Challenges in Exploratory Clinical Research

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$2.6 billion average cost of a drug

What we are used to...

10 000 molecules → Candidate drug → First administration to humans

0 YEARS 4 6 12.5 14 20 YEARS
FROM DISCOVERY TO REGISTRATION OF A DRUG FROM REGISTRATION TO LAUNCH FROM LAUNCH TO PATENT EXPIRATION

Basic research
Preclinical development
Clinical studies
Registration
Market access

| Phase I: small cohorts healthy volunteers | Phase II: relative small patient cohorts | Phase III: large patient cohorts | Phase IV: postmarketing |

Challenges in Exploratory Clinical Research
Increasing complexity of early clinical trials through innovation, new techniques and adaptive trial designs

• Implementation of study specific and safety related biomarkers;
• Introduction special procedures (CSF, biopsy, different types of fluid sampling);
• Implementation and use of novel technologies, wearables and devices (e.g. smartphone applications, etc.);
• Adaptive trial design.
Bringing patient into early phase clinical trials

• More combination protocols (including SAD, MAD and patient parts)

• Need for early patient data readout in order to reduce timelines and costs

• Patients are treated by Health Care Professional (HCP), who often work in hospital environment

• Majority of hospitals are still in a learning cycle to expand their clinical trial expertise to include phase 1 trials

• There is an urgent need for increased patient engagement; — for example by contacting patient organisations, exploring options on social media, etc.
Roadblocks

Trial related
- Complex
- Strict phase 1 I/E criteria leading to high screening failure

Patient related
- No therapeutic benefit
- Limited Proximity phase 1 facility
- Limited knowledge

Investigator related
- No therapeutic benefit
- Often placebo controlled
- No phase 1 capacity
- Limited scientific interest
- Less publication possibilities
- Less compensation
- Limited in time

Limited in time
Redirecting Phase 1 patient trials to hospitals and patients

Patient Centric

➢ Responsible HCP always involved;
➢ Trials nearby home, reducing travelling time;
➢ Possibility of home visits.

Investigator Centric

➢ Always individual contact with research physician of CPU;
➢ Early involvement in I/E criteria;
➢ PI role or recruiting role;
➢ Different levels of support (logistical, administrative, trial execution);
➢ Possibility to support at any location;
➢ Contracts in place to speed up timelines.
Patient trials: Flexible trial execution

- Screening
- Randomisation
- Medical care
- Hospital network
- Recruitment
- Study specific assessments
- CPU
- Medical care
- Patient Organisations

Challenges in Exploratory Clinical Research
Patient trials: Need for hospital networks

- Screening
- Randomisation
- Medical care
- Recruitment

Hospital network

- Patient Organisations
- Recruitment
- Study specific assessments
- Medical care
- CPU

- Scientically interested physicians
- Phase 1 unit
- Contracts

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Support at different levels

Recruitment can be supported by recruitment officers and contact with patient organisation.
Patient trials can be conducted at different sites and settings with the quality of Phase 1 trials.

Mobile unit equipped with Medical and Lab equipment (centrifuges, fridges, freezers, scales, ...and an electronic Data Capture system.

Support can vary from providing validated equipment to full trial conduct.
Critical selection points during feasibility

- Database
- Social media
- Advertisement
- Patient organisation

Feasibility study

- Kill
- Not Ready
- Go ahead

- Recruitment
- HCP
- Hospital Unit
- Support
- Mobile Unit

Define Roles and Responsibilities in contracts and work orders

Trial execution

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Studies are investigator centric and tailor made

1 PI
1 Trial center
# Support models

# PI’s
# Trial centers
# Support models
Trial designs - HCP in the role of PI

Trial execution at Hospital site

- Logistical, administrative support and performing of trial assessments
- HCP can act as a recruiter only and/or perform Trial specific assessments (as defined in R&R)
- Possible use of mobile Unit

Trial execution at CPU

- HCP: referral only or perform trial specific assessments (as defined in R&R)
- CPU: Full Trial execution
Trial designs - CPU in the role of PI

Trial execution at Hospital Site

- Logistical, administrative support and performing of trial assessments
- HCP can act as a referral and perform Trial specific assessments (as defined in R&R)

Trial execution at CPU

- HCP: referral only or perform trial specific assessments at CPU (as defined in R&R)
- CPU: Full Trial execution

CPU assumes the role of PI
Key Messages

• World of clinical trials is changing
  – High need of quick early patient data
  – Cost & timeline reduction
• Recruitment and trial execution in early patient trials is challenging
• Building HCP network is crucial for recruitment
• Patient centricity is mandatory
• Move towards investigator centricity
Thank you