EUPATI
European Patients' Academy on Therapeutic Innovation

EUPATI's workshop on patient involvement in industry R&D

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Recopilación de diapositivas webinario EUPATI 5-11-2014

IMPROVING INVOLVEMENT OF PATIENTS IN CLINICAL RESEARCH ACTIVITIES

Welcome – please wait, webinar starting soon

The project is receiving support from the Innovative Medicines Initiative Joint Undertaking under grant agreement n° 115334, resources of which are composed of financial contribution from the European Union's Seventh Framework Programme (FP7/2007-2013) and EFPIA companies.

European Patients' Academy
on Therapeutic Innovation

IMPROVING INVOLVEMENT OF PATIENTS IN CLINICAL RESEARCH ACTIVITIES

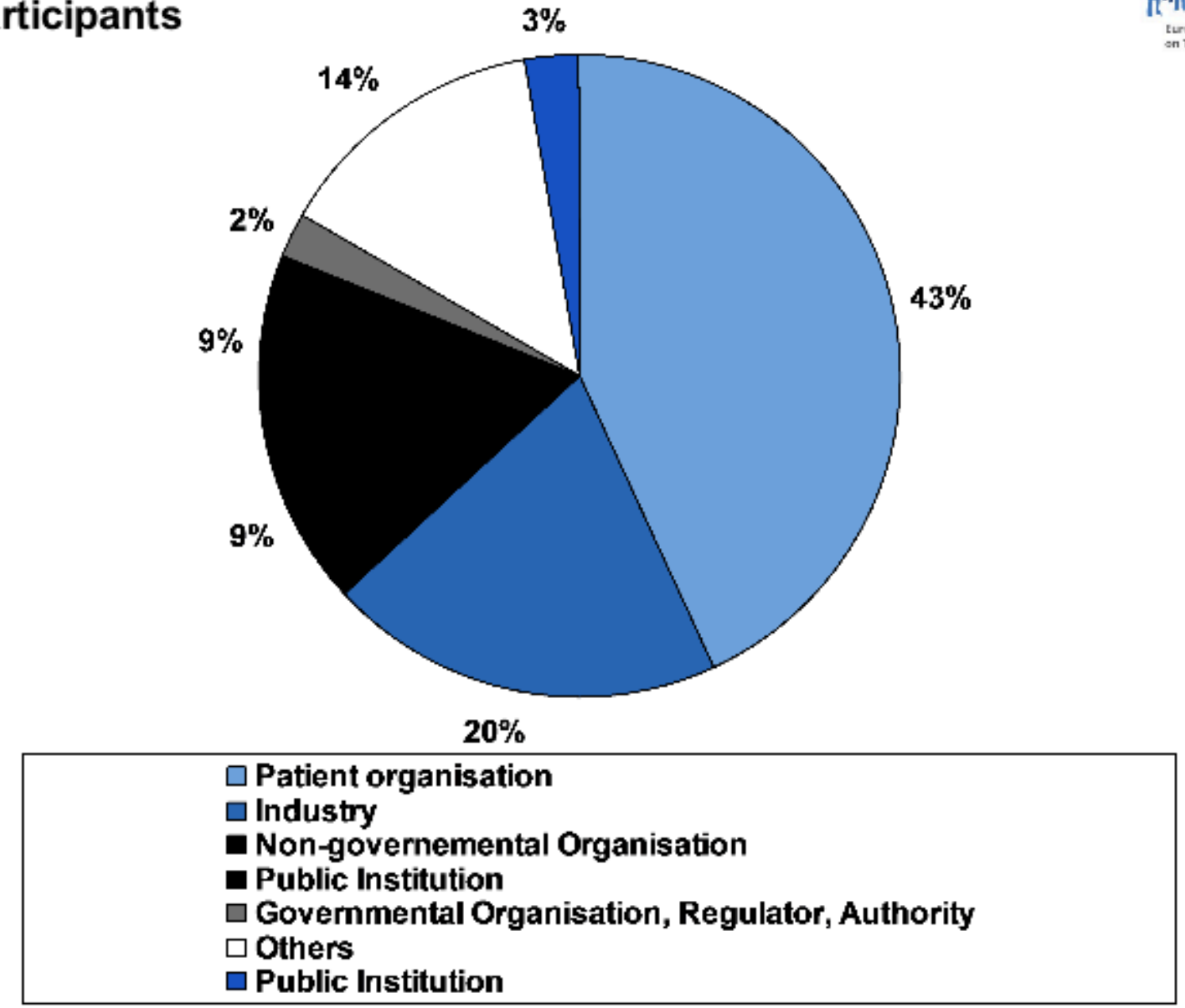
Welcome – please wait, webinar starting soon

Background to this webinar

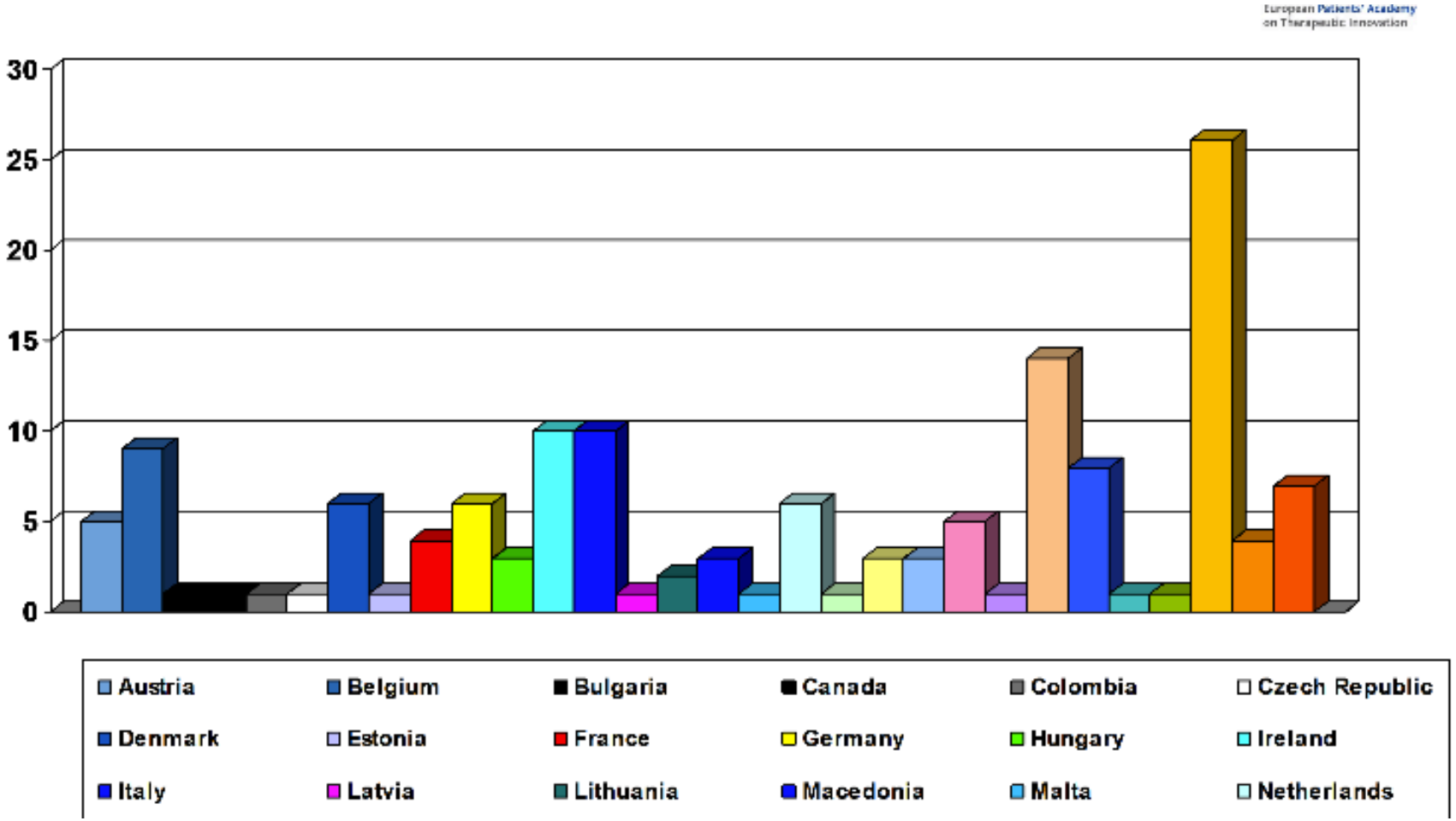
- Focus on improving the involvement of patients in clinical research activities.
- Background to the workshop hosted by EUPATI with consortium partners representing patient groups and industry.
- Panelists outline the topic from their perspectives.
- Your input will help us obtain a broader opinion and form a stronger, more informed voice.

Participants Profiles

145 participants



Países participantes en el webinar



EUPATI Workshop - Berlin

- Taking concrete actions that help support patient involvement in research and development (R&D).
- Important because patients want to be involved; will be more educated in the process of medicine development through EUPATI's deliverables
- Success = all stakeholders join together to implement true partnerships to address common goals
- Every attendee attended three breakout sessions designed to explore:
  - the benefits of patient and advocate involvement
  - the current barriers that exist
  - the relevant compliance codes and frameworks that need to be updated to enable partnerships



Overview

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Aim

- To understand how Patient Public Involvement (PPI) can enhance relevance of clinical research and deliver patient benefits

Objectives

- Introduction to Patient Public Involvement (PPI)
- PPI impact on clinical trials - case studies
  - Study literature & informed consent
  - Study logistics
  - Recruitment & Retention
- Tips for delivering quality and meaningful PPI

Benefits of PPI

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User involvement in clinical research is valuable and ensures:

- different perspectives heard
- research priorities identified by clinicians are also important and relevant to pat*ients*
- Inclusion of outcomes important to patients
- improved research design
- improved trial logistics
- access to patients - via peer networks
- access 'hard-to-reach' patient groups
- effective dissemination

Improved research that addresses:
patient needs, achieves recruitment & retention
and delivers to target

PPI in Designing a Trial

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Questions asked in Designing a Trial

WHY

WHAT

HOW

4th Question – what do patients think?

- Are we asking the right question to improve the health and quality of life for patients?

Case Study 1: Getting the right research question(s)

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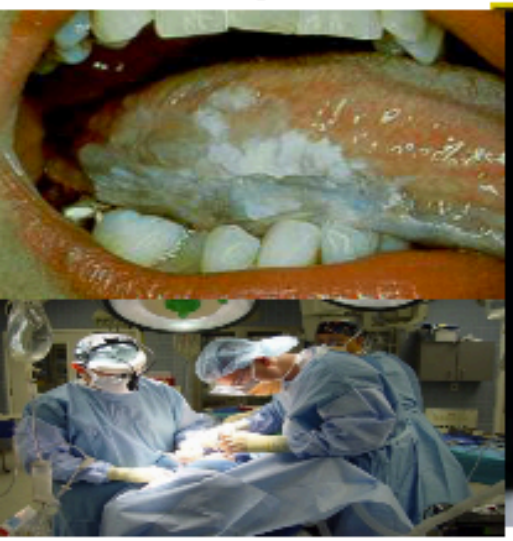
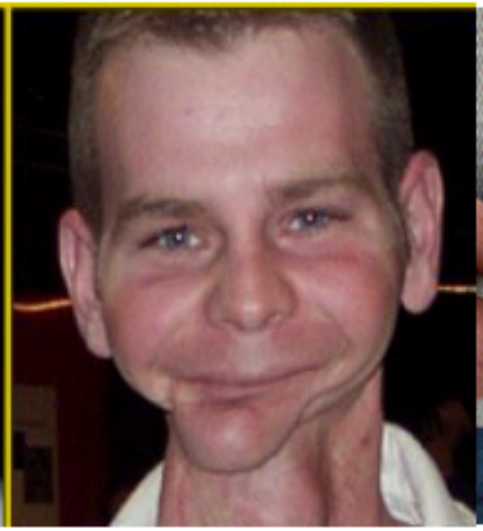
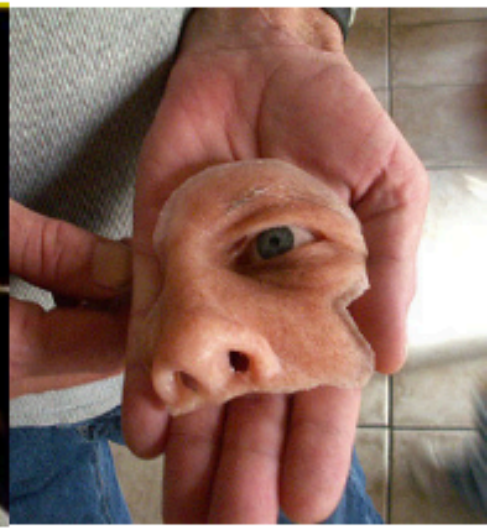
- Example from Oral Cancer



Oncologist & surgeon – focus 5-year survival at any cost

Patient – focus quality of life issues – function & disfigurement

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The importance of PPI in the design of Trial Operations and Logistics

Improving participation and retention by minimising and over burdensome trial design and listening to what patients need

Lessons learnt from the ProtecT Trial

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ProtecT Prostate testing for cancer treatment

- Recruiters found it difficult to explain the uncertainty about treatment and did not present options equally
- Non-treatment arm described as *"watchful waiting"*
- Patients interpreted as if clinicians would *"watch while I die"*
- PPI changed descriptor to *"active monitoring"*

- Recruitment rates increased from 40% to 70%

Case Study 3: PPI – Impact on Trial logistics

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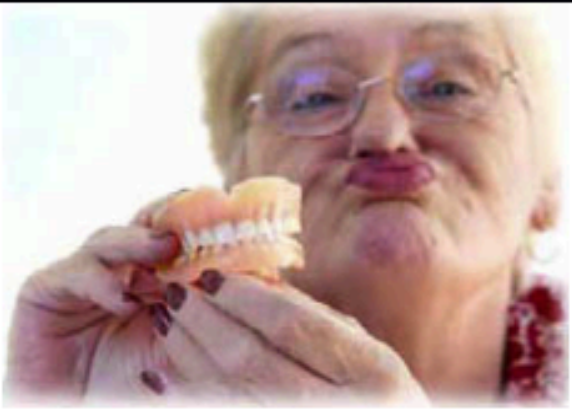
IMPROVDENT

- An RCT to improve the fit of dentures by testing two dental impression materials



Trial Operational input

- Appointments available largely between 10am - 3pm
- Accommodates travel to appointments on Senior Citizen Bus Pass

Improved Trial Operations

- Trial is "user friendly"
- Participants less inconvenienced
- Few cancelled appointments
- Recruitment to schedule

PPI and Developing Good Working Relationships

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- Information and communication
- The 5 R's
  - Role
  - Remit
  - Representation
  - Responsibilities
  - Relationships
- Facilitating meaningful participation
  - Understanding and fostering interest
  - Access & needs (physical, circumstantial)

PPI and Developing Good Working Relationships

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❑ Information and communication

- Be clear about the level of involvement
- Thorough preparation to help understand the research
  - ❖ Proper briefing
  - ❖ Person specification and role description
  - ❖ Clarity about the degree of involvement
  - ❖ Motivation
  - ❖ Co-ownership of the particular aspect of involvement
  - ❖ Whole picture rather than an individual's experience
- Consider special needs but avoid 'paternalism'
- Ensure Regular feedback to contributors at all stages

When PPI Works Well

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Trust & value

- Clarity on the project and roles, time commitments
- The PPI reps felt able to ask questions
- The views and input of service user researchers were valued by the academic researchers

Preparation & knowledge

- Guidance and support available
- Maximised engagement of PPI when chair took time to explain research / concepts to PPI rep – often via debriefing session

Openness

- The academic researchers were honest about the shortcomings of the project and prepared to discuss issues as they arose throughout the course of the research process

When it doesn't goes so well

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- **PPI reps were not part of the initial application for funding**
  - Did not have ownership of the process from the beginning
- **Lack of induction and/or poor early team building led to misunderstandings**
- **Short deadlines**
- **Effective communication channels were not established**
- **Inadequate preparation for working with PPI reps**
- **Insufficient support for users available during the process**

The Future - PPI Integral to Clinical Trial Design & Recruitment Strategy

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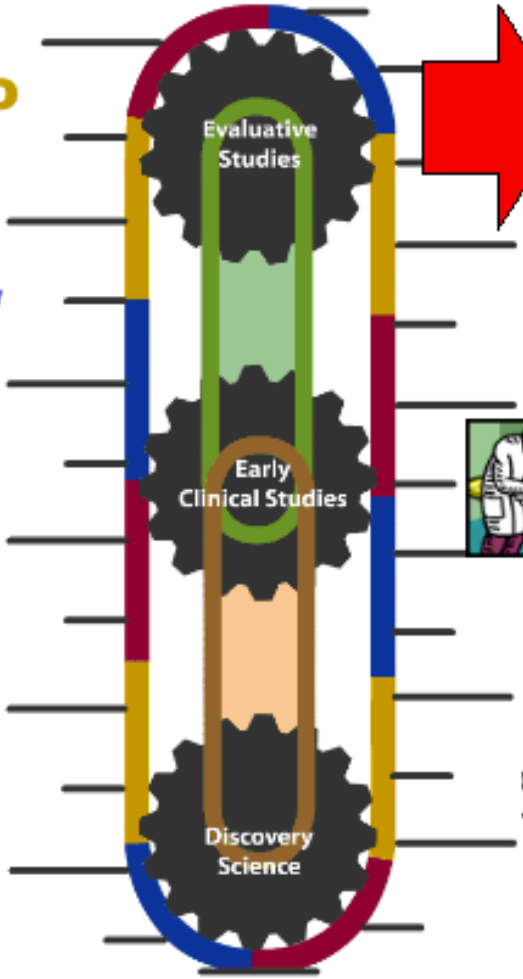
- **Trial designed to take account of patients needs**
- **Trial operations / logistics made patient friendly**
- **Trial literature simplified**
  - Ensuring informed consent



Improved Recruitment & Retention

Patient Benefit

Improved Health

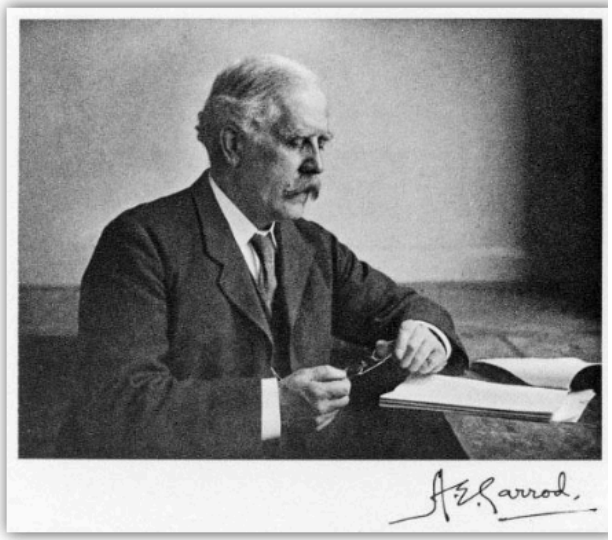
Improved Delivery of Trials & Evidence-based Medicine

Improving involvement of patients in clinical research

Lessons from Black Bone Disease

Dr Nicolas Sireau
Chairman and CEO, AKU Society
Chairman and Co-founder, Findacure

1902: Sir Archibald Garrod

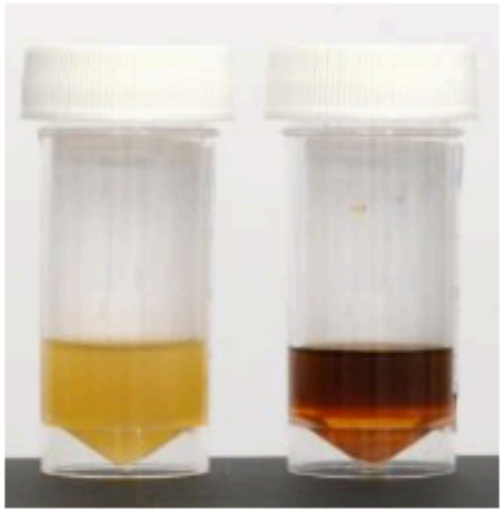


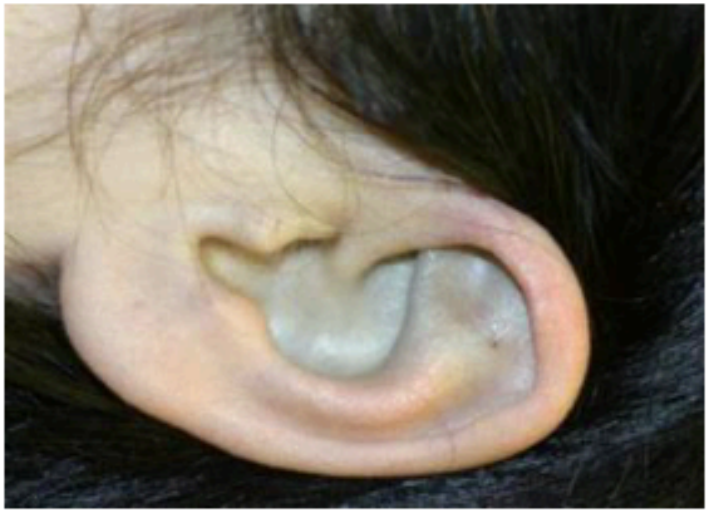
Harwa

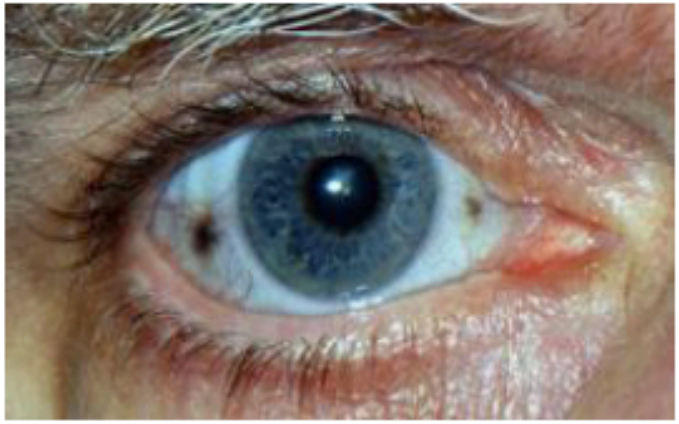
Oldest AKU Patient
1500BC

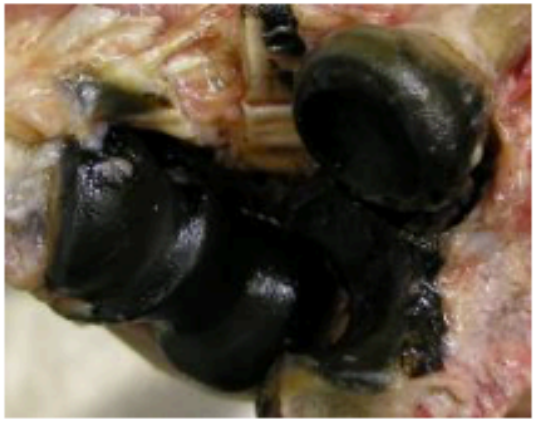
Stenn et al 1977

The AKU tetrad

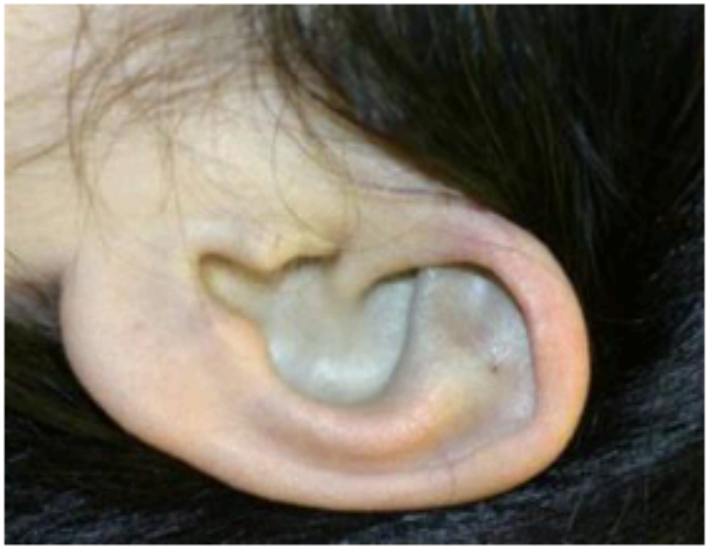


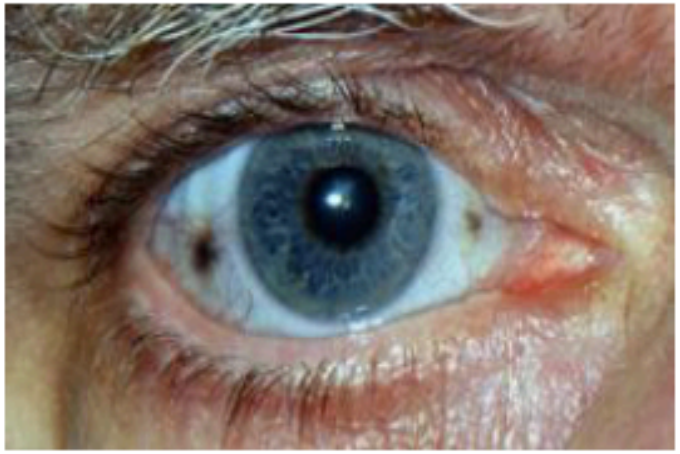


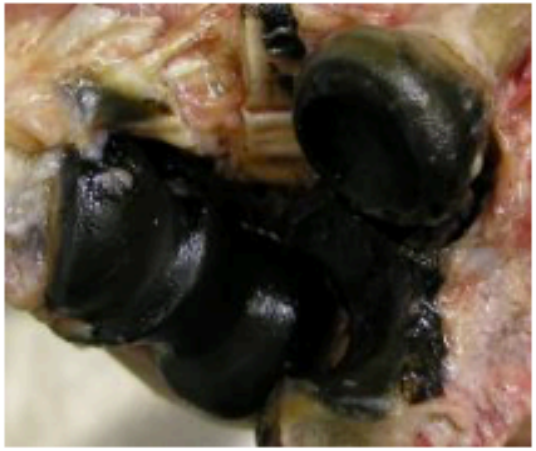




The AKU tetrad







Nitisinone reduces homogentisic acid by

95%

Three Studies

Trial Name	Description	Sites
SONIA 1: Suitability of Nitisinone in Alkaptonuria 1	3-month phase II study	UK/Slovakia
SONIA 2: Suitability of Nitisinone in Alkaptonuria 2	4-year phase III	UK/Slovakia/France
SOFIA: Subclinical Ochronosis Features in Alkaptonuria	Cross-sectional study	UK

Three Clinical Trial Sites

1) Liverpool, UK
   Royal Liverpool
   University Hospital
   PI: Prof L Ranganath

2) Paris, France
   Hôpital Necker
   PI: Prof Pascale de Lonlay

3) Piešťany, Slovakia
   National Institute of
   Rheumatic Disease
   PI: Prof Jozef Rovenský

The DevelopAKUre partners

The Royal Liverpool and Broadgreen University Hospitals NHS Trust

UNIVERSITY OF LIVERPOOL

NÁRODNÝ ÚSTAV REUMATICKÝCH CHORÔB

Hôpital Necker Enfants Malades

PSR theorphanEXPERTS

UDOS Drug development consultancy

INSTITUT NECKER

UNIVERSITY OF SIENA

sobi SWEDISH ORPHAN BIOVITRUM

nordicbioscience

AKU Alkaptonuria Society

ALCAP Association pour la Lutte Contre l'Alcaptonurie

"These trials have given us great hope. This treatment could completely change our lives. We're that one step closer to a cure."

- Belgium AKU patient

Challenges and solutions for patient groups working with industry and academia

EURORDIS
Rare Diseases Europe

The Voice of Rare Disease Patients in Europe

EU Clinical Trials Register

RARE-Bestpractices

International Rare Diseases Research Consortium

EURORDIS Summer School

Who we are

EURORDIS is a non-governmental patient-driven alliance of patient organisations representing 634 rare disease patient organisations in 58 countries covering over 4000 diseases.

- Our members
- Our mission statement

What are you looking for:

Patients and services

Elysha didn't sit around being sick at Barretstown Therapeutic Recreation Programme. She had nonstop fun!

Featured Event

Don't miss the chance to download the speaker presentations, available online and on the mobile app

2014 Berlin

Members' Corner

Findacure Training workshop: How to develop clinical trials as a small patient group. 24 July, London, UK

findacure

EURORDIS TV

Watch EURORDIS TV's video of the week!

Multiple Endocrine Neoplasia explained to children: Daniel and his mum have MEN1


countries
finally
trials
treatment
also
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Potential advantages of patients involvement in R&D

COLLABORATION DURING PROGRAM DEVELOPMENT

- Relevant especially (but not only) for new programs and indications
- Better understanding or real needs
- Identification of opportunities and hurdles at early stages

Have more (relevant) real life insights and better outcomes for patients

janssen

Event Manager Event Manager (Host)
Sabine Brookman-May
Daphnee Pushparajah
Ilaria Piuzzi
Ingrid Heyne
Kay Warner
Nick Sireau
sue Pavitt
Attendees: 75 (1 displayed)
View all attendee
Ferrero Paz (me)

Chat
from Event Manager Event Manager to All Attend
Please feel free to type in any questions or comm
discussion

Send to:
Select a participant in the Send to menu
and send...

Potential advantages of patients involvement in R&D

COLLABORATION DURING PROTOCOL DESIGN

- Identification of major hurdles for trial conduction and patient recruitment from patients perspective
- Identification of side effects that patients are willing to accept
- Definition of relevant Patient Reported Outcomes (PRO) and patient relevant endpoints (Do physicians actually know what is relevant for the patients?)
- Reduce complexity of trials by patient engagement

Improve the protocol to conduct the trials in the best possible way

Potential advantages of patients involvement in R&D

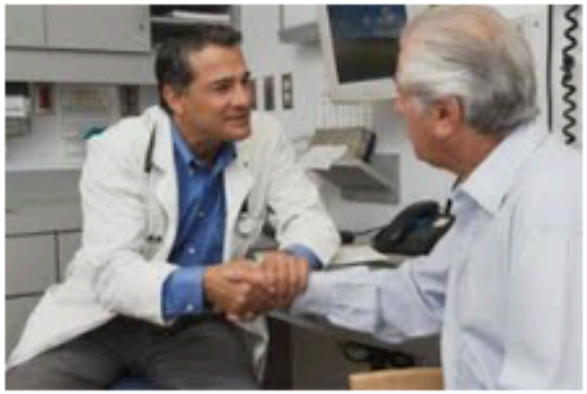
Collaboration before/during trial start-up and in ongoing trials

- Collaborate with advocacy groups to identify ways to spread information of trials
- Raise awareness of trials amongst patients
- Overcome eventual unexpected hurdles together with patients

Faster Study Enrolment

Enhanced access to trials for patients

Research & Development in Oncology



Specific situation in oncology as compared to different therapeutic areas

- very high unmet medical need

- to be considered:
  - (Study) patients are predominantly in a palliative situation
  - In most trials cure of disease is not the goal
  - Long term treatment in a trial may be necessary
  - Patients have often already reduced performance status → study medication may further impair patient status
  - If patients are asymptomatic → study medication may impact the quality of life
  - Physicians sometimes estimate patients´needs differently

- Patients need to balance pro and cons in depth before entering in a trial
- Patients perspective is even more required than in other therapeutic areas
- Furthermore to be considered: pediatric trials in oncology

Example of patient research

Janssen Phase III trial for prostate cancer with a new compound/indication

- Unexpected hurdles for patient recruitment - patients are not found at the study sites
- Need to inform patients about this trial

Collaboration with EUPATI, Europa Uomo, BPS, African-American Prostate Cancer Advocay Groups and local advocacy groups in the countries

- **Patient advocates confirmed high unmet medical need in this indication and the need for additional information**
- **Collaboration has just started - outcome cannot finally be estimated until now, but from a first perspective we are very confident**

Challenges, hurdles and remaining questions

- No previous experience within oncology R&D
- No clear rules/no awareness of rules for outreach to patient advocacy groups in the countries
- Needs to have a code of practice to involve patients
- Needs to be a trade-off between the wishes of a patients and what is realistically feasible
- Needs to measure the experience to demonstrate the overall benefit for all the parties involved

Many questions need to be answered:

- Are industries allowed to reach out to patient advocacy groups proactively?
- Are there any local/regional differences?
- How can we implement collaboration in the best way?
- How can we ensure a comprehensive compliance in the process?

EUPATI's workshop - outcomes

EUPATI Workshop - Berlin

- **WHY**
- Clearly make the case for patient involvement in medicines development
- Scope key actions to document and communicate the impact and benefits
- Create a platform for sharing case studies of good practice and developing training for industry and regulators on the value of patient engagement

EUPATI Workshop - Berlin

- **HOW**

- Develop a framework for patient involvement

- Outline the steps needed to involve patients and advocates

EUPATI Workshop - Berlin

- **DO**

- Create key performance indicators for patient involvement: Develop measures that cover quality, quantity and speed

- Create SOPs and guidance for good practice

- Develop EUPATI matchmaking as broker for patients and research

Event Manager Event Manager (Host)
Daphnee Pushparajah
Ilaria Piuzzi
Ingrid Heyne
Kay Warner
Nick Sireau
Sabine Brookman-May
sue Pavitt
Attendees: 76 (1 displayed)

Panel Discussion with Q & A:

Please submit your questions using the Q &A function

Q : (Rainald von Gizycki) - 18:18

How did you actually mesure the slow progression of your desease? Did you use objective and/or subjective outcome indicators?

A : (Nick Sireau) - 18:22

We developed a Severity Score Index with objective outcome indicators (MRI, Xray, etc).

Q : (Ferrero Paz) - 18:15

As patient organization we have a very good relationship with academia but our perception is that we do not know how to collaborate related to clinical trials.