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ICORD Meeting

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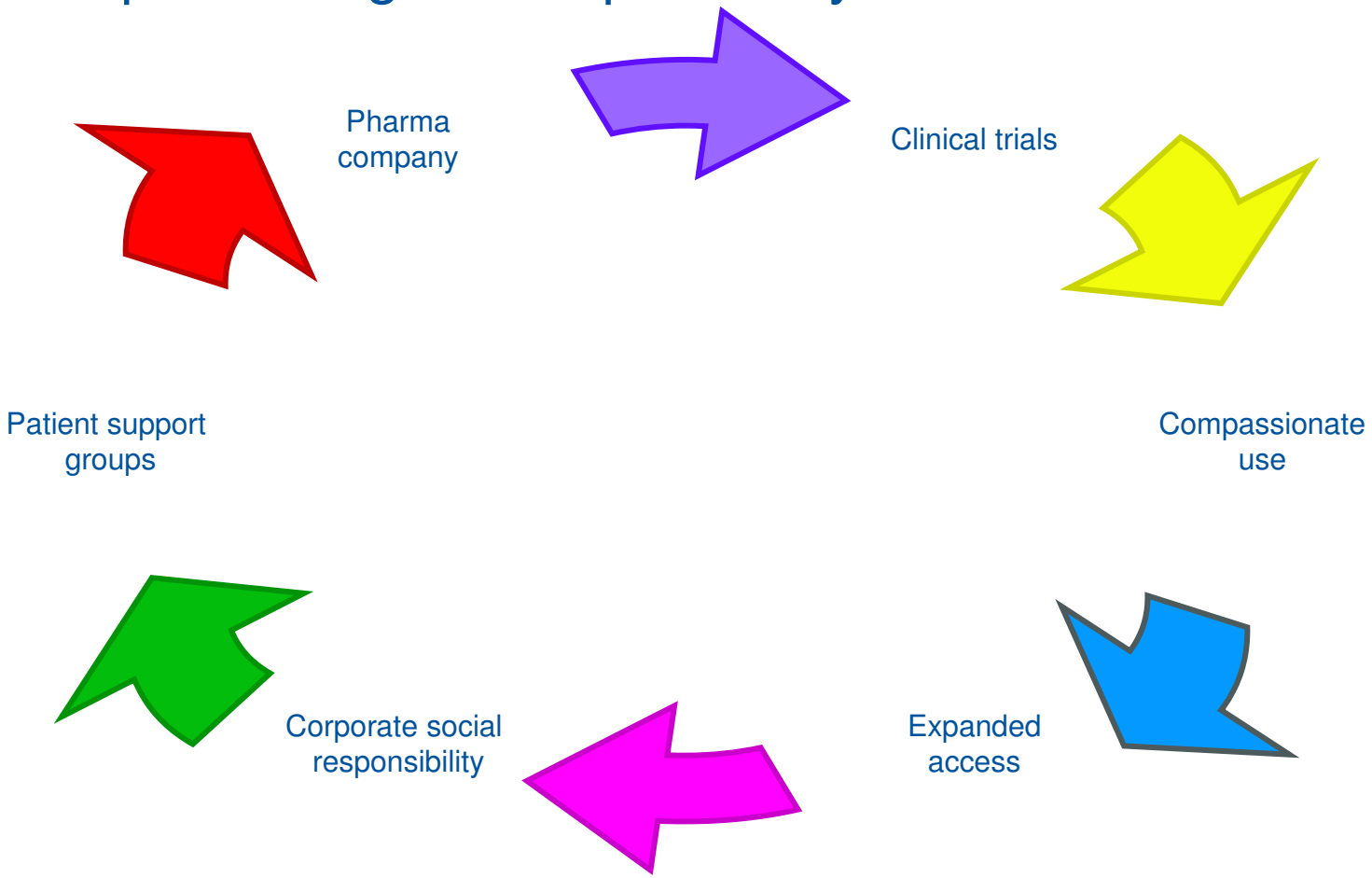
Orphan Drug Development Cycle



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Topics

- Clinical trials
- Compassionate use
- Expanded access
- Corporate social responsibility

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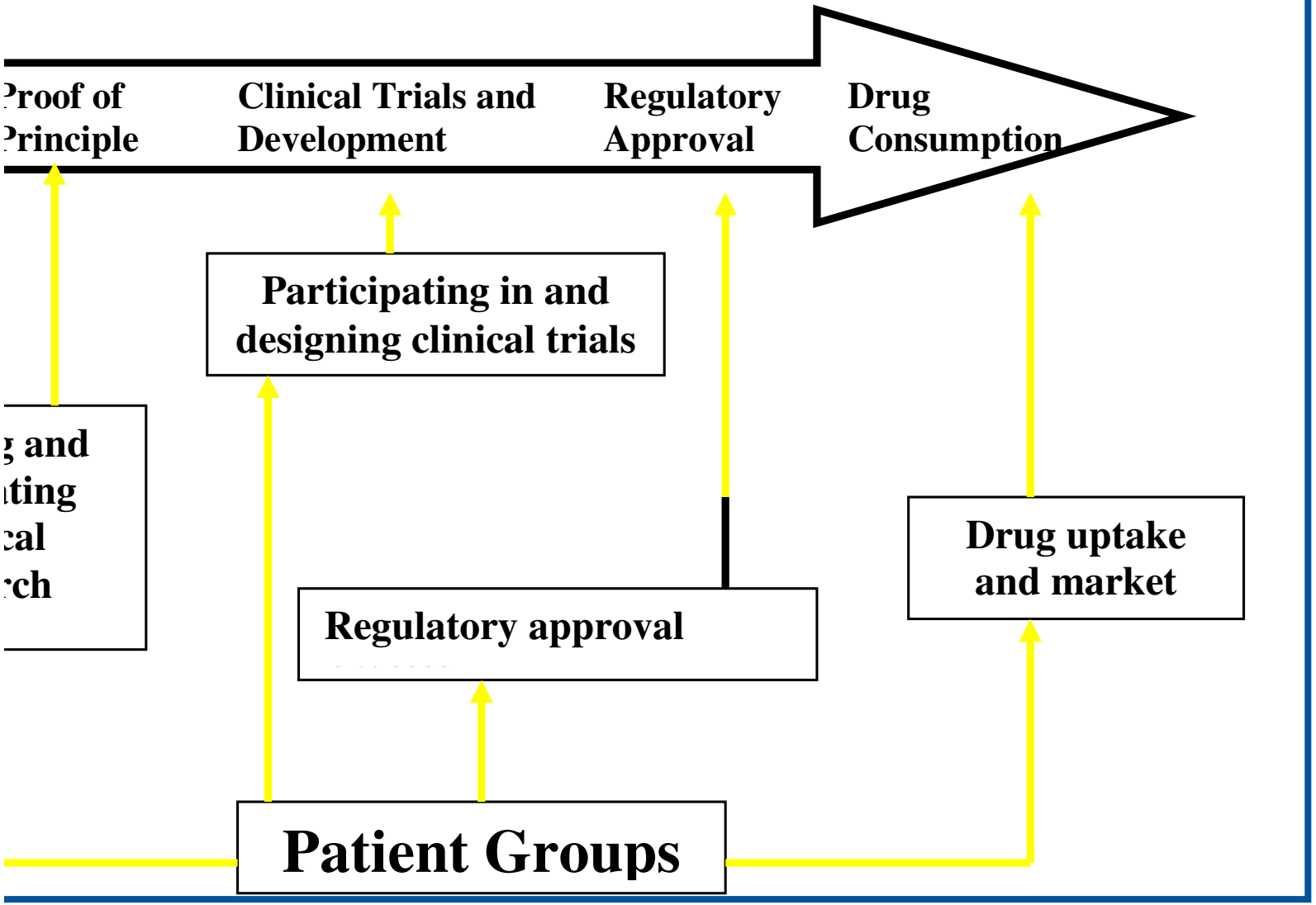
Orphan Drugs

- Strong collaborative relationship between:
  - patients
  - physicians
  - pharma
- Frequent direct contact at advisory boards, physician meetings and patient support groups
- Relationships can be stressed if clinical trials are not successful

Patient Groups and the Innovation Process



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Compassionate Use

- Important for patients with life threatening illnesses
- Single patient use is costly and difficult to obtain
- Provides little value to the drug development process
- May raise unfounded safety concerns
- Compassionate use may last for years

Expanded Access

- Phase III studies completed
- Commercial drug available
- Accountability can be difficult
- Costly to companies

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Corporate Social Responsibility

- Broad availability of medication
- Medication access programs
- Unbiased product education for HCP's and patients
- Unrestricted financial support for patient groups
- Investigator sponsored trial support

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THANK YOU