Breakout session feedback

BO session 1

Design of Master Protocols

NF Patients United | efpia European Federation of Pharmaceutical Industries and Associations | BARC | PPD | Norwegian Medicines Agency

Key outcomes

1. *Importance of an early patient engagement*
2. *Health Authorities welcome the use of fit for purpose master protocols*
3. *Need to clearly formulate at an early stage the objective of the trial, the endpoints and key design aspects*
4. *More experiences in early stages, in later stages we need to ensure we identify the right opportunities, apply the right available methods (incl. finding trade-offs) and discuss key design challenges with regulators and HTA agencies early*
5. *Need to ensure appropriate sharing of data (during the CT and beyond)*
6. *We need to learn from the existing trials and from each other: as an example, Covid platform trials have shown that different stakeholders, academics and regulators can align to design master protocols*
7. *Opportunities to expand its use in rare disease and paediatric trials*

Potential solutions/call for action

- Need to explore the use of master protocols in confirmatory settings as multi-sponsored studies
- Need to increase alignment on trial design across regulatory agencies and HTA agencies
- Need to develop efficient knowledge sharing platforms between all the key players (academics, sponsors, regulators, HTAs) to share the learnings and discuss how to advance the field
- Separate general clinical trial challenges from the challenges specific to master protocols to advance the field
- Need to manage clear accountability by careful agreement upfront
- Ensure patients are part of the whole process and are involved early

Feedback from Breakout session #2 on “Regulatory Processes and Systems”

Moderators: Anja Schiel & Lucia D’Apote

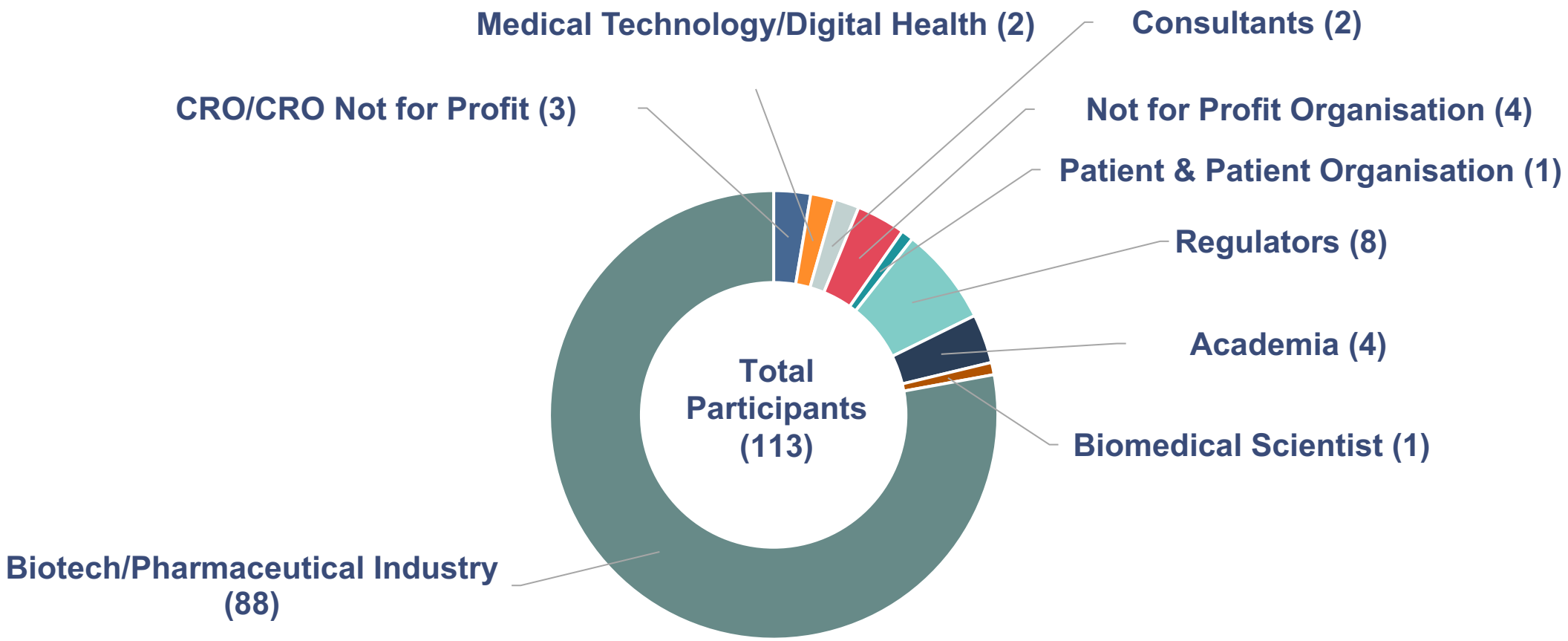
EFPIA European Federation of Pharmaceutical Industries and Associations

Norwegian Medicines Agency

Our objectives

- Compare and contrast experience from interaction with regulators on CCTs
- Identify how to reach ideal state: ***a Clear, Agile, Collaborative regulatory process that enables acceptance and use of CCTs in the EU***

Who attended this Breakout session?



Moderators and Panellists

- **Anja Schiel** - EMA SAWP, NoMA (breakout session co-chair)
- **Lucia D'Apote** – Amgen, EFPIA (breakout session co-chair)
- **Antony Humphreys** - Head Regulatory Science Strategy Task Force, EMA
- **Elke Stahl** - Chair CTFG-BfArM
- **Tomas Boran** – Director Marketing Authorisation Section, SULK - EU-IN
- **Dionne Price** - Director, Division of Biometrics IV, CDER, FDA
- **Niklas Hedberg** - former Chair EUnetHTA, TLV
- **Juliana Sholter** – Amgen (case study #1 presenter)
- **Dieter Haering/ Marius Thomas** – Novartis (case study #2 presenters)
- **Stéphanie Kromar** – EORTC (case study #3 presenter)











Key outcomes

Need in the EU for a process and system where we can all share information on CCT and learn

- Several learnings from FDA CID Pilot programme
- Currently there is no EU platform adequately agile and comprehensive to support a CCT pilot programme but there is commitment to explore options and establish it.
- Possible options: INNO Project platform, ITF+ (interim?), Link SAWP/PDCO with EU-IN (MNSA), build on experiences from the EMA-EuNetHTA dialogue
- Possibility to enhance collaboration/dialogue between EMA and FDA on CCTs
- Experience and training need for the system and assessors
- Consider needs of different stakeholders, including HTAs and payers, when advising /accepting CCTs .
- Involving patients is critical, they also need to understand the pros and cons

Feedback from Breakout session #3 on “Patient Involvement”

Moderators: Claas Röhl & Solange Corriol-Rohou

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Our 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.

Panellists

To set the scene:

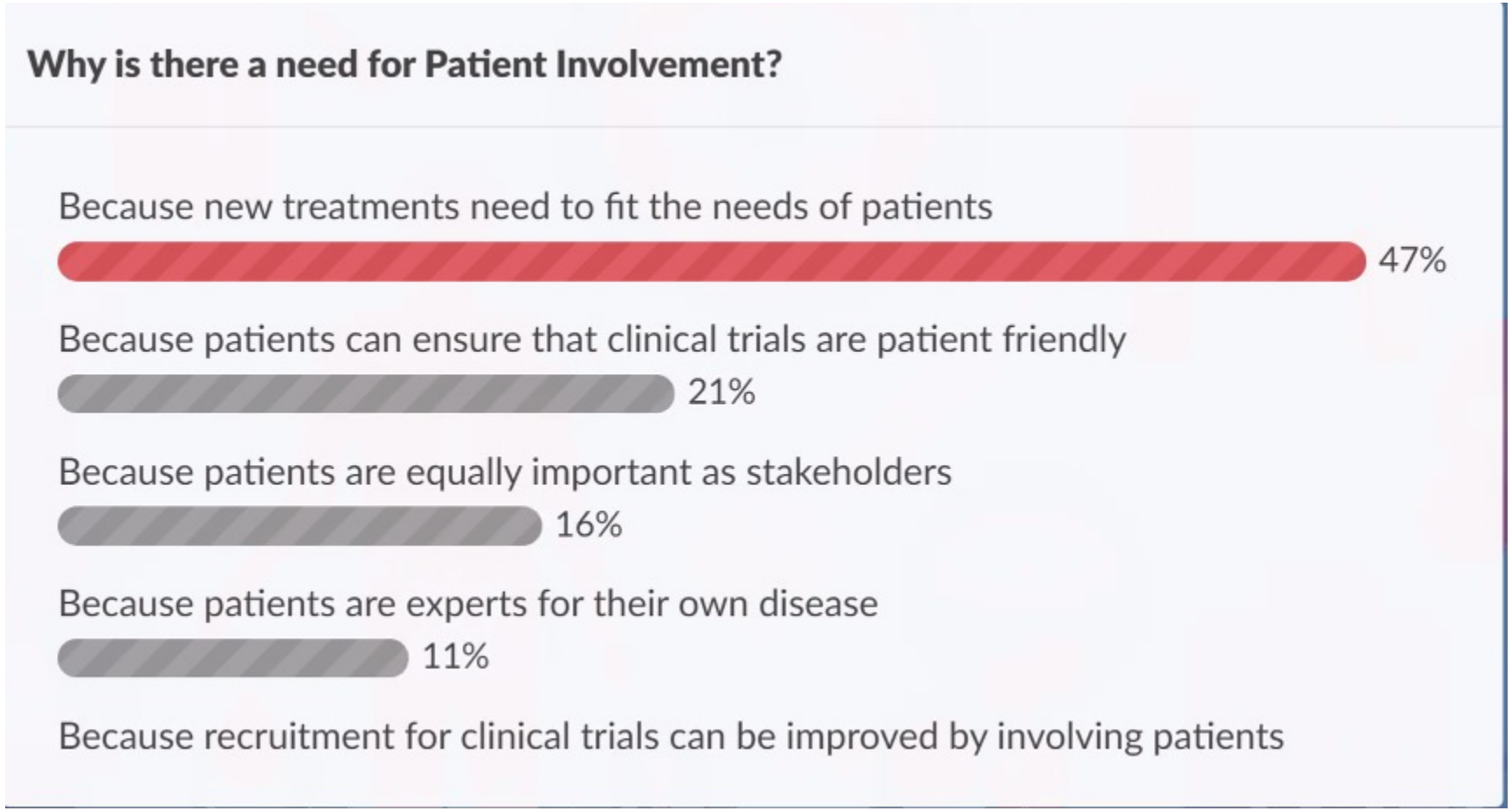
- **Ann Marie Janson Lang** - Swedish Product Medical Agency - MPA, and CFTG co-chair
- **Begonya Nafria Escalera** - Patient Engagement in Research Coordinator)
- **Nathalie Seigneuret** – the Innovative Medicines Initiative
- **Alexandru Costescu** – EU Commission
- **Birgit Geoerger** – Gustave Roussy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

Joined by:

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

Which stakeholder group do you belong to?

Why is there a need for Patient Involvement?



Key outcomes

- Paradigm shift: from a drug centric approach towards a more patient-centric approach
- Terminology: *when determining the appropriate body(ies), Member States should ensure the involvement of laypersons, in particular patients or patients' organisations – CT Regulation Preamble*
  - Clear differentiation between lay persons & patient advocates - every caregiver is a 'specialist
- Ensure early and meaningful patients' involvement since the best approach to guarantee CT designs are patient-centric
  - Patient engagement is crucial in all trials, incl. in CCTs
  - Complexity doesn't concern the trials only, but also other elements, e.g., rare disease, HAs divergences
  - Involvement of patient organizations in CT is different across MS - language barrier
  - Need for true patient involvement and collaboration – it shouldn't be a ticking the box exercise
- Involving patients in CT requires planning and expertise ➔ a need for a more systematic approach
  - Patients' organisations and established networks, e.g., YPAGs can help sponsors for patients involvement in CTs
- IMI and EU funded projects are key and have shown their value
  - Need to secure sustainability and output implementation better
- UK MHRA is also promoting patient involvement in CT development, and has a dedicated team
  - work very closely with the UK Ethics Committees, i.e., through combined reviews of CTs in the UK (Ethics and MHRA) which will become the norm in Jan. 2022.
  - ILAP (Innovative Licensing and Access Pathway) is a new scheme recently launched which has specific tool for focussing on Patient Involvement.

Potential solutions/call for action

- Is there a need/value to document Patient Involvement in the CT Application?
  - Develop a section in the CTA which denotes at the co-design stage the perspective from the patient/participant/carer and provide details on how their input made a difference to the design

  ➔ Still an open question, which would need further discussion

- Ensure early and meaningful involvement of the patients in CT design
  - Companies to ensure that the CT teams value Patients' involvement and feedback
  - How to consider/aggregate the feedback from multiple countries?
  - Is there a need for contractual framework to ensure proper remuneration, but also confidentiality?
  - How to measure its impact and make it visible? There is a risk not to involve patient

  ➔ Optimise the use of the Patient Engagement Tool Box developed by IMI Paradigm

- Need to work towards the capacity building of the patient's community; they need good background and expertise on CCT

- Training for patients and patients advocates, but also for other stakeholders, e.g., physicians, regulators

for an engaged discussion!