

Integrating Expanded Access into Rare Disease Drug Development

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Topics

- When during the development process should an Expanded Access Program be opened?
- What are the major challenges to operating an EAP?

**When during the development process
should an EAP be opened?**

In Rare Disease EAP Can Compete With Clinical Trials

- Most rare diseases do not have adequate treatments
 - May have high demand for access to investigational medicines
- Finding patients for rare disease clinical trials can be very challenging
 - Rare disease are rare
 - Challenges with accurate diagnoses
 - Disease experts may not be used to running clinical trials
- EAP patients very unlikely to qualify for clinical trials because of prior exposure to investigational medicine
- Patients themselves very unlikely to want to stop EAP to join a clinical trial

Reminder: General criteria for early access programs

Serious or life-threatening illness

No comparable or satisfactory alternative therapy

Potential benefit justifies potential risk and risks acceptable in context of disease

Sufficient supply available

Will not compromise drug development process

Questions the development team should ask themselves:

What is the nature of the disease?

- Life threatening, irreversible outcomes, rapidity of progression?

What is known about the investigational therapy?

- Scientific rationale, evidence of potential benefit and risk, dose, type of product?

What is potential impact of an EAP on the drug development program?

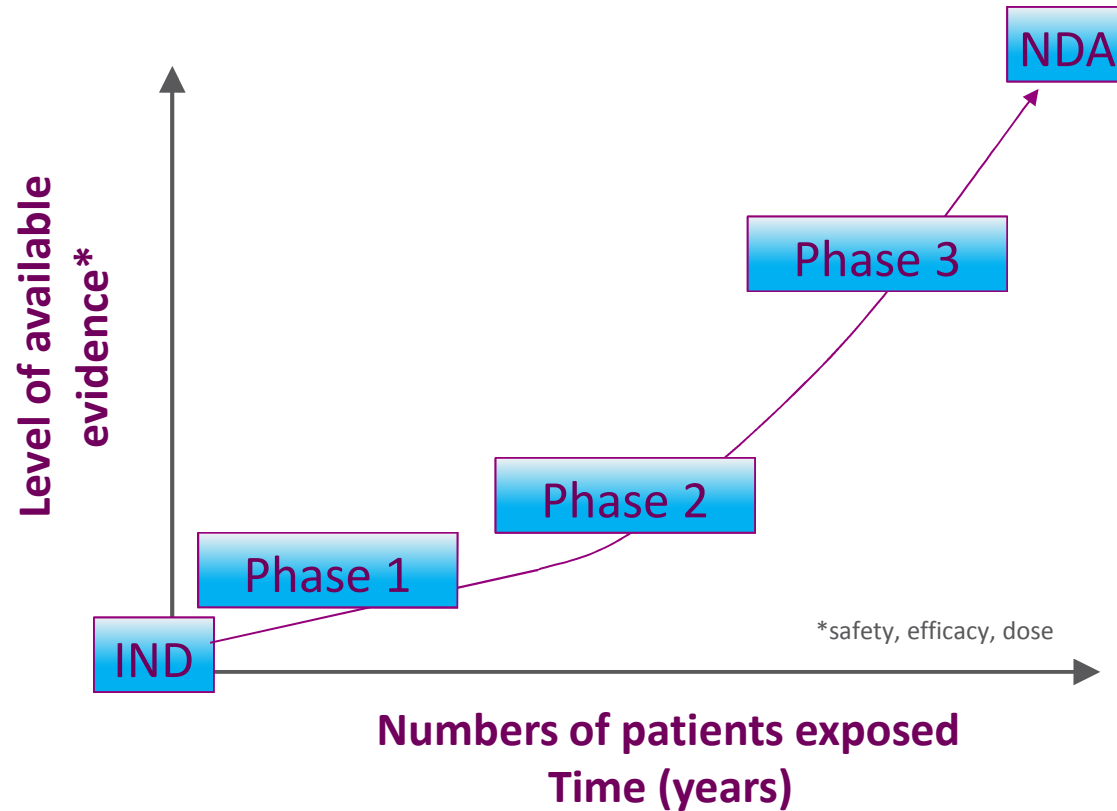
- Will effective development be jeopardized? Clinical trial option? Drug supply?

Are there spontaneous requests coming in from patients or physicians?

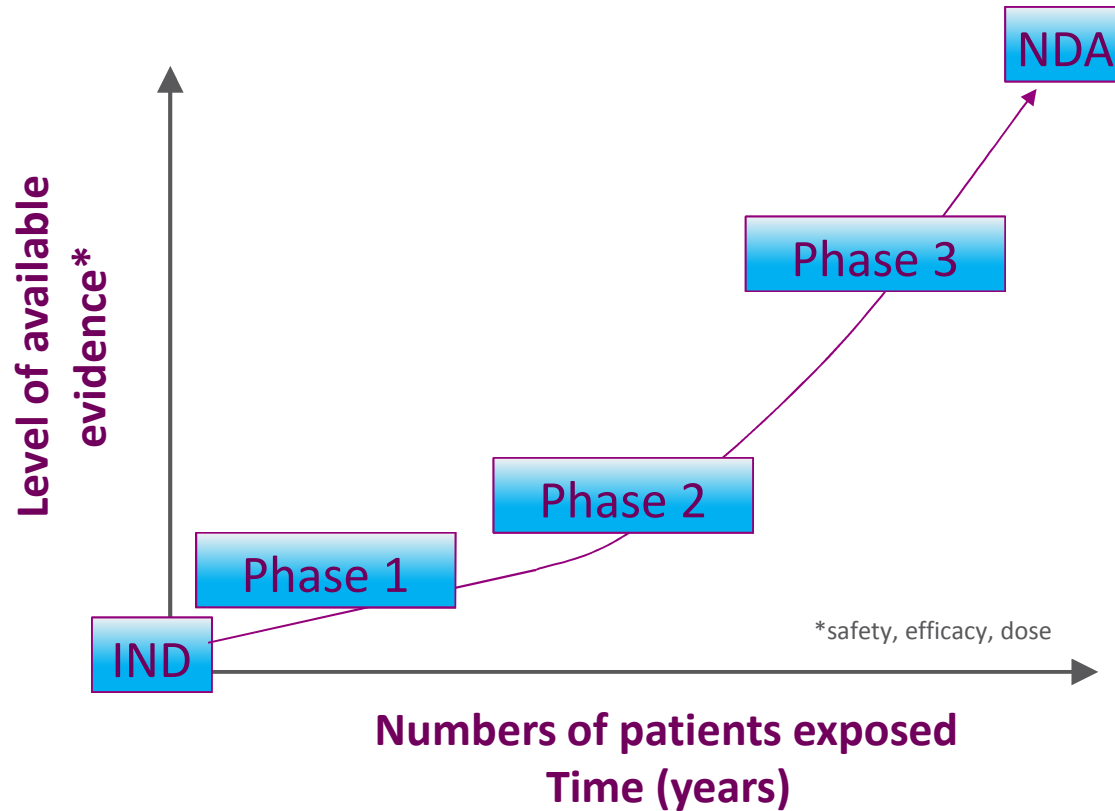
What is the company's policy?

What is the right thing to do?

Mapping the key questions for an EAP to the phases of clinical development



Mapping the key questions for an EAP to the phases of clinical development



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What are the major challenges to operating an EAP?

Some major challenges to operating an EAP

Internal alignment to start an EAP

- Do we have enough info to determine risk/benefit in context of disease?
- Will the EAP interfere with enrolling patients in our clinical trials?
- Will we generate safety data that will derail our program?
- If we offer comp use to 1 – 2 patients, will we be flooded with requests?

Sorting out **disease-specific criteria**

Competition with clinical trials program for **resources** (drug, people, and time)

Developing operational **efficiency** through repetition

Managing physician expectations – it takes time for all the logistics prior to shipping IP

Getting necessary **paperwork** from treating physician

Getting it wrong could be detrimental to **patients**

Getting it wrong could be detrimental to **company reputation**

Thank you

