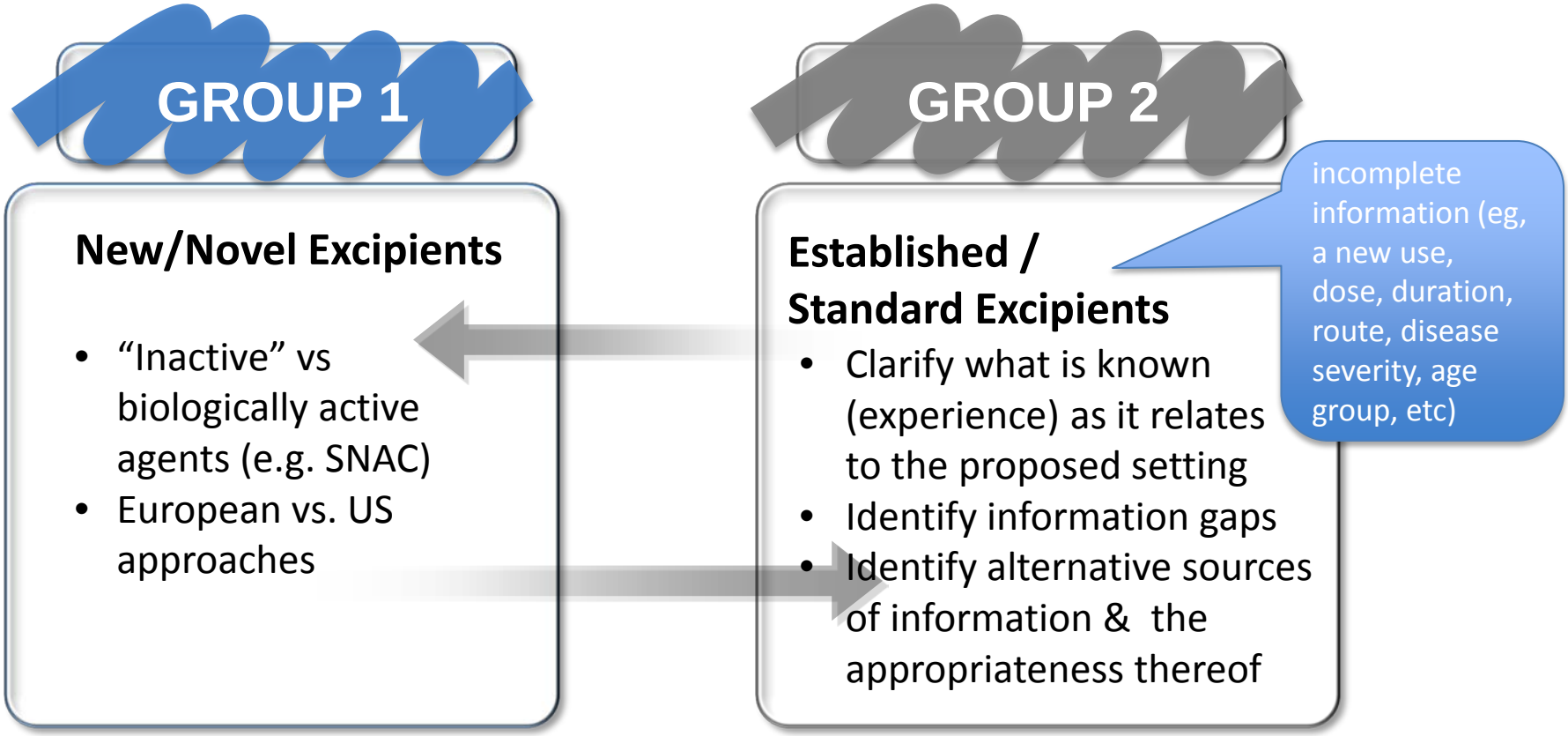
Session 5: Case Scenarios & Questions to Inform Development of a Risk Assessment Framework

Two Breakout discussion groups:



Breakout session discussion

How to justify excipient use (novel, established) in paediatrics? What are the hurdles?

Risk assessment & Information needs

- Can a **common template or approach** (framework) be developed for implementing risk assessments for individual excipients?
- What **minimum information** is required? What additional data is required?
- What circumstances and factors should be considered regarding the justification for **juvenile tox studies**?
- Should toxicology studies with the final formulation be conducted? If so, **when & which studies**?
- What **alternative options** are available if no additional information is available?
- What **clinical trial design factors** can be incorporated to provide information on the safety of excipients?
- Where are the **knowledge gaps** and how would you **prioritize studies needed** to approach the evaluation of excipients for paediatrics?

Information sharing platform

- Where to find the existing information?
  - Platform to share information? (eg, STEP database)
  - extending the FDA inactive ingredient database to paediatrics

Proposed Framework – Your opinion matters!!

- Would the proposed framework help address the issues of use of excipients in paediatrics?
  - What are the **pros and cons** of the presented framework ?
  - What **additional elements** would you consider in the framework?
- Can we evaluate data on excipients & present in a format which will satisfy regulators?

Breakout Deliverable: Proposed Framework to Assess Safety; Needs, Possible Solutions

1. **Is the use of the excipient justified?**
2. **Is there an established regulatory guideline or opinion?**
3. **Is there relevant prior human use? (pharma, food)**
4. **Is there other supporting information?**
   - **Clinical – Adult, Paediatric, PK, PD, Modeling**
   - **Nonclinical – Toxicology, PK/PD**
   - **Similarity to other excipients?**
5. **Trials/Studies to fill the gaps**
   - **Design, Value**

Consider relevance to intended Age, Route, Dose, Duration, Disease, etc … What are the Gaps?

Better info sharing?

- What are the **pros and cons** of the presented framework?
- What **additional elements** would you consider in the framework?