Breakout session #3 on Patient Involvement

Moderators: Claas Röhl & Solange Corriol-Rohou

EFPIA European Federation of Pharmaceutical Industries and Associations

Norwegian Medicines Agency

Day 1
5 October 2021

Breakout session 3
Patient Involvement

Multi-stakeholder workshop
5 - 6 October 2021

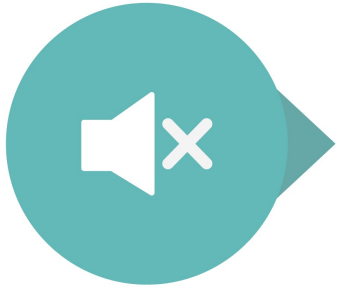
Chairs:

Claas Röhl (NF Patients United, AT)
Solange Corriol-Rohou (AZ, EFPIA)

Patient involvement in clinical trials is attracting more and more interest, and experience is growing. This interactive breakout session will be not only the opportunity to share the experience so far but also to identify recommendations to optimise patient's involvement in the design and conduct of complex trials. A few flash presentations will open up the discussion by representatives of key stakeholders. We cannot expect to solve all the issues through this session, but the goal is to identify recommendations and next steps including synergies with the Education & Training breakout session.

House-keeping rules

(For active participants in the Zoom call)



- Mute your sound and video when not speaking

- Flag your intention to take the floor by raising your hand or by inputting your name into the Zoom-chat

- Introduce yourself (name, company, role) when taking the floor

Join our Q&A and online polls at

Slido.com
#867 213

Direct link to our Q&A:
https://app.sli.do/event/htnou5aw

You can scan the QR-code with your mobile device for direct access to the Q&A

Our breakout session objective

- To evaluate the needs and expectations for Patient Involvement
- To identify a clear, collaborative process that will enable Patient Involvement in CCTs in the EU
- We cannot expect to solve all the issues through this session, but the goal is to identify recommendations and next steps including synergies with the Education & Training breakout session to be held tomorrow.

The breakout session

- Patient involvement in clinical trials is attracting more and more interest, and experience is growing.

- This interactive breakout session will be not only the opportunity to share the experience so far but also to identify recommendations to optimise patient's involvement in the design and conduct of complex trials.

- A few flash presentations will open the discussion:
  - **Ann Marie Janson Lang** (Swedish Medical Products Agency, and CFTG co-chair) will set the scene from a regulatory standpoint, with a focus on the application of the clinical trial regulation in January 2022;
  - **Begonya Nafria Escalera** (Patient Engagement in Research Coordinator) will explain how important complex trials are for paediatric development;
  - Colleagues involved in IMI and at the EU commission – **Nathalie Seigneuret**; **Alexandru Costescu** - will share what has been done and could be expected in the future through Public Private Partnership projects;
  - **Dr Birgit Geoerger**, onco-paediatrician, will share her experience conducting trials.

Setting the scene with

- **Ann Marie Janson Lang** - Swedish Product Medical Agency - MPA, and CFTG co-chair
- **Begonya Nafria Escalera** - Patient Engagement in Research Coordinator)
- **Nathalie Seigneuret** – the Innovative Medicines Initiative
- **Alexandru Costescu** – EU Commission
- **Birgit Geoerger** – Gustave Roussy Institute, France

September 30 2021

Patient involvement strengthened when the clinical trial application applies in EU

Ann Marie Janson Lang

Expert, MD, PhD, Assoc Prof,

Swedish Medical Products Agency

Co-Chair

Clinical Trials Facilitation and Coordination Group (CTFG)

Regulation (EU) No 536/2014

- Replaces existing legislation on clinical trials of medicinal products (national laws for authorities and ethics committees)
- Single decision per Member State (joint input from authority & ethics committee)
- Applies from Jan 31 2022
- Defines clinical trials and clinical studies
- Clarifies obligation to involve laypersons/patients/patients' organisations

New legally-defined roles in clinical trials for laypersons, patients and patients' organisations

Change from roles as 'participant in trial' and 'stakeholder receiving trial information' into legally-defined more **active** roles

Those include involvement in

- **assessment** of clinical trial **applications**

  Article 9.3 3 "…At least one layperson shall participate in the assessment…"

- **trial design** as described in the **protocol**

  Annex I, Application dossier, Protocol 17 (e) "…where patients were involved in the design of the clinical trial, a description of their involvement…"

Regulation (EU) No 536/2014

Clinical Trial - only conducted if two conditions are met:

1. rights, safety, dignity and well-being of subjects are protected and prevail over all other interests

2. trial is designed to generate reliable and robust data

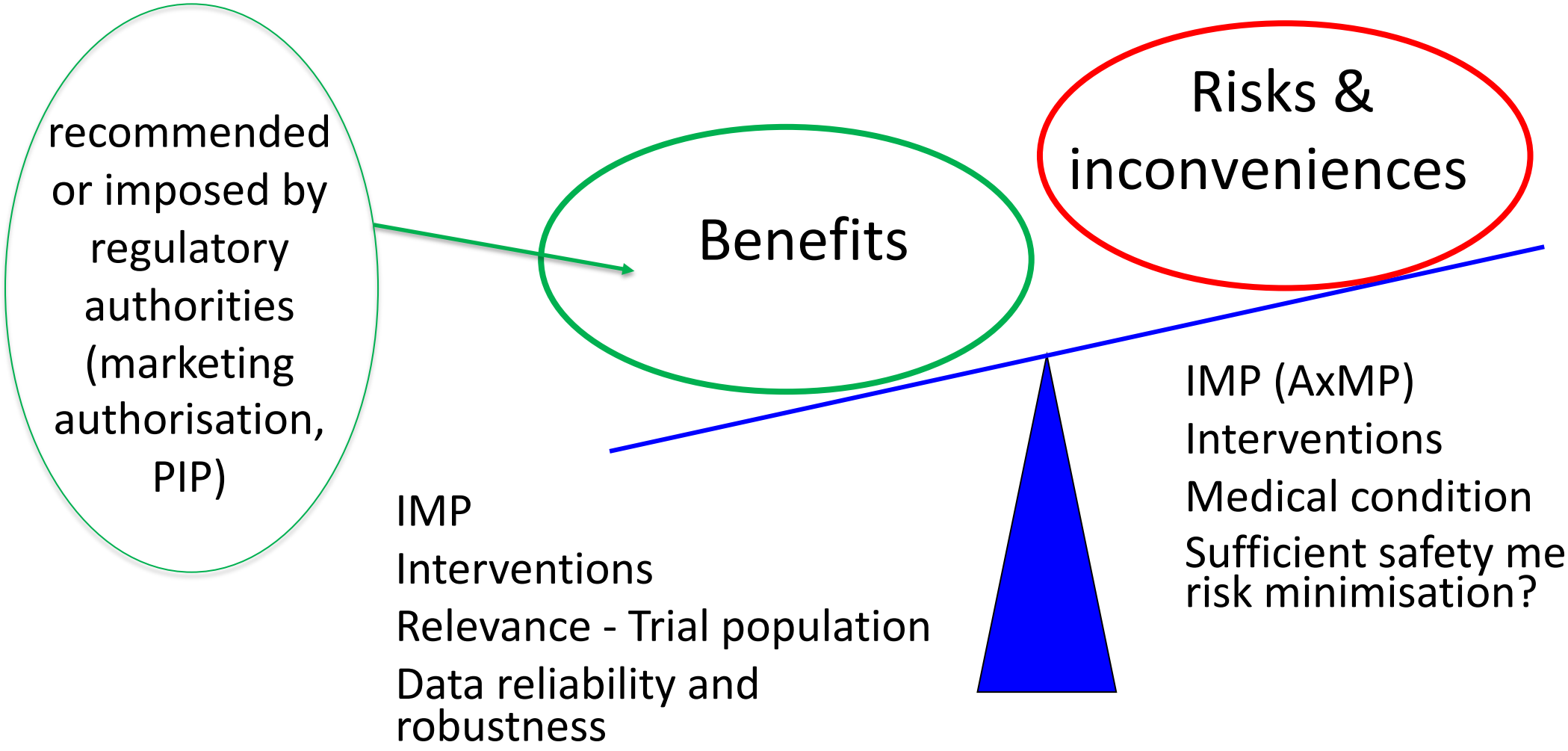
Key aspects of trial benefit/risk assessment

- **Benefit** - individual, group or public health?
- Emergency situation - scientific grounds to expect potential of direct **clinically relevant benefit** for the individual subject

  "…a measurable health-related improvement alleviating suffering and/or improving health of the subject, or in the diagnosis of its condition…"

- **Risk** - only to subject (not to environment, e.g. by gene modifying organism)

Assessment of trial application Regulation Article 6.1(b)



Thanks for your attention!

Questions welcome

annmarie.jansonlang@mpa.se

SJD Sant Joan de Déu
Barcelona · Hospital

Patient involvement in CCT
(From the patient perspective)

Begonya Nafria- Sant Joan de Déu Children's Hospital

SJD Sant Joan de Déu
Barcelona · Hospital

New paradigm shift:

from a drug-centric approach towards a more patient-centric approach

Right to be involved in science

Children and young people have the right to **freely express their views** (CRC art. 12), the right to the highest attainable standard of health and to facilities for the treatment of illness and rehabilitation of health (including **research**).

Convention of the Rights of the Child-
1989 – United Nations

Legal and ethical frameworks:

- ✓ Clinical Trials Regulation (536/2014)
- ✓ Paediatric Regulation (1901/2006)
- ✓ Declaration of Helsinki (2008)
- ✓ General Data Protection Regulation (2018)
- ✓ Charter on Fundamental Rights of the European Union (2012)

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Role of patients involvement in complex clinical trials

"Nothing about me without me.

Defining the patient voice

"***The patient voice adds a different perspective to everything that goes on in Health care and can point out real gaps in the system***".

K. Niehaus, Patient Advocate-
MSKCC - NYC- US

Asessment of clinical trial **applications**

Article 9.3 "…At least one layperson shall participate in the assessment…"

Trial design as described in the **protocol**

Annex I, Application dossier, Protocol 17 (e) "…where patients were involved in the design of the clinical trial, a description of their involvement…"

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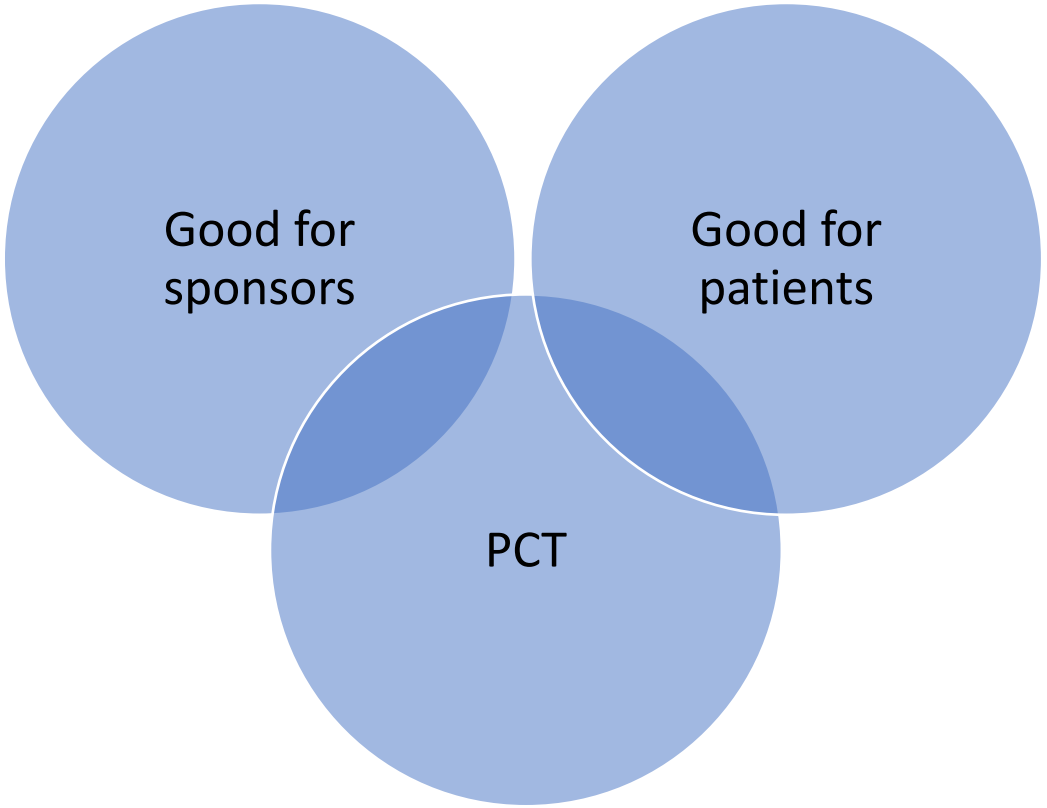
AWARENESS	DESIGN	RECRUIT	CONSENT	RETAIN	FOLLOW UP
Advertise to people not participants Building HCP advocates Educational awareness materials	Consumer tech approach Patients as a parterns Inclusive protocols	Use digital and print channels to connect with Patients Educational materials	Consent as a Process Continuous Communication eConsent	End-to-end Experience Technology to support communication Consider the needs of the patient's lifestyle	Inform along the process Share results in a friendly way

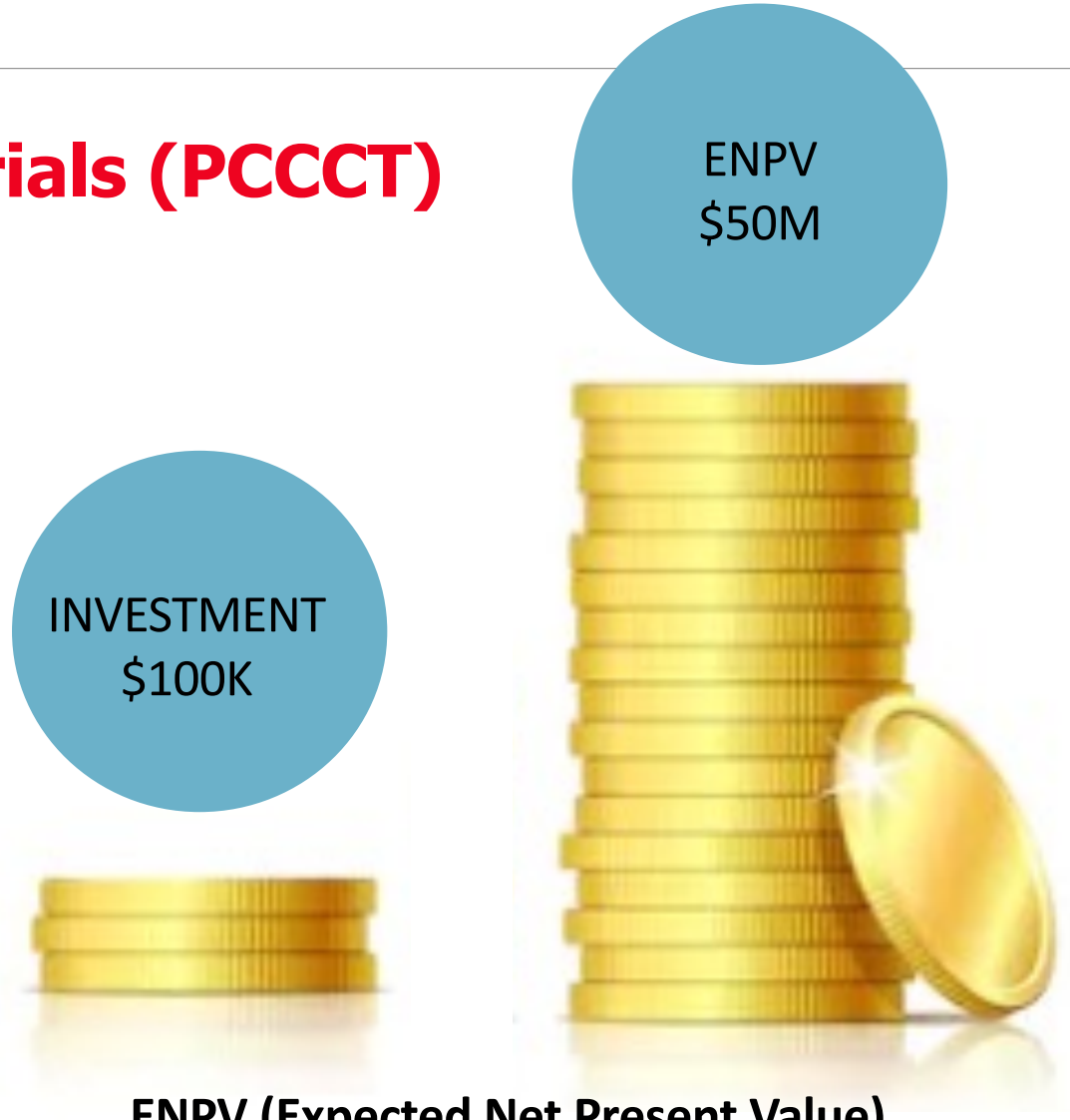
UNMET MEDICAL NEEDS

REVIEW OF PROTOCOLS
INVOLVEMENT IN ETHICS COMMITTEES

TRIAL STEERING COMMITTEE
DATA & SAFETY MONITORING COMMITTEE

Patient Centric Complex Clinital Trials (PCCCT)





ENPV (Expected Net Present Value)

Source: Levitan B, Getz K, Eisenstein EL et al. Assessing the financial value of patient engagement: a quantitative approach from CTTI's patient groups and clinical trials project. Ther Innov Regul Sci 2018

Beyond the ROI there is the ROE…

We need to work towards this common direction

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Key messages about patient involvement in CCT

Why involve patients?

Is the best approach to design **patient-centered trials.**

They can benefit the research itself but especially the patient experience for the ones that will participate in the CCT.

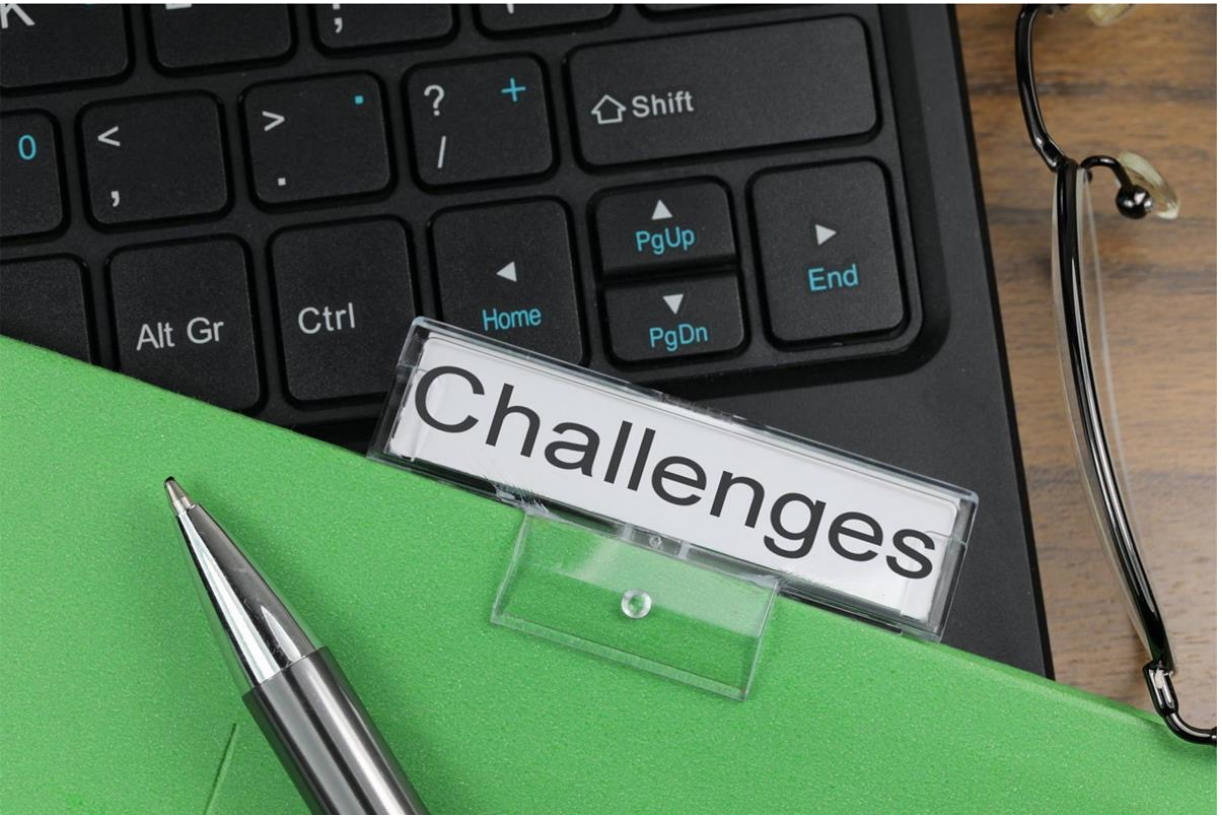
Patients deserve to be involved along with the clinical trial. As soon the better.

How involve patients?

Involve patients requires **expertise**, **planning** and **assessment**.

Diversity needs to be included in any patient involvement activity.

Patients organizations and devoted networks of **YPAGs** can help sponsors in the amazing journey of patients involvement in clinical trials.



- ✓ Training for patients and patients advocates
- ✓ Value of the patients involvement in the clinical trial application
- ✓ Ensure early and meaningful involvement of the patients

Thank you so much!

begonya.nafria@sjd.es

Accelerating Adoption of Complex Clinical Trials in Europe and Beyond

Break out session

Patients Involvement

Nathalie Seigneuret, Senior Scientific Project Manager, IMI

05.10.2021. Multi-stakeholder Workshop

IMI: Ecosystem for innovative collaborations

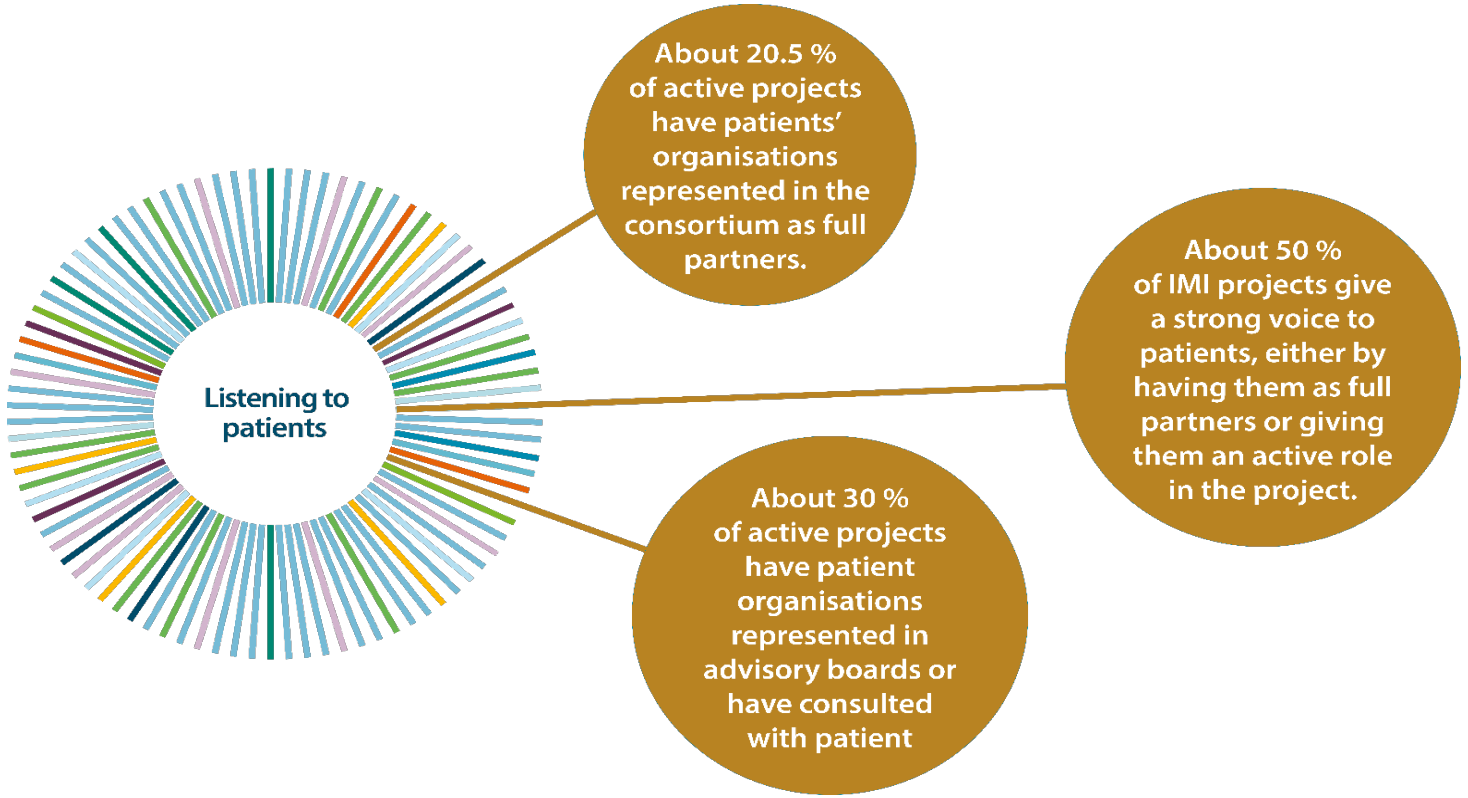
IMI is a **neutral platform** where **all involved** in drug development can engage in **open trustworthy collaboration** on **shared challenges in areas of unmet medical needs**.

€5.3 bn **174 projects** **> 5 200 participants** **> 7 000 outputs**
> 7 000 publications **38 Associated Partners**

Academia
Charities
Regulators
SMEs
HTA bodies
Pharma companies
Payers
Diagnostic companies
Healthcare practitioners
Other sectors (e.g imaging, nutrition…)
Public health bodies
Patients

innovative medicines initiative

IMI: Patient involvement in IMI projects



EUPATI
European Patients' Academy on Therapeutic Innovation

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

Patient engagement toolkit and models

imi innovative medicines initiative

IMI: Advancing clinical research

- Many IMI projects develop tools/methodologies to optimise clinical trial design with focus on patient-centricity

Trial design

EU-PEARL (integrated research platforms)

GetReal/GetReal Initiative (RWD in drug development)

EPAD (Alzheimer's prevention)

INNODIA/INNODIA HARVEST (T1 diabetes)
UNITE4TB (tuberculosis)

Digital tools

Mobilise-D (digital mobility endpoint)

Idea-Fast (digital endpoints)

Radar-CNS (wearables)

Radar-AD (wearables)

Trials@Home (decentralised clinical trials)

PharmaLedger (blockchain technology)

Data sources

EHDEN (federated big data)

ConcePTION (use medicines in pregnancy)

Gravitate Health (e-product information)

Clinical trial networks

c4c (paediatric)

Combacte-Net (AMR)

INNODIA/INNODIA HARVEST (clinical network)

EU-PEARL (hospital hubs)

Outcomes

BD4BO projects

H20 (health outcomes observatory)

SISAQOL-IMI (HRQL and PROs in cancer)

List non-exhaustive

imi innovative medicines initiative

Complex clinical trials - IMI projects

- **EPAD** - Proof of concept platform developed to run Phase II clinical trials with research participants with preclinical and prodromal Alzheimer's disease, with biomarker evidence of Alzheimer's disease pathology using a consistent set of outcomes. EPAD was unique in its ability to recruit from the EPAD readiness cohort.
  patient organisation in the consortium

- **INNODIA** - Clinical trial master protocol specifically designed for phase 2 clinical trials of people who have just been diagnosed with type 1 diabetes allowing for adaptive trials to test different drugs in parallel.
  patient organisation in the consortium + patient advisory committee

- **COMBACTE-NET** – HONEST-PREPS observational study for a subsequent platform trial (HONEST-PLATFORM: HOspital NEtwork STudy – PLAtform Trial FOR antibacterial Molecules) to set up an infrastructure to prospectively enroll patients at risks of HAP/VAP in ICU

Complex clinical trials - IMI projects

- **NECESSITY -** clinical trial to validate the newly defined clinical endpoints and the identified biomarkers: innovative “multi-arm multi-stage platform trial” able to include all the different types of patients with primary Sjögren’s Syndrome in different arms, with different types of drugs and with different methodology.
  patient organisation in the consortium + patient advisory group

- **EU-PEARL –** 4 diseases trial ready IRP networks (Major Depressive Disorder, Tuberculosis, Non-Alcoholic Steatohepatitis (NASH) and Neurofibromatosis
  patient organisation in the consortium + associate collaborators

- **UNITE4TB** – Phase 2 TB clinical trial designs, utilising simulation tools to identify optimal doses in phase 2A trials and apply a multi-arm multi-stage adaptive randomised controlled 2B/C trial design capable of rapid and simultaneous evaluation of the best candidate regimens.
  patient organisation in the consortium

innovative medicines initiative

Patients engagement in research

- Patients engagement crucial in all trials
- Involvement of patients in IMI projects as beneficiaries, Patients Advisory Group
- IMI projects examples shows that patients have a role in:

  - Defining research questions
  - Ensuring patients' perspective embedded
  - Reviewing CT protocols
  - Helping with recruitment and engagement of CT participants
  - Communicating project's goals, results including feedback to trials participants
  - Educating and empowering patients
  - Selecting compounds?

IMI now in transition…….to IHI

(the Innovative Health Initiative)

- New PPP proposed and due to replace IMI
- It will bring in new founding members from the Medtech/Dx industry and the imaging/digital/medical instrument producers (they will join the EU Pharma industry partners)
- The overall budget will be around €2.4 billion
- Strategic Research and Innovation Agenda being finalised
- Legislative process under way
- Optimistic scenario would allow a first call for proposals at the end of 2021

More information at:
EC website (legislative process and proposal) https://ec.europa.eu/info/horizon-europe/european-partnerships-horizon-europe/candidates-european-partnerships-health_en; https://ec.europa.eu/commission/presscorner/detail/en/ip_21_702
Inter-associations website (draft SRIA, case studies) https://www.euhealthppp.org/
IMI website https://www.imi.europa.eu/about-imi/innovative-health-initiative

imi innovative medicines initiative

IHI Objectives

Overall: Healthcare interventions that respond to accelerate the development of safer and more effective innovative unmet public health needs, and that can be taken up by healthcare systems

Strategic Research & Innovation Agenda *draft as of June 2021*

- Better understanding of the determinants of health and priority disease areas
- Integration of fragmented health care Research & Innovation efforts
- Development of people-centred, integrated health care solutions
- Full use of digitalisation and data exchange in health care
- Assessment of the added value of innovative and integrated health care solutions

Full text - see www.EUHealthPPP.org

Credits: COCIR, EFPIA, EuropaBio, MedTech Europe

innovative medicines initiative

Conclusion

- Collaboration key and IMI (and future PPP) as a neutral broker allow participation of all stakeholders incl. patients
- IMI and future IHI focus on patient-centricity and contributes to regulatory science Complex clinical trials discussed at the IMI-EMA-FDA Regulatory Science Summit held in 2019
- Safe harbour like IMI (and future PPP) create common understanding of problems, pilot new ideas/proof of concepts and find new solutions that would inform the evolution of the regulatory practice
- Co-creation with patients critical to maximise impact
- Many opportunities to address research gaps for the running of complex clinical trials and patients' engagement in the new cross-sectoral PPP for health under Horizon Europe: Innovative Health Initiative

EU Health Partnerships and Patient Involvement

Alexandru Costescu, MD

Policy Officer, DG R&I, Health Innovations & Ecosystems

Patient involvement breakout session

EUPATI Patients United · efpia European Federation of Pharmaceutical Industries and Associations · BARC · PPD · Norwegian Medicines Agency

HORIZON EUROPE

EURATOM

SPECIFIC PROGRAMME: EUROPEAN DEFENCE FUND

Exclusive focus on defence research & development

- Research actions
- Development actions

SPECIFIC PROGRAMME IMPLEMENTING HORIZON EUROPE & EIT*

Exclusive focus on civil applications

Pillar I
EXCELLENT SCIENCE

- European Research Council
- Marie Skłodowska-Curie
- Research Infrastructures

Pillar II - €58.9 billion
GLOBAL CHALLENGES & EUROPEAN INDUSTRIAL COMPETITIVENESS

Clusters

- Health – €8.25 billion
- Culture, Creativity & Inclusive Society
- Civil Security for Society
- Digital, Industry & Space
- Climate, Energy & Mobility
- Food, Bioeconomy, Natural Resources, Agriculture & Environment

Joint Research Centre

Pillar III
INNOVATIVE EUROPE

- European Innovation Council
- European Innovation Ecosystems
- European Institute of Innovation & Technology*

WIDENING PARTICIPATION AND STRENGTHENING THE EUROPEAN RESEARCH AREA

- Widening participation & spreading excellence
- Reforming & Enhancing the European R&I system

EURATOM

- Fusion
- Fission
- Joint Research Center (JRC)

* The European Institute of Innovation & Technology (EIT) is not part of the Specific Programme

European Commission

New approach to European Partnerships

European Partnerships bring the EC and private and/or public partners together to address Europe's most pressing challenges through concerted research and innovation initiatives. They are a key implementation tool of Horizon Europe.
New generation of objective-driven and more ambitious partnerships in support of agreed EU policy objectives:

Key Features

- **Strategic orientation**
- **Systemic approach**
- **Simple architecture and toolbox**
- **Common set of criteria for the life-cycle**

CO-PROGRAMMED
Based on Memoranda of Understanding/contractual arrangements; implemented independently by the partners and by Horizon Europe

CO-FUNDED
Based on a joint programme agreed and implemented by partners; commitment of partners for financial and in-kind contributions

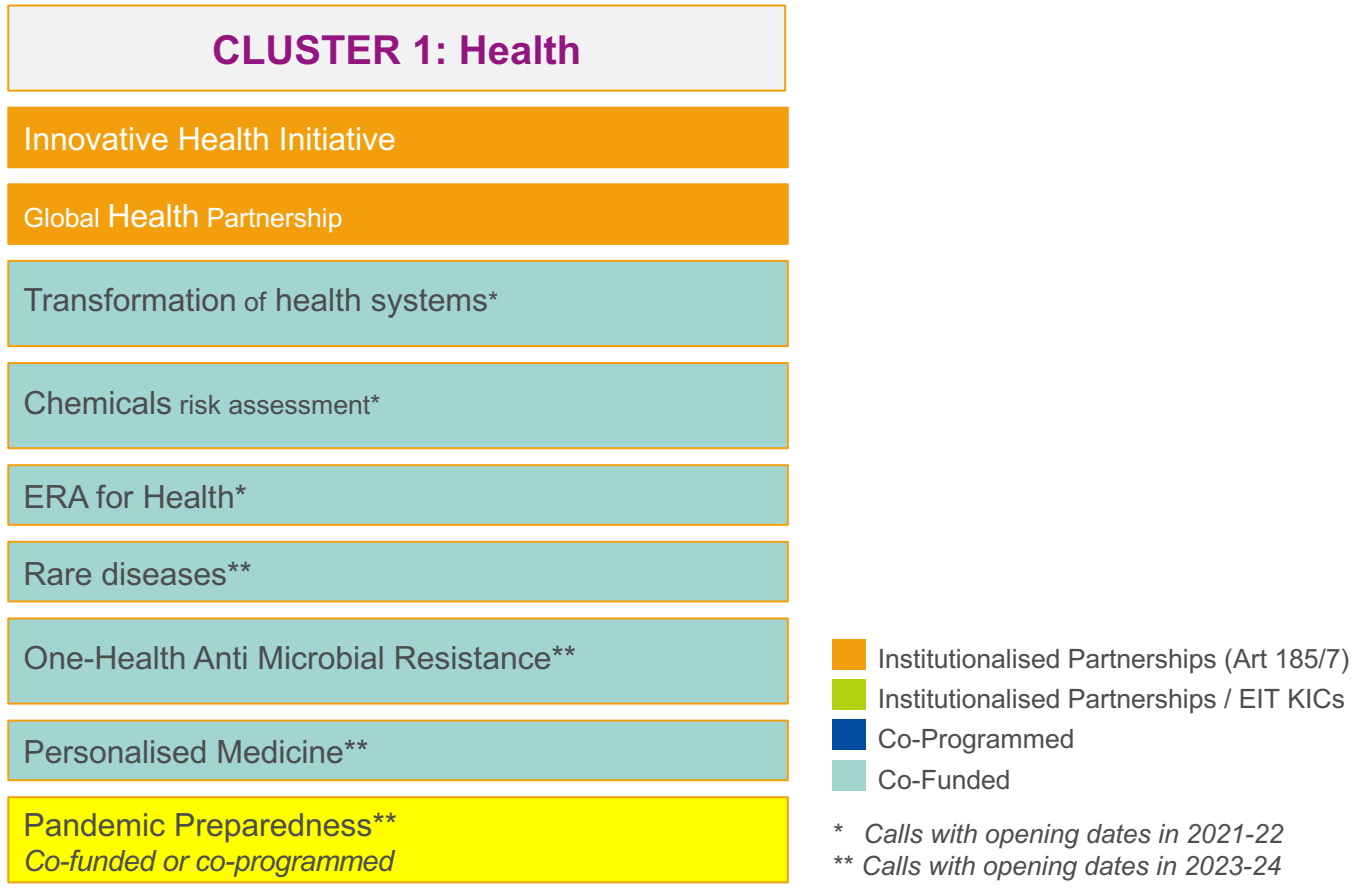
INSTITUTIONALISED
Based on long-term dimension and need for high integration; partnerships based on Art 185/187 of TFEU and the EIT legal acts for 2021-2027

European Commission

Horizon Europe – 49 European Partnerships

Partnerships

- Strategic Plan identifies 49 co-programmed and co-funded partnerships:
  - public-public;
  - public-private;
  - global initiatives.
- EC proposals for 10 institutionalised partnerships based on Art 185/187 (23 Feb 2021)



European Partnership on Health and Care Systems Transformation

Aims to contribute to the transition towards more sustainable, resilient, innovative and high-quality people-centred health and care systems

- ✓ *provide multidisciplinary R&I actions in priority areas to fill knowledge gaps, produce evidence and develop guidance on how to transform health and care systems*
- ✓ *develop new solutions for health and care to support and maintain people's health*
- ✓ *improve the ability of relevant health and care actors to take up innovative solutions, including organisational, service and policy innovations*
- ✓ *establish a platform for connection and coordination of relevant stakeholders*

- partnership under preparation, expected to launch calls in 2023
- benefits of TO-REACH support action, EIP-AHA, AAL2, JPI MYBL

European Partnership for Personalised Medicine

Aims to coordinate research in personalized medicine between the EU, EU countries and regions, and international partners

- ✓ *faster uptake of research and innovation results into clinical practice*
- ✓ *secure Europe's position in state-of-the-art healthcare provision*
- ✓ *facilitate a shift from a 'one size fits all' approach towards taking into account individual differences and better utilising the accumulating data to manage health, disease and its predisposition*
- ✓ *sustainable health systems and independence in data intensive healthcare*

- partnership under preparation, expected to launch calls in 2024
- benefits of past PM calls, the ERA PerMed Cofund and the ICPerMed

European Commission

PERMIT – PERsonalised Medicine Trials

- funded by Horizon 2020 and coordinated by the European Clinical Research Infrastructure Network (ECRIN)
- aims to establish, with all relevant stakeholders, recommendations ensuring the robustness of personalised medicine trials, including validation of the stratification methods
- November 2021 - a workshop to prepare a report on the ethical and data protection issues in personalised medicine research
- one of the focus areas will be on **patient involvement in the co-design of personalised medicine research programs**, including complex clinical trials
- representatives from patient organizations in different disease areas, as the **applications of complex trial designs in personalised medicine are now expanding beyond the field of cancer into other diseases**
- **increasing health literacy among patient communities** is essential for better patient involvement in the co-design of trials

European Commission

European Partnership for Rare Diseases

Aims to develop diagnostics and treatments for rare diseases through multidisciplinary research and innovation programmes

- ✓ *shorten the average time to correct diagnosis to 1 year*
- ✓ *have 1000 new therapies for rare diseases and methodologies to assess the impact on patients by 2027*
- ✓ *provide an efficient and effective "pipeline" from research to healthcare to ensure that R&I results reach patients as quickly as possible*
- ✓ *collect and share relevant rare disease data at EU and international level*
- ✓ *provide evidence for fit-for purpose regulatory framework*

- partnership under preparation, expected to launch calls in 2024
- benefits of past RD calls, EJP on Rare Diseases and the International Rare Diseases Research Consortium (IRDIRC)

European Commission

EJP on Rare Diseases

- brings over 130 institutions (including all 24 ERNs) from 35 countries
- to create a comprehensive, sustainable ecosystem allowing a virtuous circle between research, care and medical innovation
- the **training activities for rare disease patient representatives** aim at:
  - ✓ providing patients with knowledge of the therapeutic development and regulatory processes for medicinal products in the field of rare diseases;
  - ✓ equipping patient advocates with the knowledge and skills required to become legitimate collaborators in rare disease scientific and translational research;
  - ✓ empowering patient representatives in their roles as valued and efficient partners in research and scientific projects;
  - ✓ empowering paediatric patients with knowledge, skills and comprehensive educational tools covering some of the main knowledge gaps related to the paediatric developmental specificities.

European Commission

CONCLUSIONS

- While very complex, the EU health research funding landscape has become more focused, more inclusive, impact driven and less bureaucratic.
- The quality and sustainability of health systems will depend increasingly on finding more efficient, patient-tailored treatments, as currently envisaged by the personalized medicine and rare disease areas. Perceived expensiveness will be offset by economies of scale and reducing inefficient prescriptions.
- In conjunction with the development of targeted treatments, the recourse to complex clinical trials is expected to increase tremendously.
- Appropriate patient involvement at all levels will be paramount to ensure the safe and effective running of complex clinical trials.
- Empowering patients with the necessary roles, knowledge and skills required to collaborate effectively in the design and running of complex clinical trials will ensure the success of our common endeavor.

Thank you!

HorizonEU

http://ec.europa.eu/horizon-europe

A clinician & investigator view point

Birgit Geoerger, MD

Gustave Roussy Institute, France

Patient involvement breakout session

EFPIA European Federation of Pharmaceutical Industries and Associations

Norwegian Medicines Agency

Panel session

Our speakers who set the scene, are now joined by

- Martin Brunner, Medical University of Vienna, Austria
- Dimitrios Athanasiou, World Duchenne Organisation, PDCO member
- Mireille Muller, Novartis

Breakout Session #3
Patients' Involvement
Questions for discussion

EUPATI Patients United · efpia European Federation of Pharmaceutical Industries and Associations · BARC · PPD · Norwegian Medicines Agency

Experience, and stakeholder views

1. Do you have experience in involving Patients in the design of CCTs?

2. What are the main elements to consider to involve fit-for-purpose Patients

Challenges & Solutions

1. Looking particularly at collective learning and precedence, what can we learn from previous experience of patient involvement in CT design?
    - From a patient representative perspective, and
    - from a sponsor perspective?
2. How to get patients involved?

New frontiers

1. Is there a need to document Patient Involvement in the CT Application? How could this be done?

2. How to use the law at the best to optimize patient involvement in drug development?

Call for action

1. Call for the future? ALL & Audience

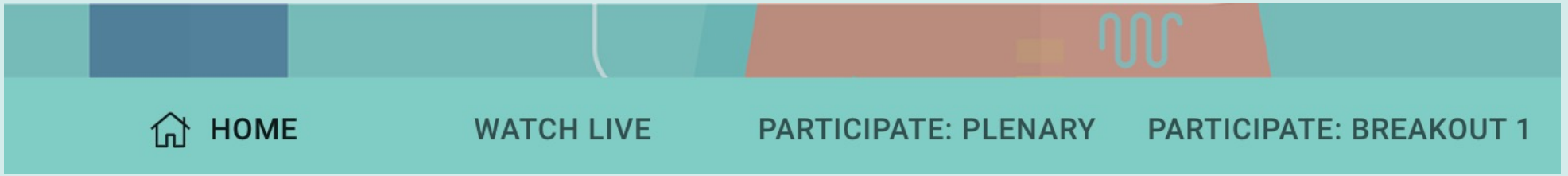
Attendees' questions

It’s time to…

How to go back to the plenary session?

As a viewer

Click on the “home” and “Watch Live” respectively in the navigation and find the continued plenary session and click on “Live”.



As an active participant

Close the zoom session of your breakout session and go back to the webinar platform and chose the continued plenary session. If you are an active speaker, panelist or moderator, click the ”Participate: Plenary” link.