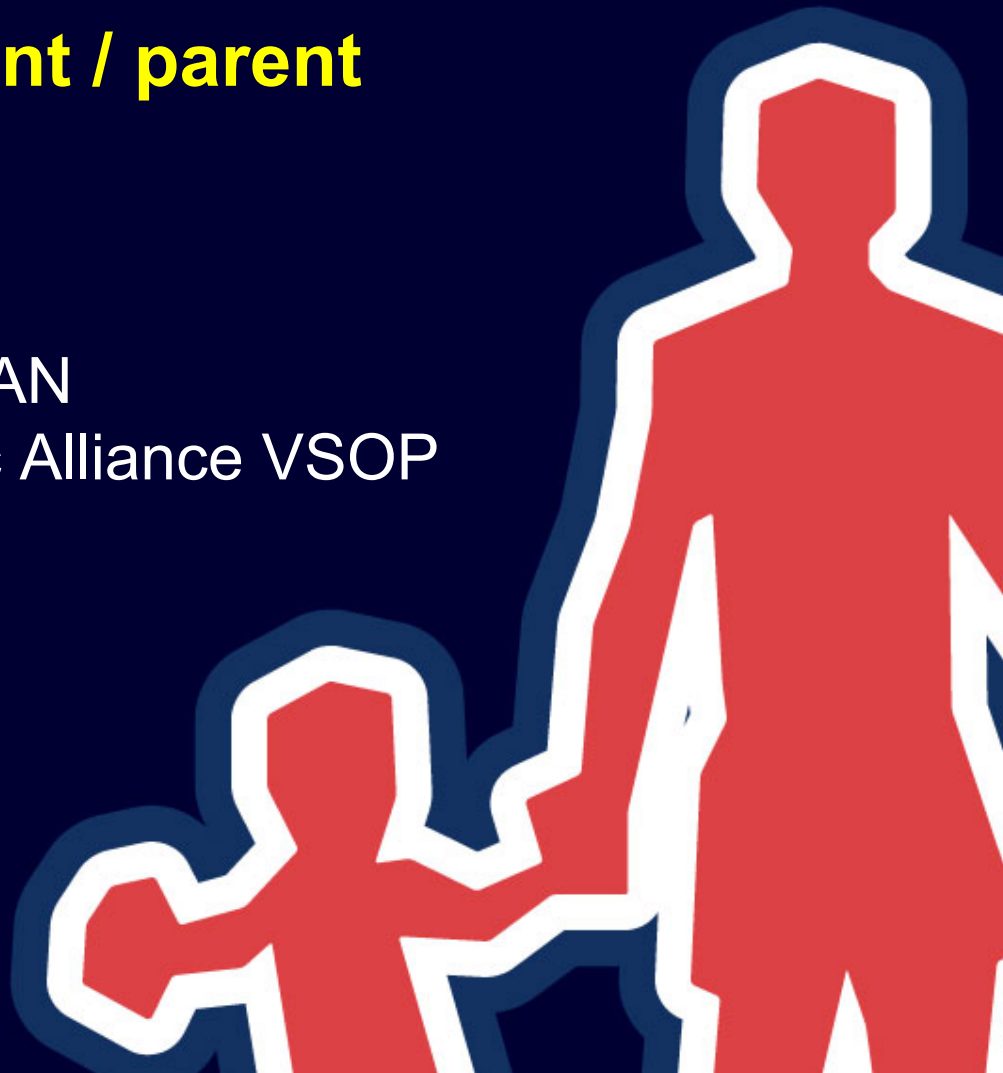
Secretary General, EGAN
Director, Dutch Genetic Alliance VSOP

EMA, London
11 March 2011



VSOP Mission EGAN

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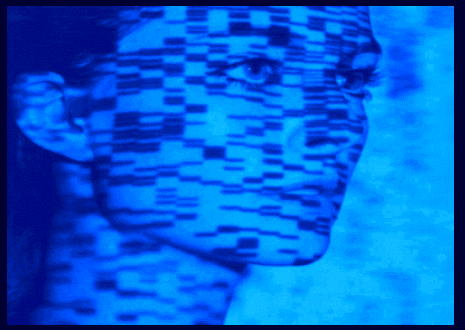
a. to create a patients' voice in health policy and health research,

b. to seek a world in which genetic, multifactorial and congenital conditions are diagnosed in it's early stages of development, understood, effectively treated, prevented

c. and the people involved adequately supported

Rare *and* common disorders

Genetic Alliance UK
Supporting. Campaigning. Uniting.



Burden of disease

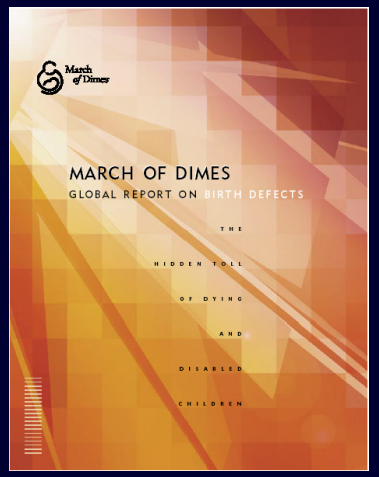
Annually
- 8 million children born with a serious disorder
- 3.3 million children under the age 5 die

for those who survive,
lifelong intellectual, physical, auditory & visual disability".

Every day
- 30.000 children under age 5 die from **preventable causes**

Prevention
- **50 – 70 % can be prevented by**
  - **better infra structure and information/education**

Developing world
- **90% of all children born in medium**
- **& low income countries**



UN Millennium development goals 2015

Reduce child mortality by 2/3

Improve maternal health by 2/3

Participation Ladder

Patient Partner

VSOP Added value of pp

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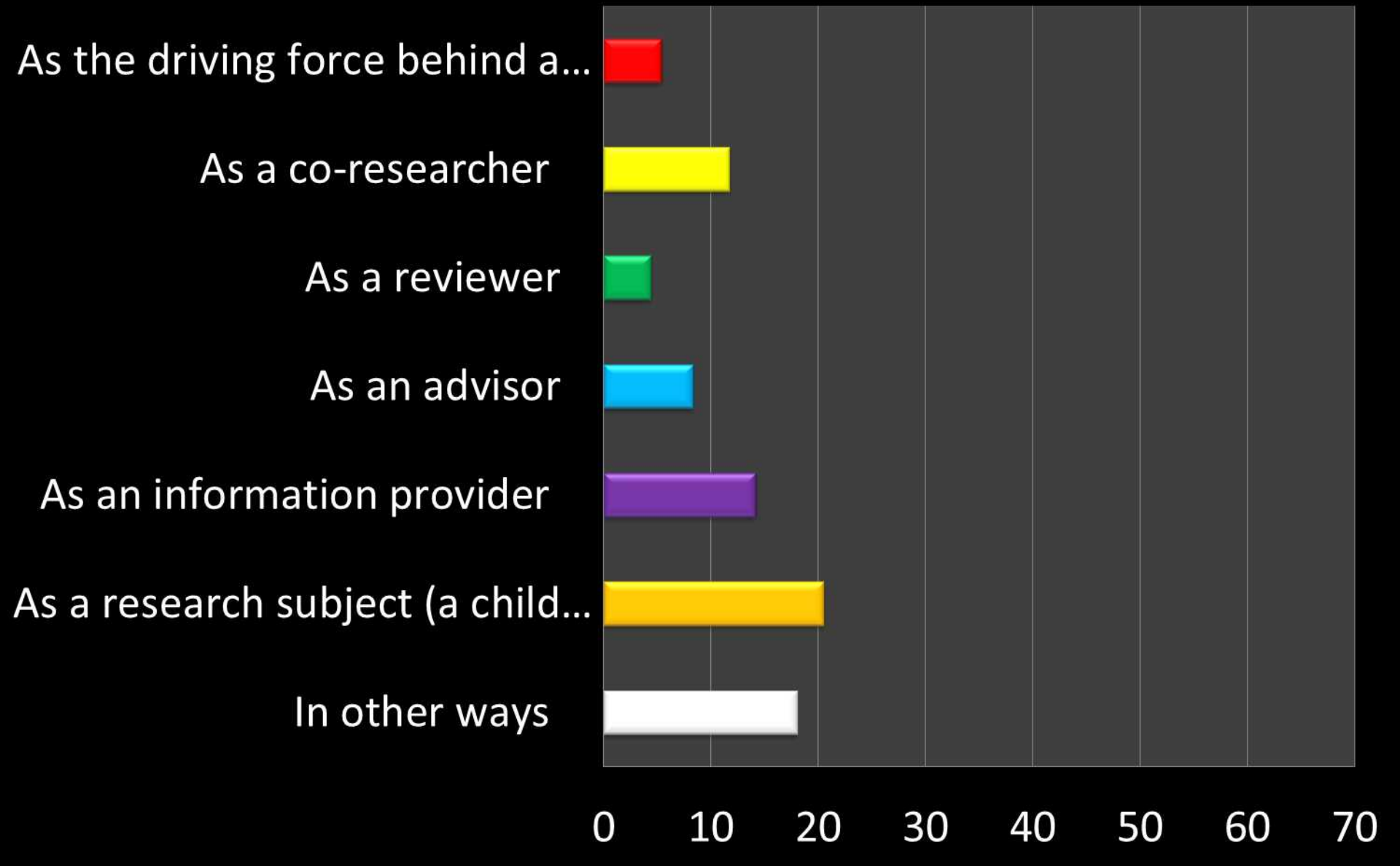
Before the CT
- Identification of indications, therapy features, patient population
- Patient perspective on ethical and risk/benefit dilemmas
- Defining patient-oriented outcome measures

During
- Managing of expectations: hope or hype
- Patient inclusion and compliance ↗
- Data quality ↗
- Patient and public confidence in clinical research ↗

After:
- Quality of life, quality of healtcare ↗
- Therapy compliance↗
- (Cost-)effectiveness ↗

Patient Involvement in Clinical trials with children



Enpr- EMA: Criteria public involvement

Involvement in:

1. Prioritisation of needs
2. Protocol design
3. Creating the protocol information package

VSOP Enpr- EMA - We offer

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- Our sick children
- Our partnership
- Our experiences
- Our expertise
- Our ethics
- Our public, societal influence
- Our registries
- Our network(s)

VSOP Enpr- EMA - We expect

- Passion
- 5Ps: PPPPP
- Early and structural partnerships
- No tokenism
- Infrastructure for:
  - Long-term follow-up (shares responsibility)
  - Patient registries
  - Matching patients and (clinical) research)
  - Information / education / support

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Some thoughts after listening

GRiP – Global Research in Pediatrics

- Dissemination / networking: Facilitate patient engagement, identification of suitable patient groups.
- Develop training program: modules for patient groups
- Interoperability: harmonisation of patient information
- New methods: extrapolation, sample size, comparator
- Formulations: patient preferences

EUPATI: European Patient Academy On Therapeutic Innovation: IMI pre-proposal approved

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biomedinvo4all
Roadmap to Treatment

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Biomedical research

- Medical data / biobanks
- Medical genetics
- Animal research
- Clinical Trials
  - Basics
  - Ethics
  - Politics
  - Getting Involved
  - Glossary
  - Links
  - Trials for children
    - Basics
    - Ethics
    - Politics
    - Getting Involved
    - Links
- Pharmacogenetics
- Gene therapy
- Stem cell therapy
- Future themes

Trials for children

Many medicines administered to children have not been specifically developed for them. This means that no paediatric clinical trails were conducted to determine whether the drug is safe and effective in children.

Medicines authorized for adults are usually administered to children by decreasing dosages on the basis of weight, which is extremely hazardous. Children are not small adults and respond very differently to medicines at various ages and stages of their development.

This use, defined as off-label in children, is widespread and has been an increasing concern over the last years. Serious consequences, inefficacy and side effects often stem from incorrect dosage. In the European Union (EU), fifty per cent or more of medicines used in children have never actually been studied in this population, but only in adults, and not necessarily in the same indication (or the same disease).

The need for more studies to obtain paediatric information for medicines used in children is now a matter of consensus on a global basis. This section provides information on the necessity for clinical trials with children, and what ethical and political aspects are involved. It also provides links to other websites you can visit to look for answers to your questions if you or your child is getting involved in a clinical study.

Click on the relevant subsections in the left panel to go to the topics you're interested in.

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FAQ on social and psychological aspects of medical testing

This leaflet aims to provide ideas and guidance for patients and families who are offered diagnostic or predictive medical testing of one sort or another. It builds on the shared experience of pati...
Read more >>

FAQ on Clinical Trials (various languages)

This booklet brings together some of the questions that are frequently asked by those thinking about joining a clinical trial Read more >>

FAQ on Personalised Healthcare (various languages)

In one sense, personalised healthcare is nothing new. It is what doctors have aimed to provide for their patients through the exercise of their clinical judgement, backed up by specialist knowledge... Read more >>

FAQ on Biobanks (various languages)

To help promote understanding and to secure informed involvement with the activities of biobanks and the research they make possible, we have assembled a list of questions that have been frequently...
Read more >>

Patients Handbook on Stem Cell Therapy

Like a new drug, stem cell therapies must be assessed and meet certain standards before receiving approval from national regulatory bodies to be used to treat people. What does this really mean for...
Read more >>

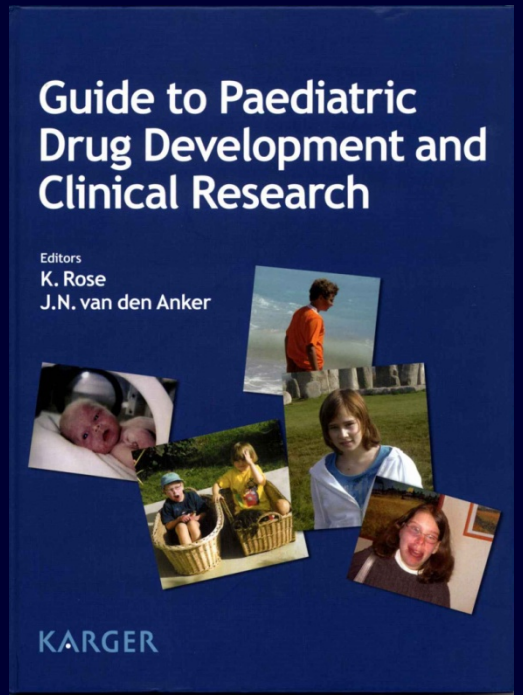
For further reading

Paediatric Clinical Research: The Patients' Perspective

Alastair Kent [a,b] *Cor Oosterwijk* [b,c] *Ysbrand Poortman* [d]

In: Guide to Paediatric Drug Development and Clinical Research

[a] Genetic Interest Group, London, UK;
[b] European Genetic Alliances' Network, Brussels, Belgium;
[c] Dutch Genetic Alliance VSOP, Soest,
[d] International Genetic Alliance, The Hague, The Netherlands



Thank you for your attention

Grazie, faleminderit, благодаря, gràcies, tak, Danke, tänan, kiitos, merci, σε ευχαριστώ, köszönöm, hvala ti, paldies!, ačiū!, grazzi, bedankt, takk, спасибо, dziękuję, obrigado, mulțumesc, хвала ти, ďakujem ti, hvala, gracias, děkuji ti, teşekkür ederim, tack

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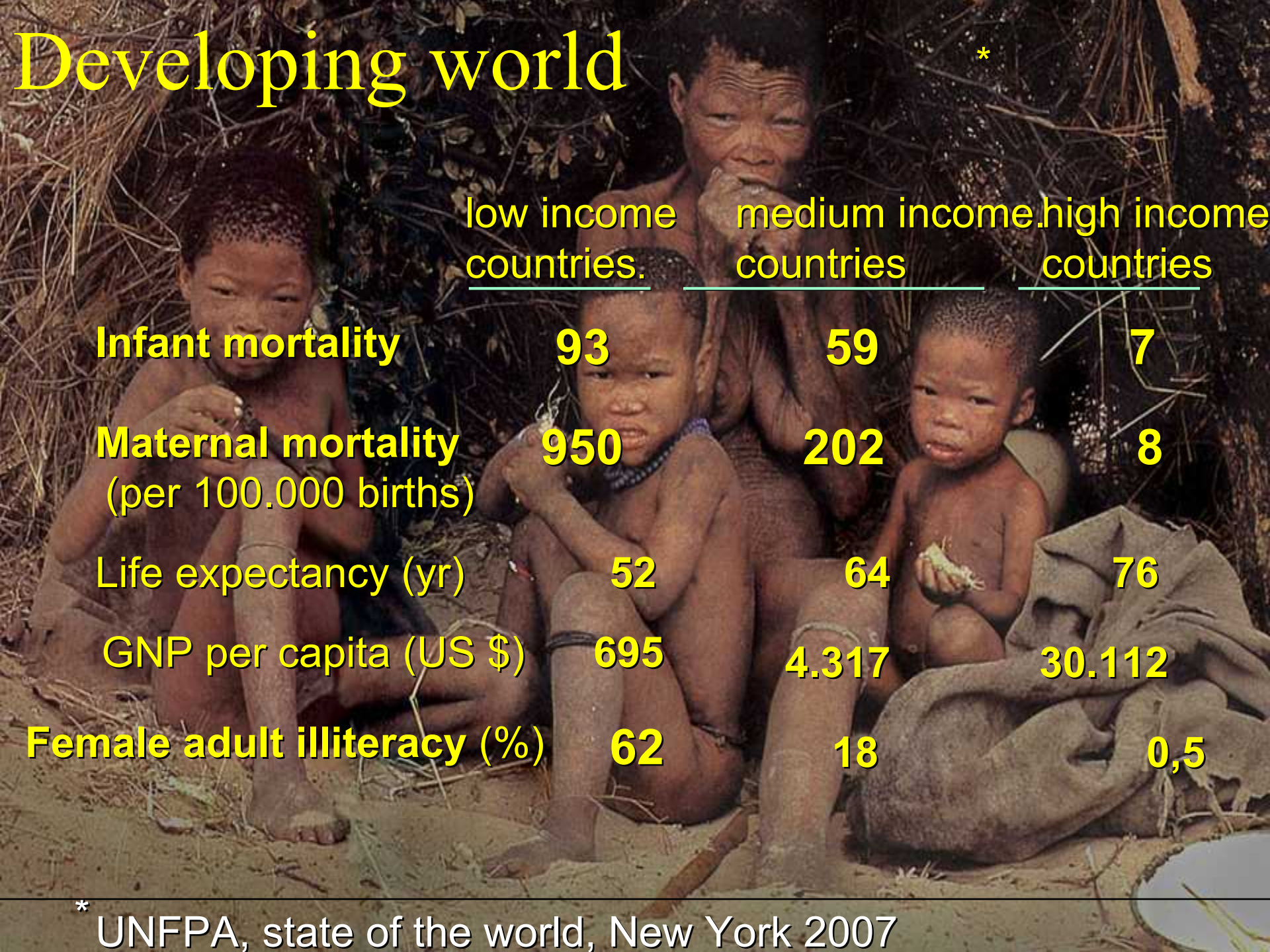
Developing world *



	low income countries.	medium income countries	high income countries
Infant mortality	**93**	**59**	**7**
Maternal mortality (per 100.000 births)	**950**	**202**	**8**
Life expectancy (yr)	**52**	**64**	**76**
GNP per capita (US $)	**695**	**4.317**	**30.112**
Female adult illiteracy (%)	**62**	**18**	**0,5**

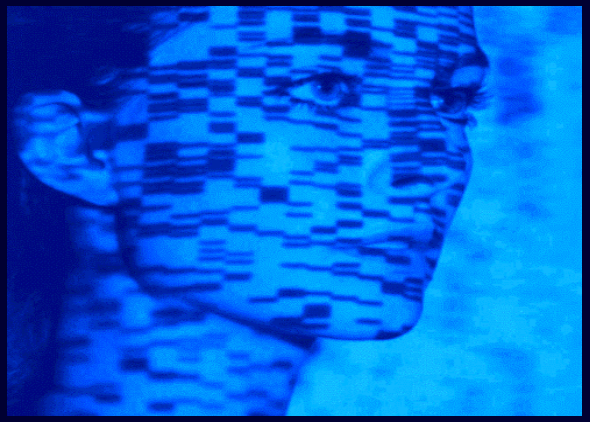
* UNFPA, state of the world, New York 2007

Related medical/policy areas

Integral approach:

- Preconception care / screening
  - Genetic
  - Environmental / nutrition
  - Disease, medicines,
  - Lifestyle
- Prenatal care / screening

- Orphan diseases
- Personalised healtcare
- Research policy & funding
- Ethical review
- Personalised medicine
- Patient registries and biobanks



VSOP Network

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- European Society Human Genetics (ESHG)
- International Society Neonatal Screening (ISNS)
- European Platform of Patients' Organizations, Science and Industry (EPPOSI)
- European Forum for Good Clinical Practice (EFGCP)
- World Alliance of Organizations for the prevention and treatment of genetic and congenital conditions (WAO)
- European Medicines Agency (EMA)

EF GCP

EPPOSI
European Platform for Patients' Organisations, Science and Industry

Earlier & current projects

- NL EU Presidency Congress (2004): Priority medicines for children

- CONSERT (gene therapy)
- GenGuide (genetic databases)

- GenCodys (mental disorders)
- PatientPartner
- Value + (EPF)
- EUPATI (IMI): European Patient Academy On Therapeutic Innovation
- ReTrac: Research Ethics Training Course
- Preparing for Life
- Nutrition & health

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Enschede, 13 May, 2000

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- Firework disaster Enschede
- 23 victims, including children

Pictures kindly provided by prof. Martina Cornel, VUMC Amsterdam

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Dutch Health Council: Investment agenda for medical products

Een investeringsagenda voor onderzoek naar innovatieve en relevante medische producten

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Importance of timely diagnosis and treatment

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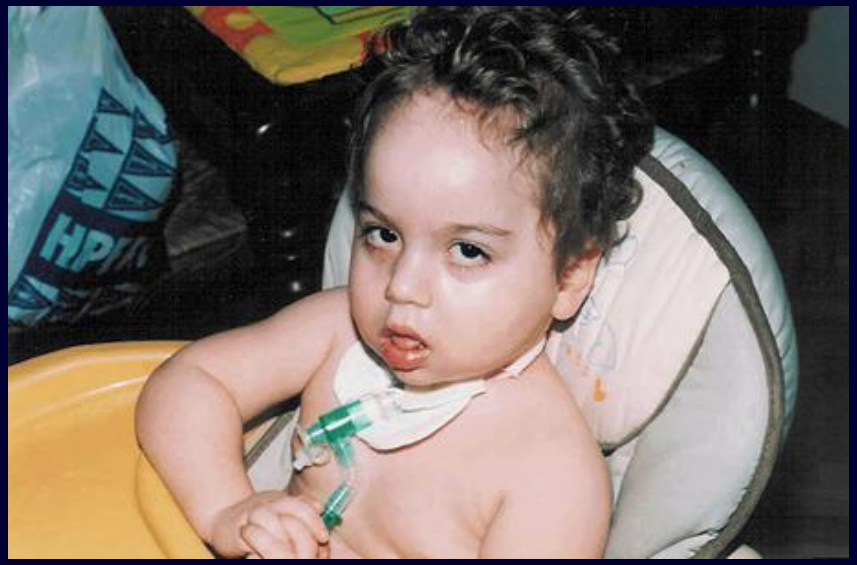
< Benthe (medication after two weeks)



^ Lucas (medication after nine months)

Earlier & current projects

- NL EU Presidency Congress (2004): Priority medicines for children

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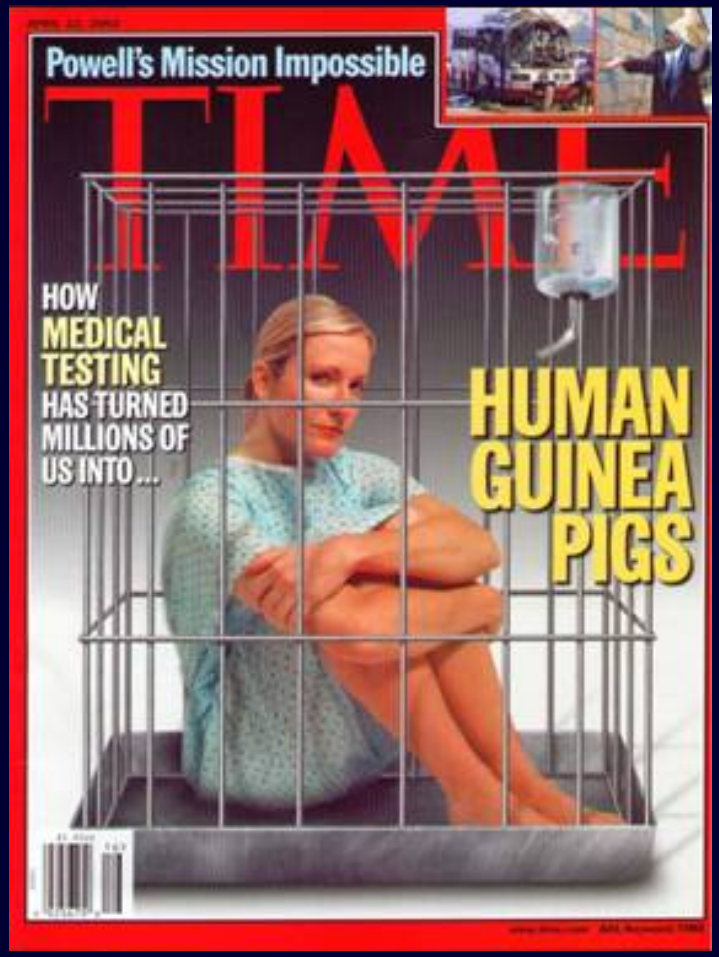
Public perception



VSOP Added Value

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- Feedback from personal experiences
- Network
- Ethical considerations
- Public perception & awareness
- Information & education
- Training: bidirectional
- Lobbying

Possible methods

- Participation in consortium meetings
- Organisation of parrallel meetings
- Consultations: internet, e-mail, interviews, focus groups
- One-sided communication: newsletters, websites (international patient academy)
- Communication & implementation of best practices

- Matching tool?
- Code of ethics?
- Sociale media?

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Patient Partnership

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Definition:

Active, structural involvement of patient representatives and organisations with the other stakeholders in the clinical trial field.

Added value of pp

Before the CT

- Identification of indications, therapy features, patient population
- Patient perspective on ethical and risk/benefit dilemmas
- Defining patient-oriented outcome measures

During

- Managing of expectations: *hope or hype*
- Patient inclusion and compliance ↗
- Data quality ↗
- Patient and public confidence in clinical research ↗

After:

- Quality of life, quality of healtcare ↗
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- (Cost-)effectiveness ↗

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Hurdles towards pp

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- Unawareness, scepticism
- No formal need or ethical / regulatory framework
- Science and industry:
    - Unfamiliar with, lacking information from, patient organisations
- Patient organisations:
    - Unfamiliar with, lacking information from, other stakeholders
    - Varying degrees of professionalism
    - How to take on a new role ?

Survey: patients perspective

How to facilitate partnership between patient organisations and the other stakeholders in clinical trials?

*"I think that researchers and pharmaceutical companies should be **obliged** to involve patient organisations.*

*I would be in favour of European **legislation** on the involvement of patient organisations in scientific search, for the pharmaceutical industry and scientific research.*

*Furthermore the involvement should also be made **financially** possible by both parties as we as patient organisations haven't got the funds for this action".*

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"Ideally the pharmaceutical industry would ask us as patients what would you like us to research and develop?"

"Patient groups could look at the protocol, patient info sheets, take care of the communication of the trial to the participants and help to recruit patients. It's about marketing the value of the trial to individual patients".

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A role for patient organisations in ethical and regulatory decision making?

"Patient organisations should definitely be heard in ethical committees as well as regulatory committees. Remember: the people who make the laws aren't doctors either".

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Patient organisations: A role in balancing real life and the need for scientific evidence of a clinical trial?

"Yes, Patient organisations can build the bridge between what is needed in structure and designs to be able to perform research and how the aspects of the daily lives can be incorporated into the trial"

"Yes, in this case your profession is being a patient. This is important to all researchers and doctors. They do not know the real 24 hour life of the patient. It's something else".

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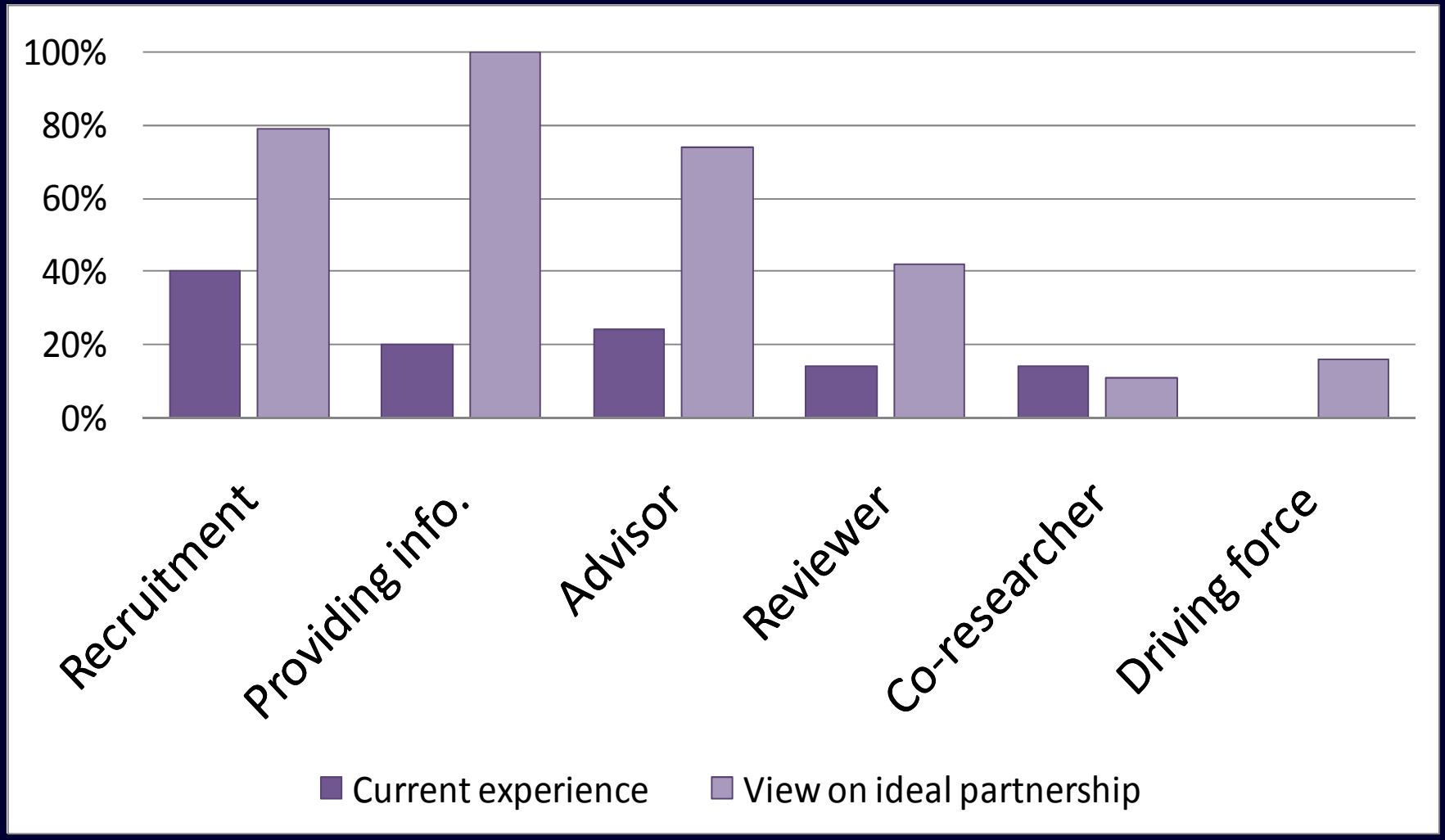
- *“the enrichment that patient involvement can provide comes from the patient’s own experience on being a patient. The patient representative should receive “training” in the committee’s habits , language etc”*

Industry perspective



What information need stakeholders from p.o.?

- Representativeness
- Competences:
- *"Patient organisations have competences that have equal value to those of the other stakeholders, only different ones that need to be treated in a different way".*
- Reasons for wanting to work with industry on ct's
- How are they organised?
- Credibility

What inform need p.o. from their partners?

- Credibility of partners: motivation, track record, transparency
- Attitude towards partnering with p.o.: listening, sharing of info
- The legal framework they abide by and how they deal with patient's rights.
- Clinical trial:
  - Reason for doing the CT
  - Methodology
  - Individual risks/benefit
  - How to get access?

Roles for p.o.

- Involved in all stages of ct from pre-clinical stage including agenda setting
- Informing and educating patient members and public about ct's
- Involved in regulatory and ethical decision making
- Supporting patient recruitment
- Support patients that take part in ct

Other findings

- Much agreement on the need for pp in clinical research
- No one-fits-all model: different needs and capacities within patient organisations
- Minor differences within Europe regarding needs, major differences regarding possibilities
- How to find each other and how to match infrastructures and cultures?
- Industry: occasional fear for delay and additional bureaucracy

Ethical principles of partnership

1. Comittee chared by Prof. Inez de Beafort

2. Mutual respect
3. Trust
4. Integrity and credibility
5. Reliability
6. Accountability
7. Aknowledgement
8. Transparancy
9. Sustainaiblity

Mutual respect

- … know, understand and respect each others envirinment and constraints

- Commen goal from different persepctives: provide benefits to patients
- Written agreement: advisable

Integrity and credibility

- Any activity should benefit the patients
- …. and operate within the regulations of the p.o.
- P.o. independence should not be compromised
- One should not seek to gain competitive or confidential information
- No endorsement of a specific product /service
- Balanced information on medicines
- Financial/in kind support must follow existing rules

Reliability

- Periodic evaluation of partnership
- Criteria for readjustement & termination

Accountability

- Agreement on external communication
- Manageable confidentiality agreements

Aknowledgement

- Prior agreement on acknowledgement issues like ownership and intellectual property
- The trial itself should be separated from the trial outcomes
- Terms of usage of name, brand, logo must be discussed

Transparancy

- Clarity about role, responsibilities, constraints
- Disclosure of other relevant collaborations
- Account for appropriate use of resources
- Acknowledgement in reporting of outcomes

Sustainability

- Minimize resource investment (administration) of p.o.
- Nu duplication of efforts
- Trial results must always, and timely, be communicated, also to the patient community

Missing/incomplete issues

- Conflict of interest: separate section
  - Example: patient = advisor and participant
- Responsibility of p.o.
- Social accountability, indemnity
- Vulnerable populations
- Make it more general (include science)
- Transform / add Memorandum of understanding
- (Legal) status of the document

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- Patient partnership should be present in all clinical research and in every single clinical trial, and from the earliest possible moment. Some countries are further along this road than others, and the sharing of best practice can help to encourage others.

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- In the current situation partnership agreements and memoranda of understanding are preferred to attempts to create legal frameworks enforcing patient involvement.

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- Resources should be devoted to ensuring that the findings of PatientPartner are taken up by other organisations and projects, including EU research programmes and Europe-wide networks, and built on.
  - **IMI project proposal: European Patient Academy On Therapeutic Innovation (EUPATI): pan-European information sharing.**
  - **EGAN-EFGCP WP: development of a training syllables**

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- Further work is needed to develop the idea of a “matchmaking” database to enable the right partners to meet each other. It should start with holding simple information, and should use linking to take advantage of information held in other databases.

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Personal take-home messages

- Patient partnership in clinical research is not (only) an emancipatory issue (“Nothing about us without us”) but a medical, scientific, ethical, and even economic necessity!
- Stimulate and facilitate both virtual and real contacts between clinical research and patient organisations
- Basic training of patient representatives and basic, uniform information to patients (and citizens) is needed.
- More efforts are needed to address the media / public on the nature of clinical research and the position of patients in it
- Involve patient representatives also at a governance level: in the setting of research priorities and ethical review.
- Patient organisations, as well as the other partners, need training and matching tools to make the partnership work .