



Trends in Clinical Trials and Strategies for the Future

Lynda Shelley
Executive Director, Clinical Operations
Novotech (Australia) Pty Ltd

Sponsored by Industry and Investment NSW
Ausbiotech, October 2009



Intro and background

Drug developers face

- Continued downward pressure on pricing / reimbursement
- Increasingly stringent safety requirements (greater patient numbers, more trials, longer follow up)
- High level of scrutiny on development costs from shareholders / boards to protect their investment and ensure ROI
- Regulators challenged to keep pace with rapidly developing healthcare technologies

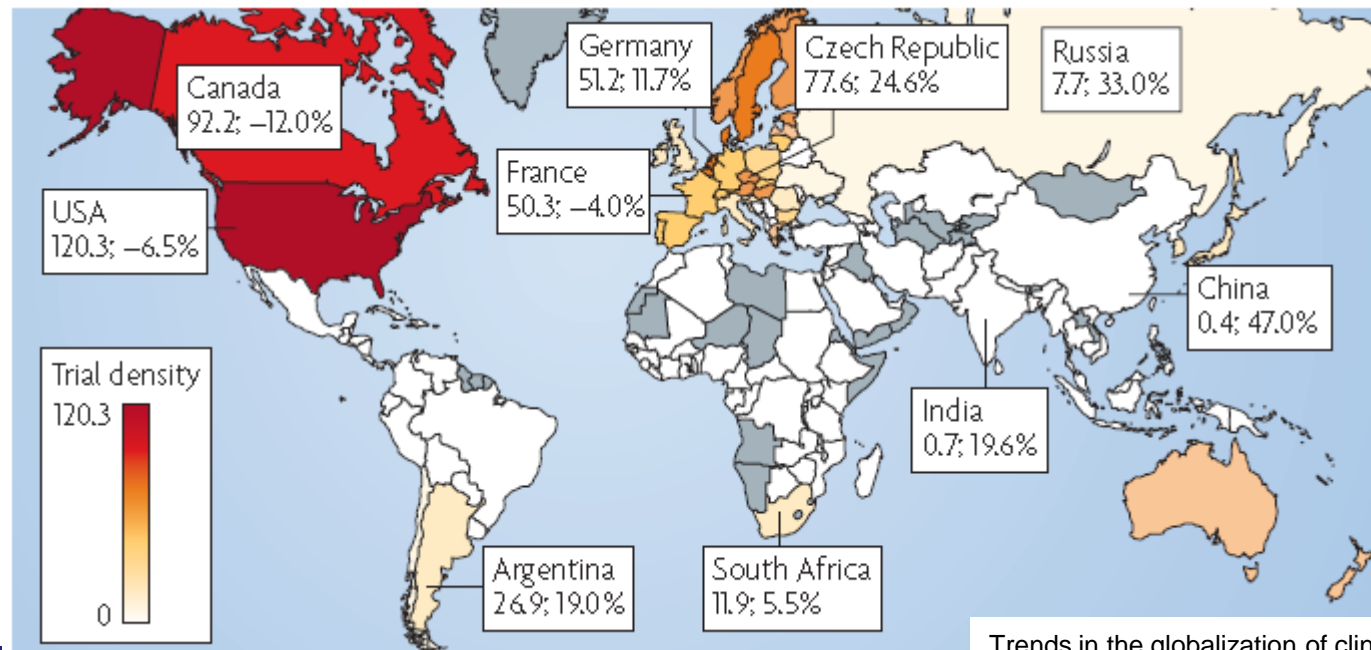
Need to employ innovative strategies to maximise efficiencies and ensure successful outcomes

- Operational efficiencies in clinical trial conduct
 - generally decreased timelines
 - efficient and effective trial design
 - Seamless phase transition

- Use of innovative technologies in clinical trials
 - Project management
 - Communication
 - Data collection

Innovation through operational efficiencies

- Internationalisation of clinical trials
- Australia still very attractive source of patients and KoLs
 - integrated cancer research institutes
 - mutual IEC recognition
 - ClinicalTrials_{NSW}
 - SMOs, professional trial sites
- Asian region
 - enormous untapped potential for faster patient recruitment
 - population + quality + price

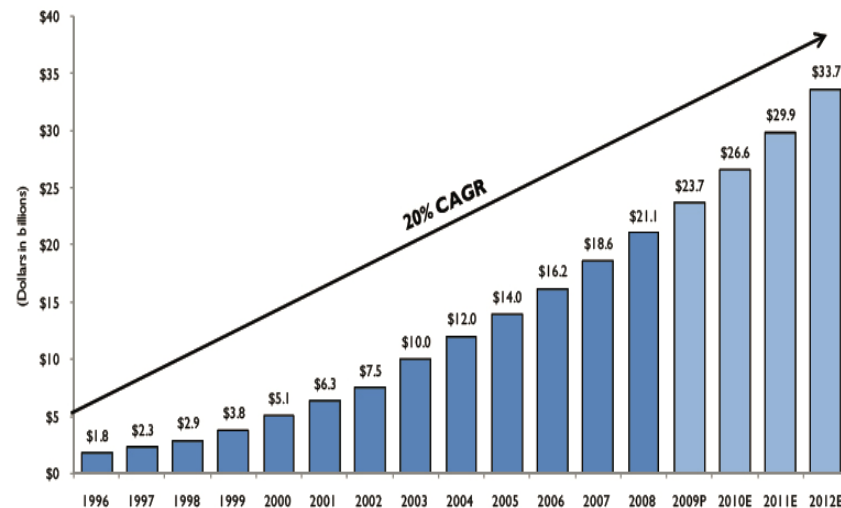


Innovation through operational efficiencies

Time and cost efficiency of outsourcing of Clinical Trial conduct decreases financial risk to Sponsor

Buy vs build approach gives access to

- Operational efficiencies of a mature service industry
- Established & accredited infrastructure and processes
- Expanded geographical reach
- Flexibility and speed of start up
- Necessary expertise as and when needed



Source: Estimates from company data, IMS Health, PhRMA, Ernst & Young, Goldman Sachs Research, and Jefferies & Company Research.

Human micro-dosing or phase 0

- FDA: “Exploratory IND”
- Radio-labelling / AMS / PET
- Early human data combined with pre-clin, *in-vitro* and *in-silico data*

Greater success rate to identify promising or high probability leads and discard others early

Adaptive trial design

- Use of available and emerging biomarkers to predict patient response early (efficacy/safety)
- Ongoing feedback gives greater opportunity to get the trial design right
 - Advantage over “one shot” approach
- Can facilitate seamless phase II/III transitions

Innovative techniques to raise awareness

- Clinical trial registries
- Use of PR to raise awareness, general community health segments
- National “disease” weeks, patient advocacy groups
- National conferences, KoL interviews
- Can have a positive flow on to patient recruitment



Techniques and campaigns to enhance recruitment

- Direct to patient advertising through all traditional media, internet, social networking sites, twitter?
- Call centres to pre-screen and direct patients to appropriate sites



DO YOU SUFFER FROM HAYFEVER?

WE ARE LOOKING FOR VOLUNTEERS TO TAKE PART IN A CLINICAL STUDY

The study will investigate a new treatment for hayfever
You may be eligible to take part in this study if you are:

- Aged 15 to 60 years
- Suffering from hayfever
- Otherwise healthy

The study will be performed over the course of 7 weeks and will require 4 visits to the Research Centres.

For more information please contact 1800 828 717

All information will be kept strictly confidential
You will be compensated for your participation in the trial.
This study has been approved by the Bellberry Human Research Ethics Committee

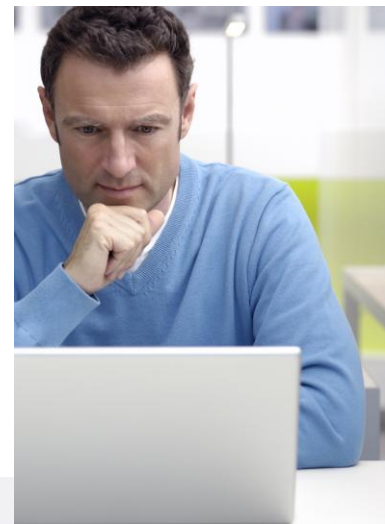
Web enabled technology to speed data capture and better control patient oversight and study conduct

■ Communication / multimedia

- Webex training for Investigators and study teams
- Use of professional AV techniques for animated mode of action / training video
- CTMS – project management tools, shared access to study status information and reporting (CRO / client / DSMB / Investigators)

■ Electronic data capture – e-CRF web enabled for real time access

- OCR and multi-lingual systems
- Interactive Voice/Web Response Systems



- Patient devices / Ambulatory monitoring
 - QoL, symptom monitoring
 - Reminders, dosing aids and records
 - Instant feedback to patient / Investigator
 - Programmable, tailored to study needs
 - Linked directly to Investigator & clinical database
 - Audit trail



Combinations of drugs with:

- Drug;
- Device;
- Diagnostic (personalised medicine);
- Carrier;
- Adjunctive therapy;
- Behavioural intervention
- Food etc

Exponential growth over the last 20-30 years

- FDA office of combination products est. 2001
- Fomalisation of GMPs for combination products ongoing
- Challenges for regulators, researchers and ethics committees to keep pace with the increasingly innovative technologies
 - Not uncommon to see combinations of unregistered diagnostic devices, drug delivery devices and drug in a single study

Drug:carrier combinations

■ EnGeneIc – EDV technology

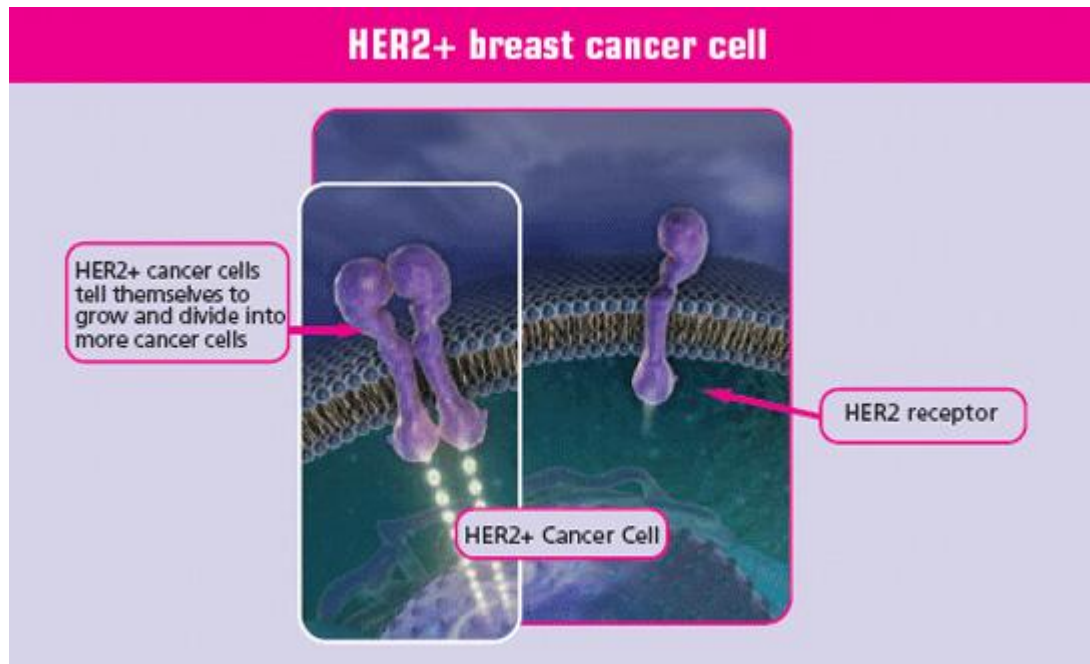
New Targeted Delivery System
for Cancer Fighting Drugs
BREAKTHROUGH

EnGeneIc's landmark publication, "Sequential treatment of drug-resistant tumors with targeted minicells containing siRNA or cytotoxic drugs", in July's issue of Nature Biotechnology journal.

[read more](#)



Drug:Diagnostic combinations



- Targetted drug delivery based on patient/disease factors
- Relatively early days for this technology
- Many believe it's the way of the future for specialist disease management

- Efficient use of available technologies to enhance to drug development process
 - Avoiding technology for technology sake
 - Appropriate and enabling

- Lack of regulatory precedents for convergent technologies
 - may be trail-blazing
 - Need for close regulatory consultation during the development
 - Differences in interpretation between EMEA and FDA

- Training and certification program for health care professionals & patients forms part of the total combination product “package”
 - Opportunities to interact with prescribers / end users
 - Role of regulation?

- Engagement of drug and device companies with varying regulatory experiences and differing opinions development pathways
 - Both “sides” have valuable learnings to bring to the table