EUPATI CASE REPORT on meaningful patient involvement in R&D and regulatory affairs

Patient input into breast cancer study design

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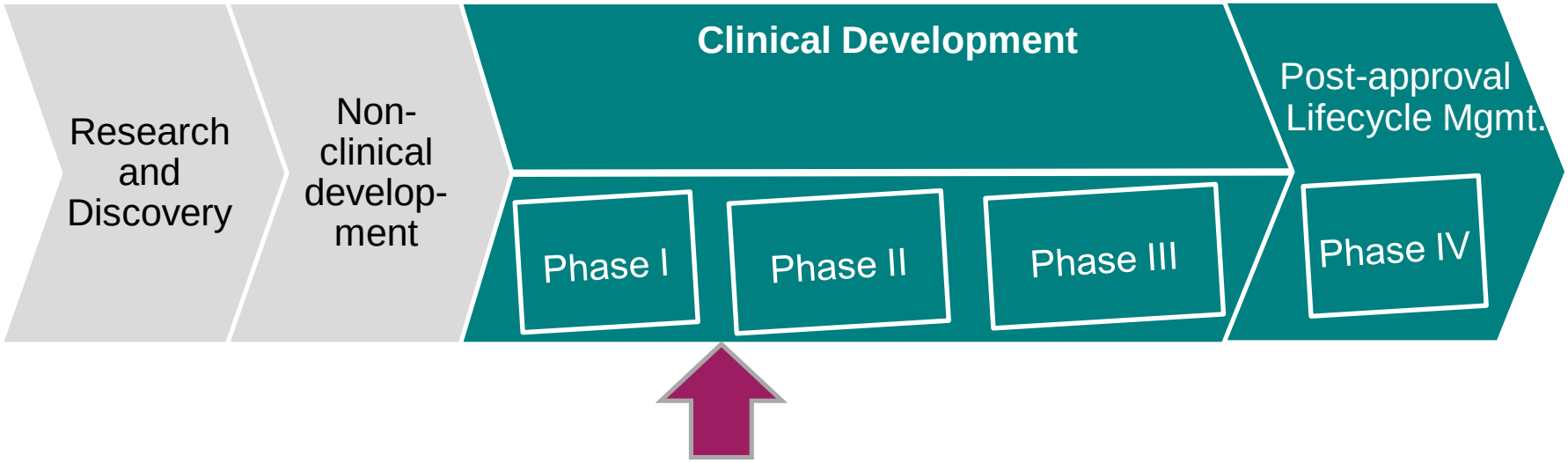
PARTNER(S) INVOLVED:
Commercial Service provider

Description of the case (how were patients involved in the R&D project? What was the objective?

- Breast cancer is a new disease area for MSD.
- We sought patient input into draft phase II (proof of concept) study design to improve probability of success in terms of generating patient-relevant data, whilst also meeting current regulatory needs.
- Two face-to-face focus groups were held. The first was relatively ‘pragmatic’ selecting women who were available on the day. The second was consciously chosen to be ethnically diverse and representative of North American population likely to be recipients of the treatment.
- The sessions were organised and mediated by a third party provider. Initially, the name of the sponsor was NOT shared, to avoid any pre-conceptions about the company, but our R&D staff were involved in person.
- Feedback was collated into themes and taken into consideration as the protocol was developed.

RESEARCH/DEVELOPMENT PHASE



Type of patient (advocates) involved

[X] Patients with personal disease experience

[ ] Expert patient / patient advocate with good expertise on disease, but little R&D experience

[ ] Expert patient / patient advocate with good expertise on disease and good R&D experience

[ ] Other:

Challenges and barriers (and how they were overcome, or which ones were unresolved)

- How best to organise this – Do It Yourself, Third part provider
- Practical arrangements – payment, confidentiality, ratio of patients to Pharma Company staff, representative sample size
- Influence of any pre-conceptions of the company or the product (high profile media product in US)
- Willingness of clinical team to engage, in particular if patient suggestions were not incorporated

Benefits (how has this collaboration improved R&D process(es) and the R&D outcome(s) or triggered R&D organisational change)

- Feedback fell into three broad themes:
  - the choice of comparator
  - the timing of unblinding of an individual patient
  - the option for crossover at point of progression
- Two of these were readily incorporated into the protocol, the third formed part of discussions with regulators prior to protocol finalisation.
- None of the issues was a surprise but the patient contribution influenced the final design.

Discussion and learnings for the company and EUPATI

- The input was generally considered a positive experience and influenced final study design. Whether that leads to a better protocol, faster recruitment, better adherence, higher probability of success at regulators or reimbursement etc remains to be seen
- External guidance on best practice will be helpful – contracting, fair-market value, confidentiality needs etc.
- How best to ‘select’ patients.
- How to engage beyond US.
- Are there shared learnings …. Maybe a publication on “what women with breast cancer want from a clinical trail” to reduce the need for each company to repeat.