Industry views on benefits of Adaptive Design to clinical development

Judith Quinlan
Director Statistics ; Biopharmaceuticals
GSK

State of Industry

- Pharmaceutical industry is under great pressure to develop innovative drugs with increased efficiency
- High attrition rate throughout life cycle
  - Even in phase III (50%)
- Being asked to do more with less…does that sound familiar to anyone?
- Patients are also a valuable resource
- Growing patient pressure to bring life saving drugs to market sooner
  - Examples of patient action to access drugs in development

State of Industry

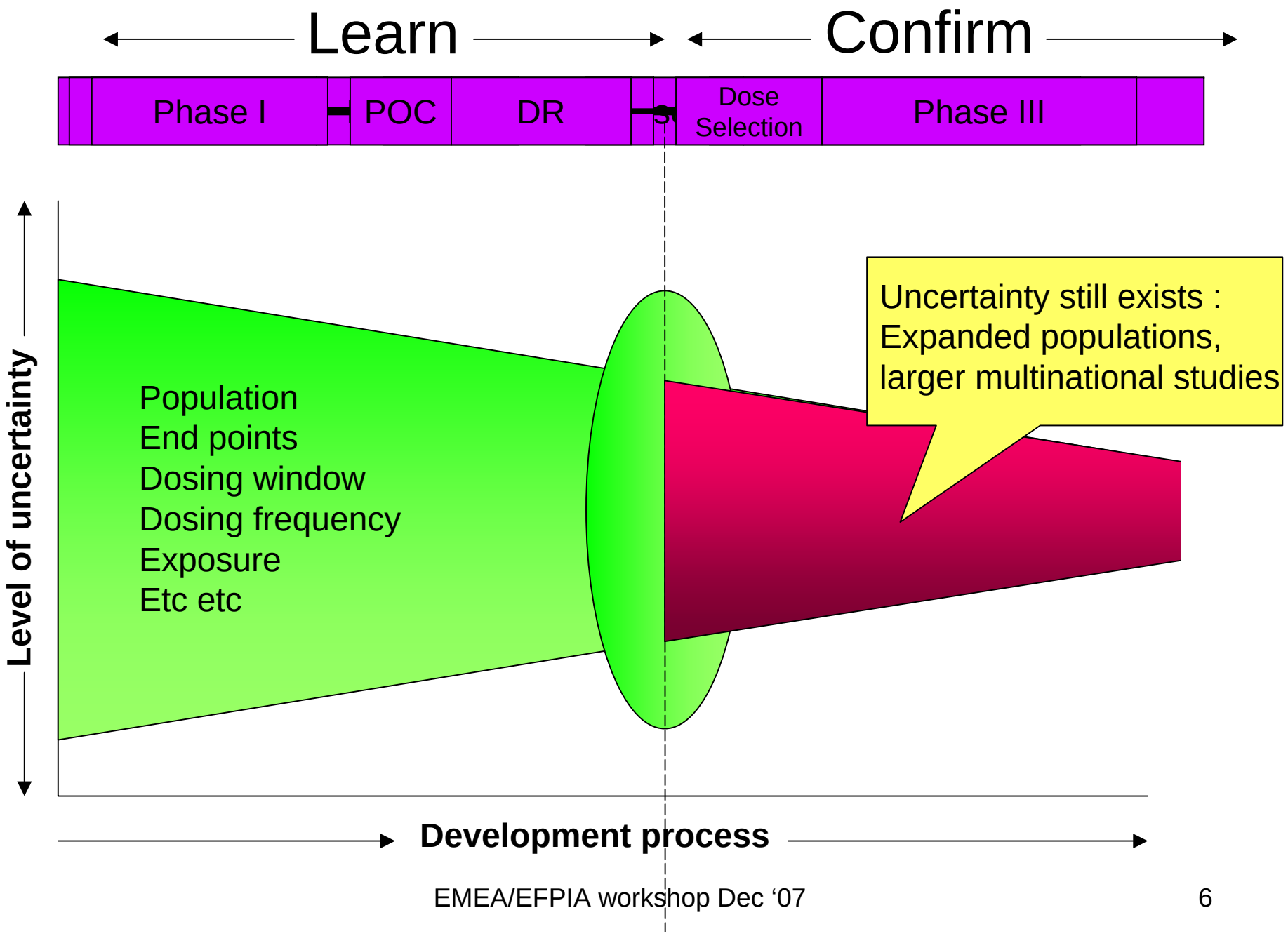
- Agencies in difficult position
  - pressure to speed drug approvals and simultaneously need to be careful to balance decisions to ensure public health safety
- Scrutiny of development process
  - Attempt to modernise the process at every stage
    - From promising compound through to approved product
- Collectively driving the need for novel approaches to better understand our assets earlier in life cycle
  - One potential area for innovation is study design

Why should anyone be interested in adaptive designs?

- **Our common goal is to make valuable medicines available to patients and doctors,…. that are safe, effective, and affordable**
  - Adaptive designs are one of many tools to move us towards that goal
- **Not** an argument to simply promote speedy development and reduce costs
  - Benefit is to improve development process
  - Better answer the ‘appropriate” development questions
- Ethical
  - Exposure of fewer patients to ineffective treatments
  - Provide better stewardship of resources (not just cost…all competing for same patients)

Why should anyone be interested in adaptive designs?

- Improve drug development process through better understanding of dose response and improved dose selection
- Seamless II/III
  - Improved understanding : simultaneous collection of surrogate and clinical endpoints on same patients
  - Improved understanding of dose and AE profile
- Transparency and consistency of results


Learn
Confirm
Phase I
POC
DR
Dose Selection
Phase III
Level of uncertainty
Population
End points
Dosing window
Dosing frequency
Exposure
Etc etc
Uncertainty still exists :
Expanded populations,
larger multinational studies
Development process

TARGET : Transitioning adaptive designs to confirmatory evidence arena

Moving forward : Full agreement between industry and regulatory agencies

- Adaptive designs are not an excuse for poor planning
  - In fact require more upfront planning
  - Statistically
  - Managing logistics of implementation

- **Plan,** Plan, Plan!!

Building blocks for moving forward

- Education
- Developing best practices
- Working together : fostering closer collaboration between agencies and industry to educate and develop best practices
- Learning together from the lessons learnt from case studies
- Openness and transparency
- Not to let the perfect be the enemy of the good