How can we create a more welcoming and innovative environment for clinical trials?

The public trialist’s perspective

The European Hematology Association (EHA)

October 22, 2024 | Martin Kaiser

First-hand experience of running a public (academic) external comparator arm trial

OPTIMUM trial (NCT03188172) for newly diagnosed high-risk myeloma

- Designed and run out of necessity as an external comparator trial: RCT was first choice
- Done in consensus between study group and patient organization:
  - **Very rare, but high unmet need group; not addressed through commercial research**
  - **No treatment standard defined at time of design**
    - **Lack of recruitment incentive perceived as biggest risk to any progress for patients**
    - **Balanced by risk of results not being considered sufficient → reimbursement**

→ **Multi-disciplinary team (statisticians, trial/data managers, clinicians, patients)**

→ **Trial was a success (https://doi.org/10.1200/JCO.22.02567)**

- **Recruitment 10 months ahead of projections, despite complex screening process**
- **Marked improvement in outcome (PFS and OS), hardly any patients needing to stop therapy**
- **Results with payers (under review)**

What could be ingredients for more innovation, patient-centricity and attractiveness in clinical trials?

Having a clear focus on unmet patient need (including equality, equity)

Making public clinical trial results matter

- Public (academic) clinical trials can offer a unique opportunity for testing new concepts in a controlled environment:
- Low-risk examples could include label extensions, post-exclusivity trials

Supporting sustainability of public clinical trials

- Reporting societal value of generating, maintaining and utilising public data (e.g. diagnostics)
- Facilitating delivery of innovative public trials

Growing tomorrow's talent to advance innovation

- Supporting training and career development of multi-disciplinary public clinical trials teams

A welcoming environment for public clinical trials can provide innovation for all stakeholders