European Federation of Pharmaceutical Industries and Associations

What are the methodological priorities from an industry perspective on patient centricity, inclusion and representativeness?

Mireille Muller

Version: final



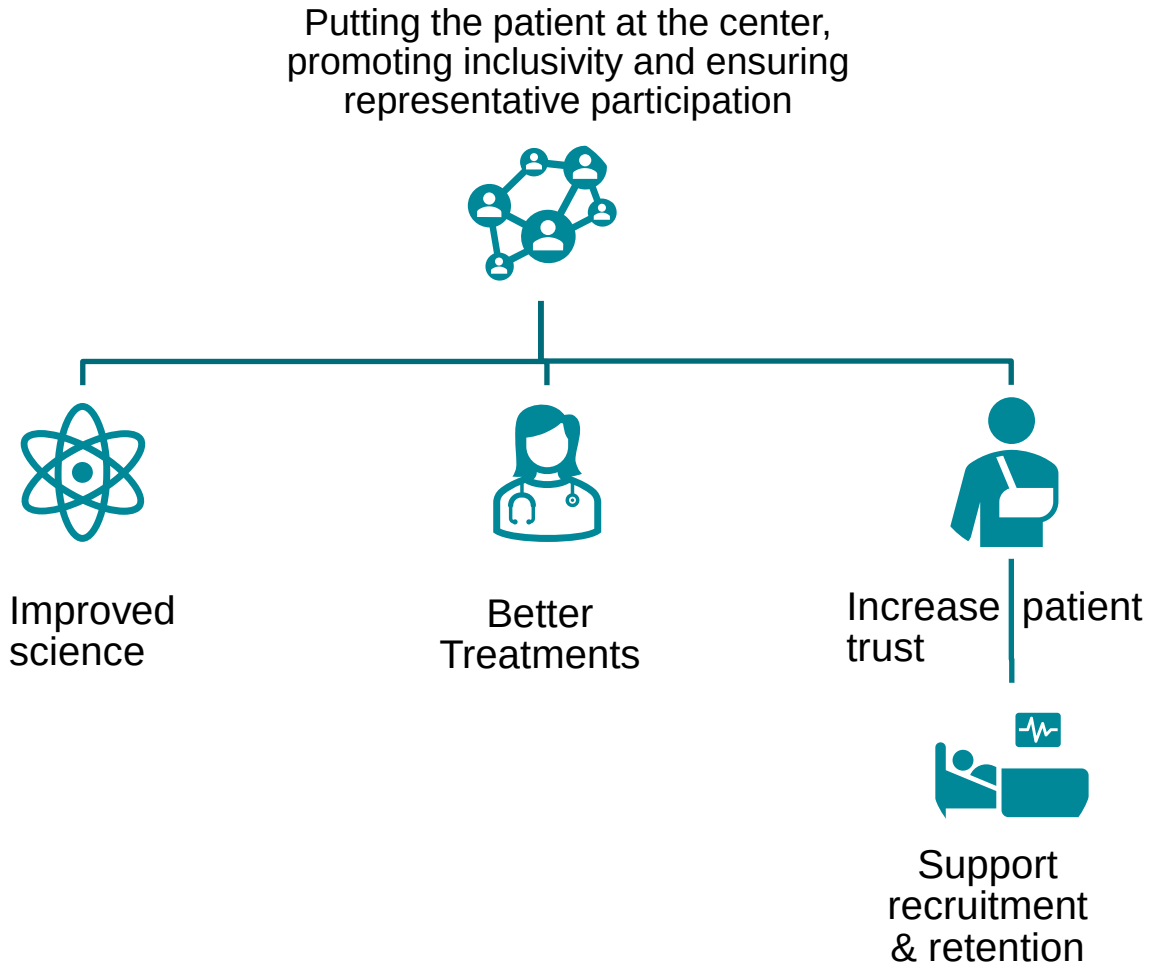



ACT EU workshop on methodology

23 November 2023

Background

Why is it important?



What is the situation in Europe?

In many EU countries health service is easily accessible by more or less everyone

However, a main question remains: Can a treating physician expect that a medicine, as investigated in clinical trials, will have a similar efficacy/safety profile compared to the patient in front of her/him?

Collective work is needed to settle on diversity enrolment goals to further address the patient centricity question

There is a need for discussion with regulators to align on the purpose for looking at diversity, namely, to have evidence about how a new drug works for each patient in clinical practice

Patient centricity

How does EMA/EMRN define patient-centric/patient-focused drug development and Patient Experience Data (PED)?

What type of decisions does EMA/EMRN expect to be impacted by PED?
For example:

- to optimise clinical trial design
- to inform COA selection
- to inform BR assessment
- Others?

What are EMA/EMRN's expectations regarding quality of PED?
For example:

- Data collection
- Analysis
- Methods
- Study types
- Other considerations?

What type of communication & dialogue related to PED does EMA/EMRN expect from industry?
For example:

- Scientific Advice meetings for patient preference studies
- Use of PED in briefing book for towards end of phase II
- Others?

What guidance does EMA/EMRN plan to release to help industry meeting these expectations?

Inclusion (pre and post approval)

Definition

What is the current EMA thinking to define diversity for clinical trials?

- FDA refers to under-represented populations, MHRA to under-served groups, Health Canada to representative CT population, considering both intrinsic and extrinsic characteristics:
  - Is it primarily representativity (according to certain factors) or focus on sub-populations that may have different disease presentation or may respond differently to the product / intervention or all of these?

Data sources

How to validate the data sources (for setting recruitment goals) used?

- How to capture clinical trial population data when there are country restrictions?
  - No standardized approach for data that should be used to benchmark global diversity enrollment goal planning and no standardized trial patient data demographic collection plan due to variances in country regulations
  - Given the lack of an EU-wide database such as SEER in the US, and the vast diversity between EU member states, what databases/sources should be leveraged from an EMA perspective, to ensure that enrolment is reflective of the population that is affected by a particular disease
  - How much and how harmonized can demographic information be realistically collected when it comes to EU clinical trial participants, given the limitations to collecting personal information (GDPR, some national limitations to collect information on race) ?

Genetics

How relevant are genetic differences for a potential differential treatment ?

- While inclusion *per se* is not expected to cause a differential treatment effect, it can be related with a causal factor e.g. being in good general health condition

What are the considerations on establishing ONE set of enrollment goals?

- How to recruit and retain diverse trial populations, yet not delay recruitment in trials?

Safety

How to adequately protect vulnerable people? For example, pregnant or very old people

- What are the implications for safety?

Enhancing the inclusivity of clinical trials leads to more robust and generalizable results that benefit a broader range of patients

Representativeness

With race and ethnicity being very US-centric constructs, what guidance can be offered on how to define diverse representation on a country/regional level?

- What would be EMA's minimal requirements for representativeness?
- What is EMA's current thinking on applicability of global data (ex-EU) collected through multi-regional clinical trials for the registration of drug products in the EU?

How to organise representativeness in a global development programme?

- How to meet at the same time the FDA and national/regional requirements?
- Is there room for cross-HA, international collaboration on a more unified guidance for industry?
- What is the potential for extrapolation where inclusion is not straightforward i.e. number of a group of representative people is too low?

What measures are being undertaken in the EU to ensure that sexes are appropriately represented in clinical trials and that potential differences in outcomes between males and females (incl. efficacy and safety outcomes) are considered?

- It is known that there may be sex differences in epidemiology, diagnosis, prognosis, treatment outcomes etc across a number of diseases.

European Federation of Pharmaceutical Industries and Associations

Thank you