Quality: is it just a fairy tale?

Mario Pennisi, CEO QCTN Inc.

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Outline

- Introduction
- QCTN and its Members
- Regulation and Clinical Trials Processes
- Challenges for the Clinical Trials Industry in Queensland

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Global Pharmaceutical Sales by Region, 200
(in billion)

World Audited Market	2006 Sales in US$	% Global Sales	% Growth year-over-year
North America	289.9	47.7	8
Europe	181.8	29.9	4.8
Japan	56.7	9.3	-0.7
Asia, Africa & Australia	52	8.6	12.9
Latin America	27.5	4.5	12.9

Source: IMS Health

> 15% spent on R&D/Clinical Development

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Australia: Active Biotech & Device Industry

BIOTECHNOLOGY	2006
Number of core biotechnology companies (listed and not)	427
Number of listed biotechnology companies	76
Total market cap of listed biotechnology companies	$15.3 b
Total revenue of listed biotechnology companies	$3.3 b

R&D 15% ~ 495 million AUD

DEVICES	2006
Number of medical devices companies	625
Number of listed devices companies	48
Total market capitalisation	$11.2 b
Total revenue	$2.6 b
Total employment across both sectors	12,100

R&D 15% ~ 390 million AUD

Total employment across both sectors	12,100

Source:
2007 BioIndustry Review - Australia & New Zealand

Kelvin Hopper and Lyndal Thorburn, Innovation Dynamics Pty Ltd

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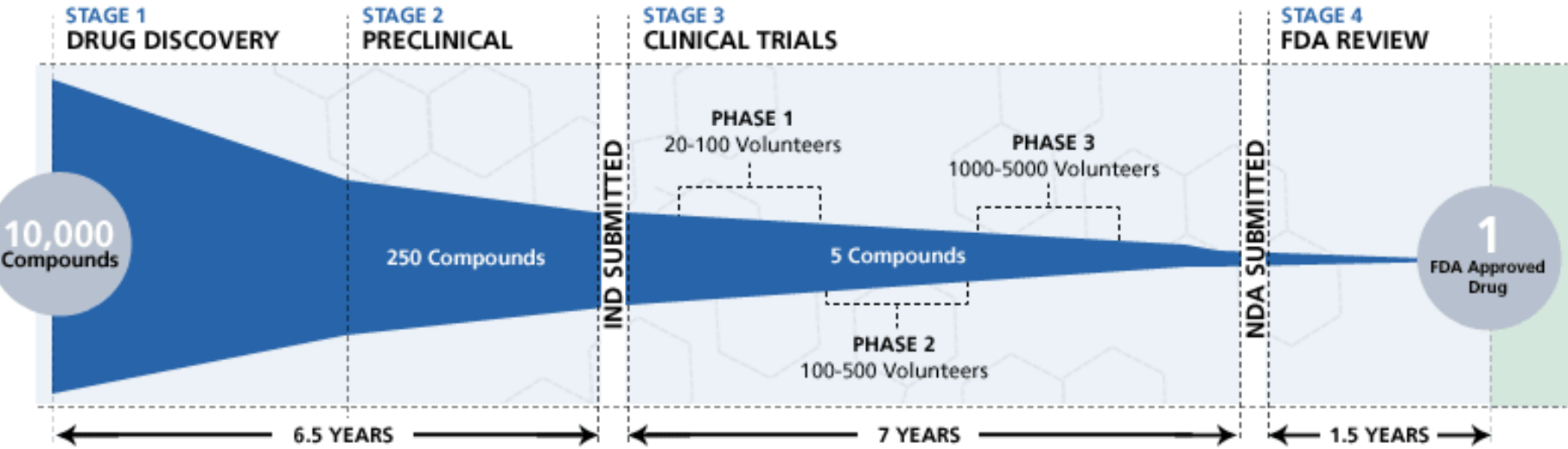
Drug Development (Leaky) Pipeline



- In the 10 years to 2000, for the then top 10 Pharmaceutical companies
  - 38% of experimental drugs dropped out in Phase I
  - 60% of remaining candidates dropped out in Phase II
  - 40% of the remaining dropped out in Phase III
  - 23% of those through the clinic failed to be approved by FDA
- Cost of getting a drug to market >US$ 1 billion

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Who undertakes Research & Development o new Drugs or Medical Devices?

- **Pharmaceutical companies**
- **Biotech companies**
- **Medical Device companies**

And

- **Life sciences R&D service providers**
  - Public: eg. university, hospital
  - Private: eg. Phase 1 Clinical Research Units, Contract Researc Organisations (CROs)
- **Quality of Services – impacts value of data obtained**
  - Due Diligence costs!

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Survey Phase 1 Unit in Australia*

"Australia has established a competitive and professional industry for the conduct of early phase trials to world-class standards with a sustainable growth path"

- **Revenue**
  - > AUD 50 Million
- **Business Sources**
  - 50-70% business from US, Europe, Japan
  - 50% repeat business
- **Competitive Advantage**
  - Current regulatory system (CTN/CTX)
  - Quality of work
  - Standards of care and medical infrastructure
  - Cost effective compared to US/Western Europe

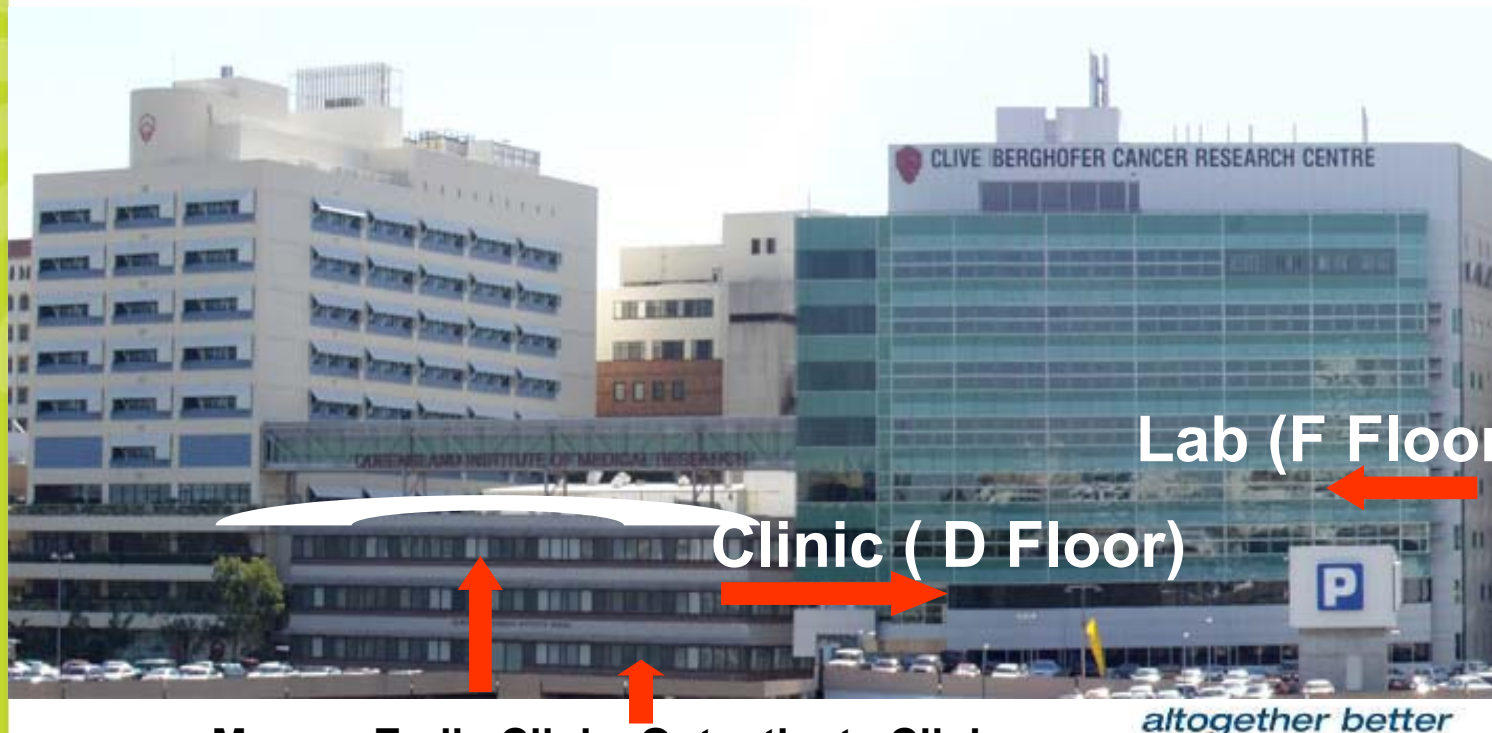
* Source: Report on a Survey of Australia's Phase 1 Clinical Trials Units, Pharmaceuticals Section, Dept. of Industry, Tourism and Resources, Australian Federal Government, September 2007

Example: Q-Pharm Pty Ltd

Brisbane, Queensland
- Royal Brisbane & Women's Hospital Complex
- Co-located within Queensland Institute of Medical Research

Specialists in early phase clinical trial

- Inspected by the FDA
- Phase I/II
- Pharmacokinetic and Pharmacodynamic studies
- Proof of Concept studies
- Bio-Equivalence/Bio-Availability



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TetraQ

- Located at The University of Queensland in Brisbane, Queensland
- State-of-the-art facilities
- ✓ GLP recognized
- Member of the Queensland Clinical Trials Network
- ✓ Bio-analytical lab: NATA accreditation/compliance with ISO/IEC standard 17025-1999

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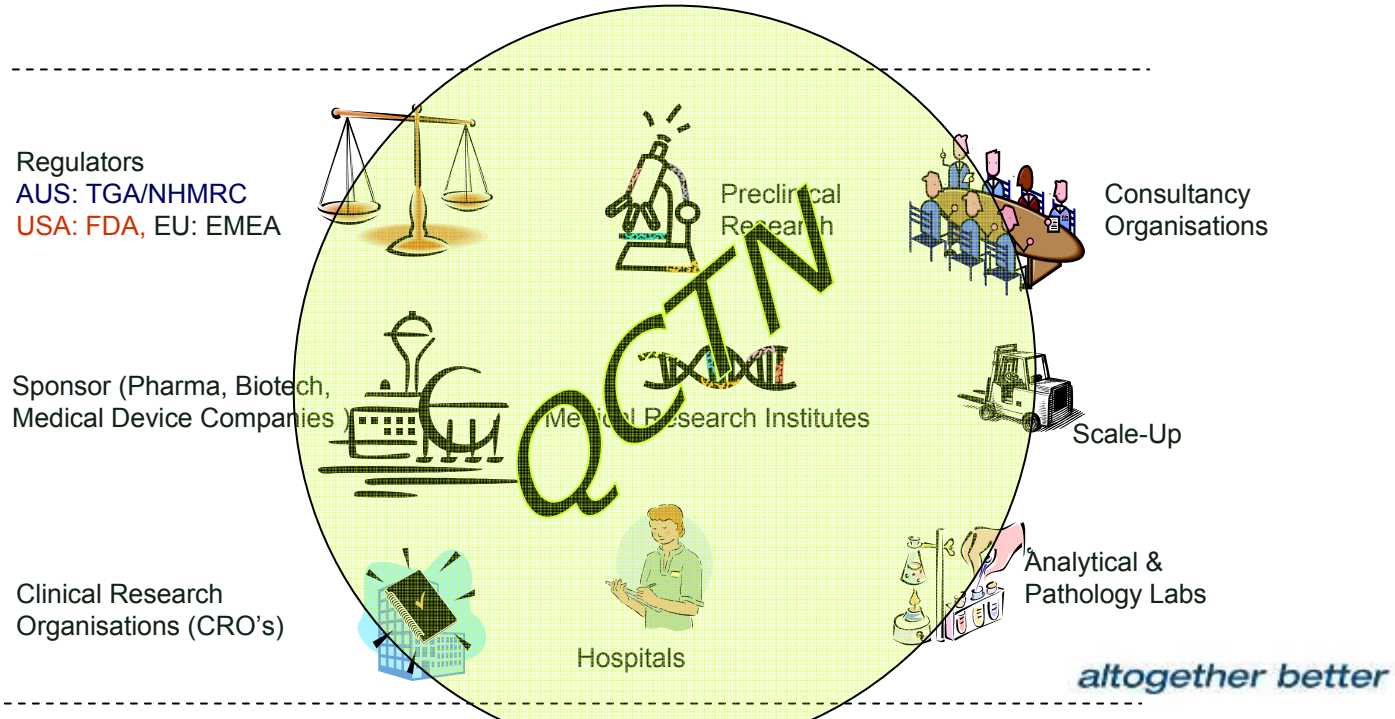
Queensland Clinical Trials Network Inc.

- QCTN is an independent association of life sciences service providers and hospitals ("umbrella organisation")

- Aim: To help organisations undertake preclinical and clinical trial activities in Australia (one-stop shop)



Clinical Trials Capabilities of QCTN Members

- **QCTN Members cover all aspects of drug development**

Clinical Network Services	CRO
Q-Pharm	Phase I-II Unit
ERA Consulting	Regulatory Consultancy
TetraQ	Preclinical Drug Profiling
The Wesley Research Institute	Clinical Trials Centre
Core Research Group	Clinical Trials Centre
QIMR	Medical Research Institute
Genomics Research Centre	Genomic & Clin.Trial Centre
Emphron	Clin. Data Mgt. & Biostat.
Symbiosis	Commercialisation

Please visit:

www.qctn.com.au/members

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QCTN Services

- **Promotion of QCTN Member Organisations**
  - Tradeshows & Conferences, QCTN Missions
  - Networking with "networks"
- **Provision of infrastructure & know-how**
  - Independent HREC= IRB and IBC (Biosafety)
  - SOS (Staff on Site), Education & Networking
- **Facilitation of industry events**
  - Clinical Research Excellence 2008 Conference (CRX-08), Brisbane, 7-9 August, 2008
  - ClubBIO, Gold Coast, 11-12 September, 2008
- **Alliances with other organisations**
  - BARQA
  - ACRP
  - KHIDI

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Australia's "Duty Impliers"

- **Australian Regulatory Authority for regulating Medicines and Medical Devices is called TGA**
  - **T**herapeutics **G**oods **A**dministration
- **Other important regulatory entities are:**
  - **Ethics**: National Health and Medical Research Council (NHMRC)
  - **Genetically Modified Products**: Office of Gene Technology Regulator (OGTR)
  - **Radiation Products**: The Australian Radiation Protection and Nuclear Safety Agency
- **Australian regulations consistent with international guidelines (ICH)**

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Clinical Trials: The Rules

- Good Clinical Practices (GCP)
- Good Laboratory Practices (GLP)
- Good Manufacturing Practices (GMP)
- Good Storage Practices
- Many other rules, guidelines, codes, directives, etc.

Essentials of predicate rules like GCP:

- **Rights, safety and well-being of subjects most important**
- **Scientifically and ethically sound studies**
- **Recording and careful handling of all information**
  - Enable verification
  - Evidence to show one is "in control"

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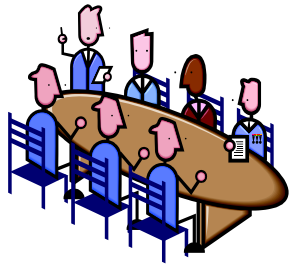
In Control of Clinical Trials: Many Parties, Many Rules

AUS: **TGA**/NHMRC
USA: **FDA**,

EU: **EMEA** + local Regulators



HREC: Human Research & Ethics Committee

Sponsor
(Pharma, Biotech, Medical Device Companies)

Experimental Drug/Device

Principal **Clinical Investigator**

Patient

Pharmacy

Hospital Personnel

Laboratory

CRO's (Contract Research Organisations – Support Services)

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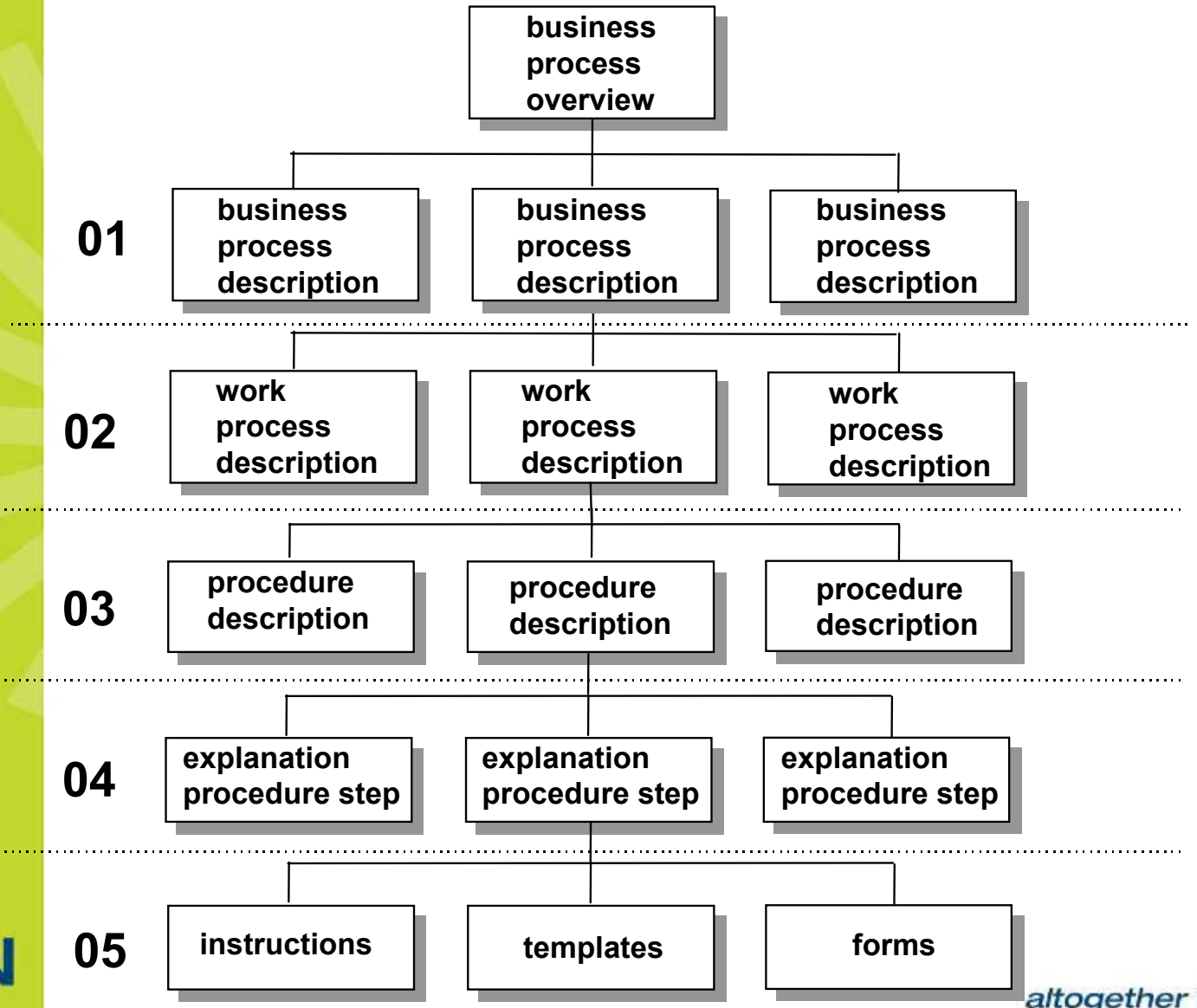
Typical model for documenting processes



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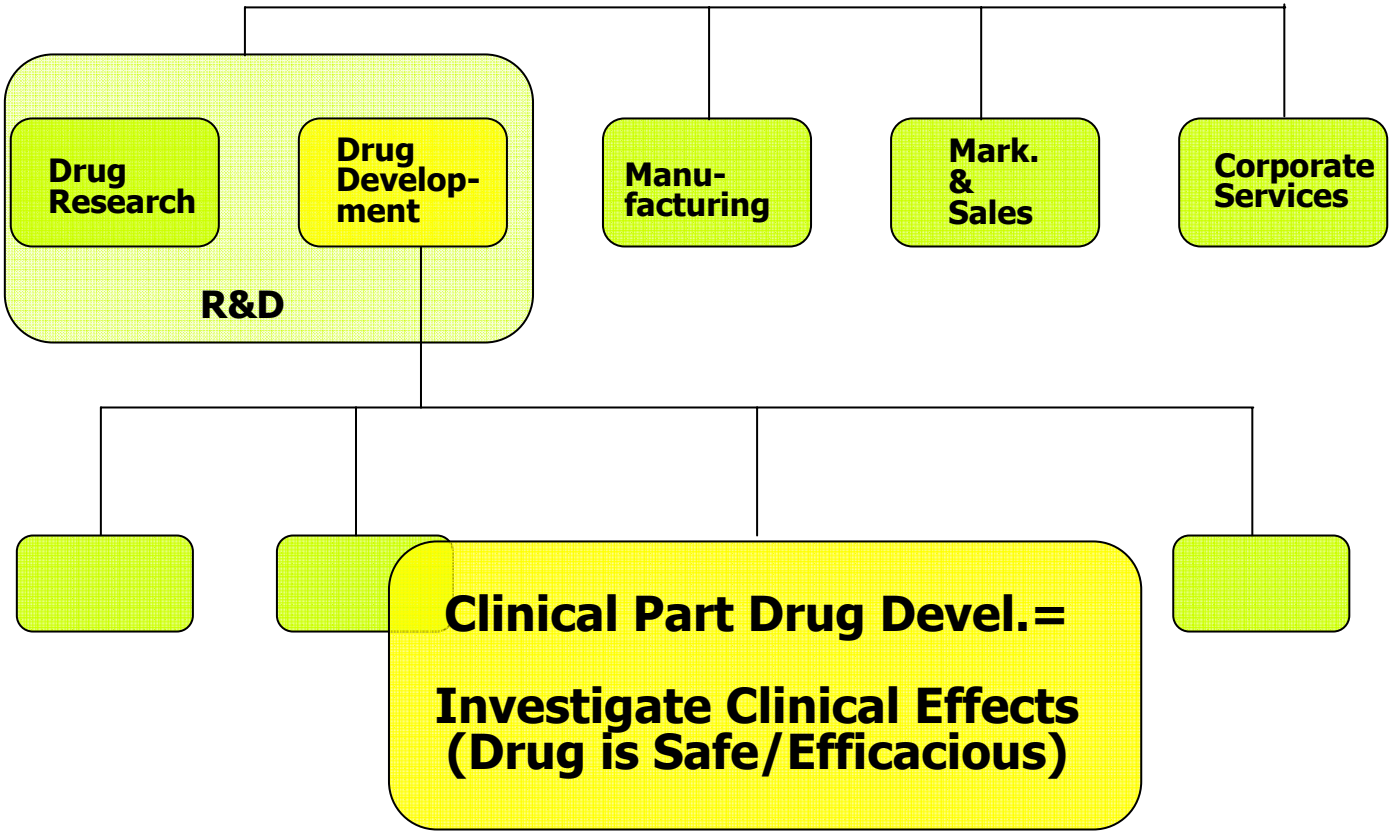
Process Hierarchy in Drug R&D – 1 of 2



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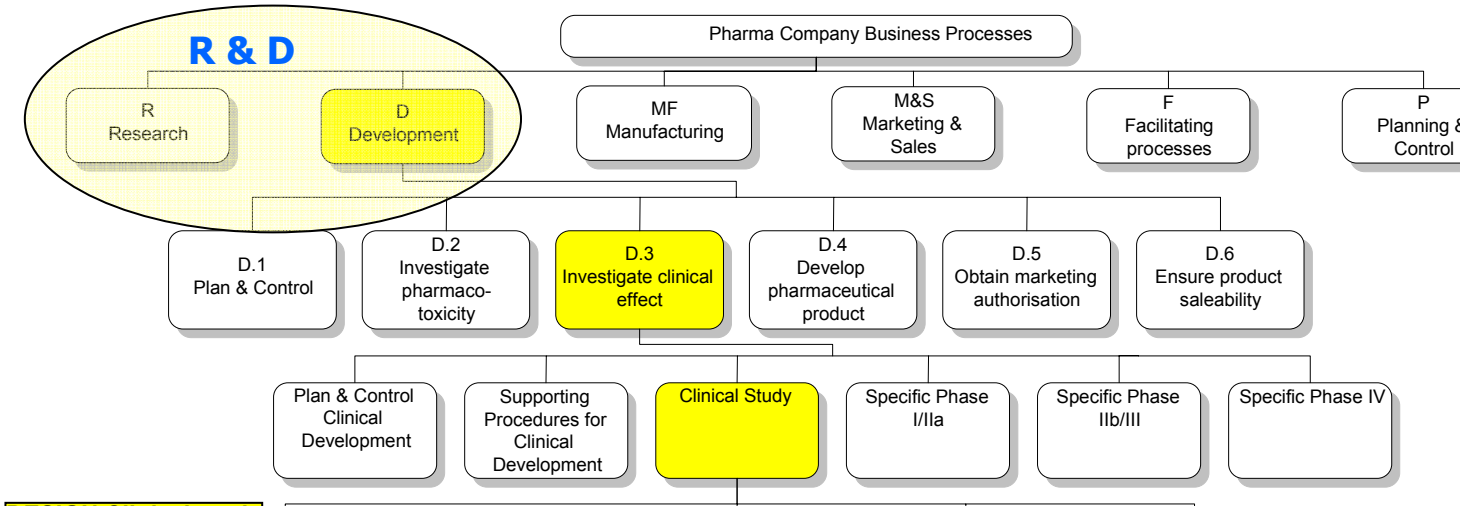
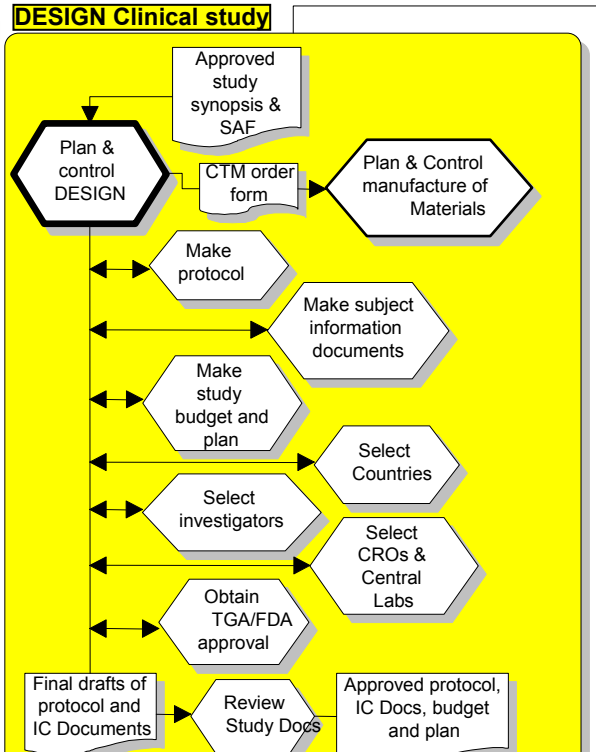
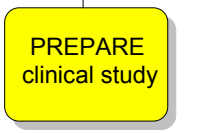
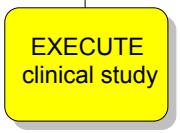
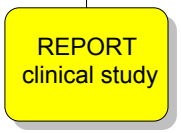
Complete Process Hierarchy in Drug R&D – 2 of 2











Summary

- Drug Research & Development is big business
- Many parts of Drug R&D can be outsourced to specialised service providers
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- Queensland has many R&D service providers, many of whom are members of QCTN
- Clinical Trials are complex, cross-organisation processes which need to meet strict regulatory guidelines

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Challenges for the Clinical Trials Industry in Queensland

- **Craft skills of clinicians not in doubt – good doctors**
- **Current environment conducive to clinicians undertaking clinical trials but good doctor ≠ good researcher**
- **Conducting clinical trials requires**
  - Compliance with regulations – most notably GCP
  - Training in research methodology: study design & analysis
  - Biostatistics support (a trial represents a sample of patients)
- **Failure to have quality systems and qualified researchers in place poses a risk to patients AND to the industry of life sciences R&D service providers**

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Overall Summary

- Drug Research & Development is big business
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- Poor quality impacts value of data
- Queensland has many R&D service providers, many of whom are member of QCTN
- Clinical Trials are complex, cross-organisation processes which need to meet strict regulatory guidelines
- To be commercially viable, one needs to have appropriate quality systems in place whereas non-commercial or inexperienced organisations may not have them in place ⇔ challenge
- A common quality standard for life sciences R&D service providers in Qld needs to be created

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Fairy Tale about Quality

- Three Little Pigs

- Straw
- Stick
- Brick

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