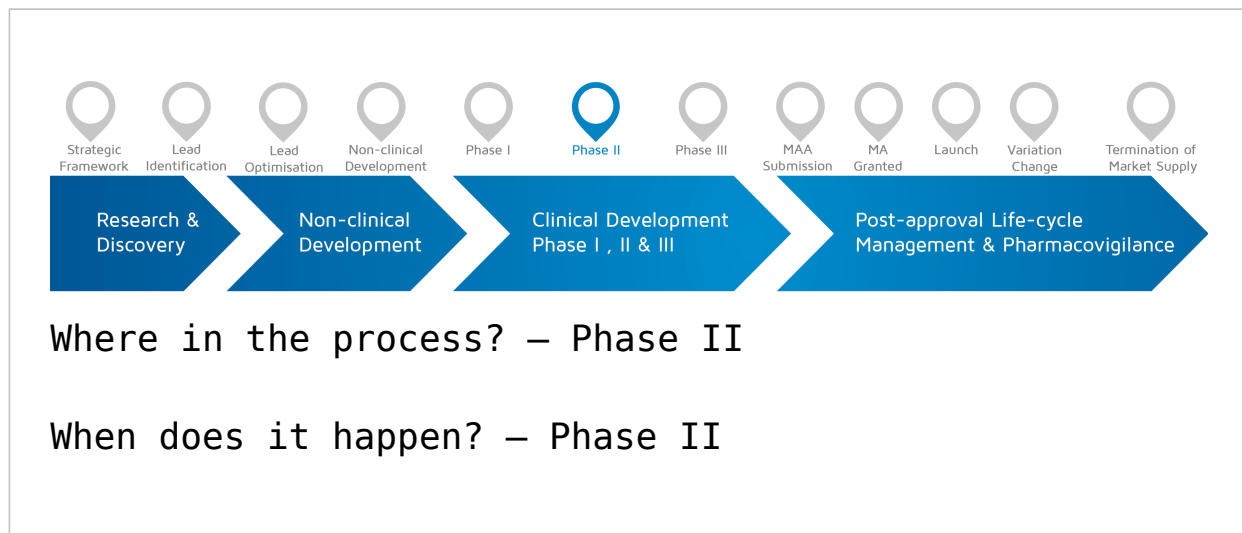


Patients Involved – Patient input into breast cancer study design

Introduction

A case report on Phase II patient involvement into Proof of Concept study design, to improve probability of success generating patient-relevant data and meeting current regulatory needs.



Description of the case

Breast cancer is a new disease area for Merck Sharp & Dohme (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.

Type(s) of patient (advocates) involved

Patients with personal disease experience.

Merck Sharp & Dohme.

Benefits of patient involvement

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.

Challenges and barriers

- 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.

Learnings

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 trial’ to reduce the need for each company to repeat.

A3-breast-cancer-study-design-V1.0

Attachments

- Patients Involved Case Report – Patient input into breast cancer study design

Size: 863,773 bytes, Format: .pdf

An infographic describing a patients involved case report of patient input into draft Phase II study design to improve probability of success in terms of generating patient-relevant data, and meeting current regulatory

needs .