

Cell Therapy Commercialisation

Look, think and act from a different point of view

AusBiotech 2010

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What sets Cell Therapies apart?

In the absence of absolute product characterisation.....

Successful clinical trials demonstrate that:

- ✓ **Focusing on consistent replication of process**
- ✓ **Combined with a well developed Quality Assurance regime**
- **Delivers acceptably consistent and quantifiable patient outcomes**

“The product is the process”

Relative to pharmaceutical models,
patient specific cell therapies require
commercialisation of a process rather than a product.

Commercialisation objectives

Deliver a highly effective and safe therapy

To as many patients that can benefit from it

Make a profit to cover the cost of development, (and those developments that did not succeed)

Maximise the positive cashflow to motivate the development of the next beneficial therapy

- **Critically assessed and approved by regulators**
- **Quality of delivery must be without compromise.**
- **Reach as many patients as possible**
 - Multiple sites
 - Multiple regulatory jurisdictions
- **Affordable cost with an attractive margin**
- **Increase sales as quickly as possible once the therapy is approved**

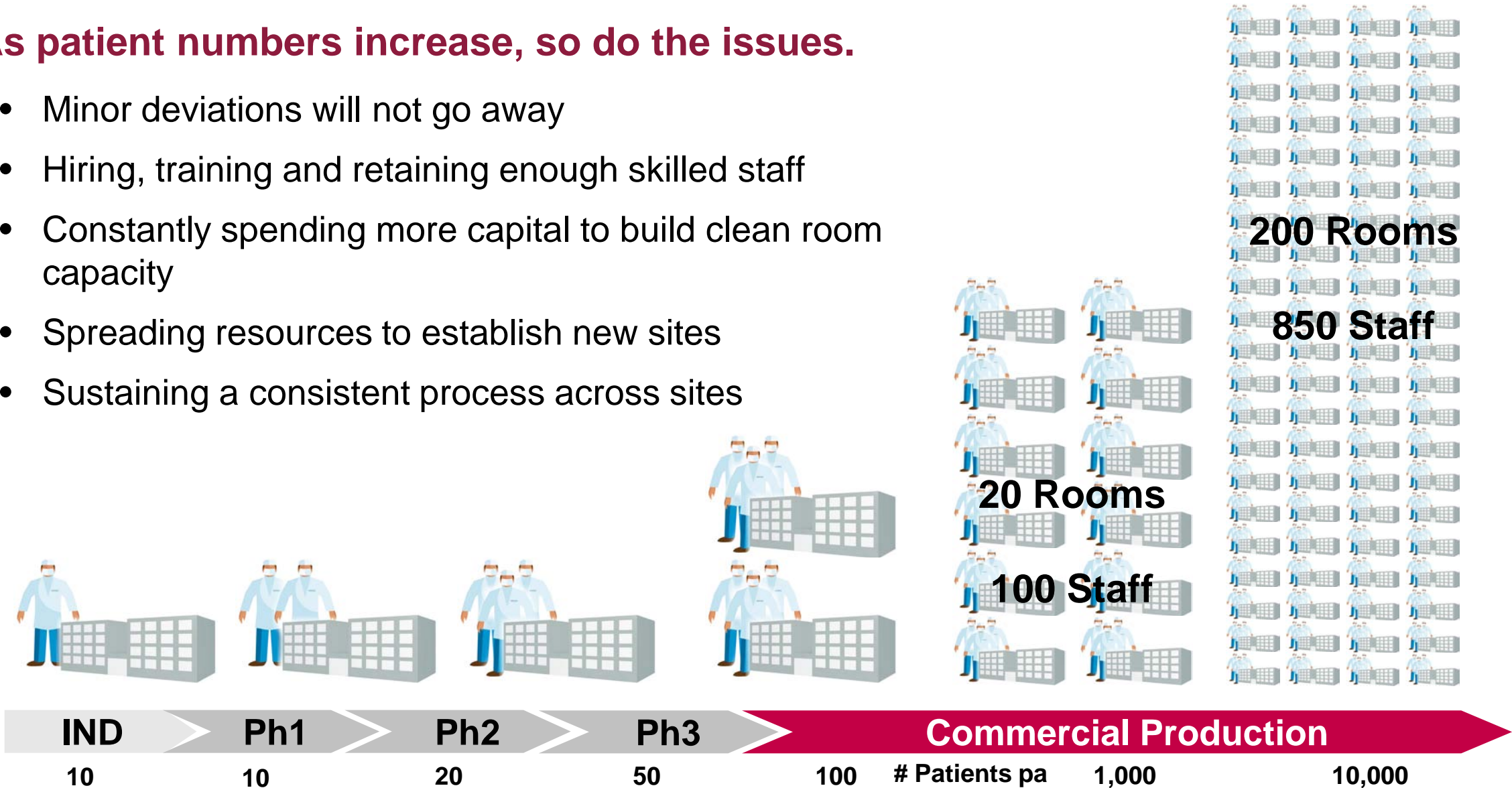
Processes developed in the laboratory are not readily scaled

- Processes are manual, labour intensive and require a high degree of skill
- Equipment was not developed or optimised to suit the process



As patient numbers increase, so do the issues.

- Minor deviations will not go away
- Hiring, training and retaining enough skilled staff
- Constantly spending more capital to build clean room capacity
- Spreading resources to establish new sites
- Sustaining a consistent process across sites



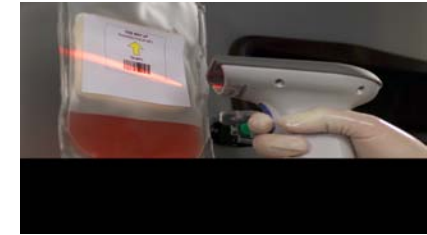
There is another way - Functionally closed automation



- Pre-sterilised single use kit specific for your process
- Patient derived materials
- Reagents & buffers

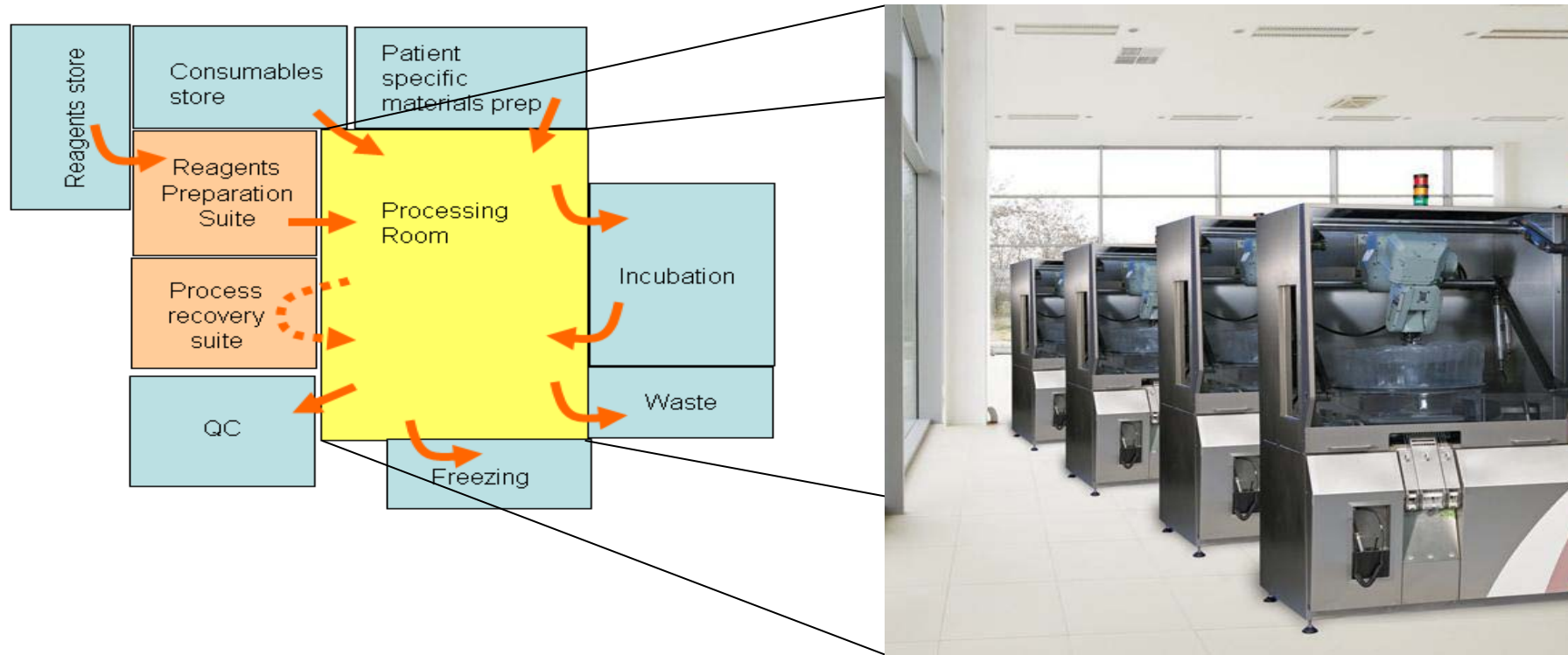


- Automated processing platform in class C area



- Patient specific product output to incubation, freezing etc.
- Batch record

Volume processing looks different.



In facilities configuration.....

and personnel

Benefits of automation - Quality

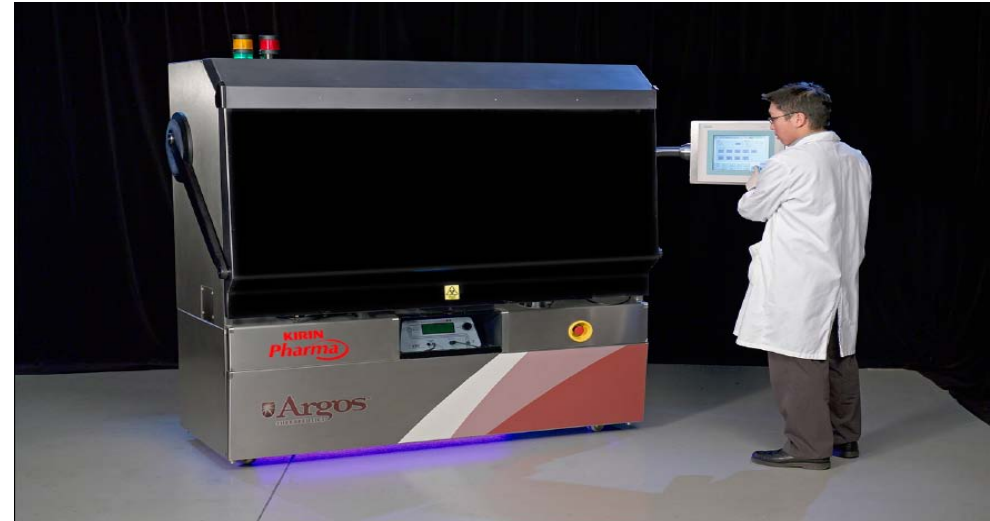
Manual & Open

- High skill level required
- Human errors create quality events
- Regulatory approval hurdles

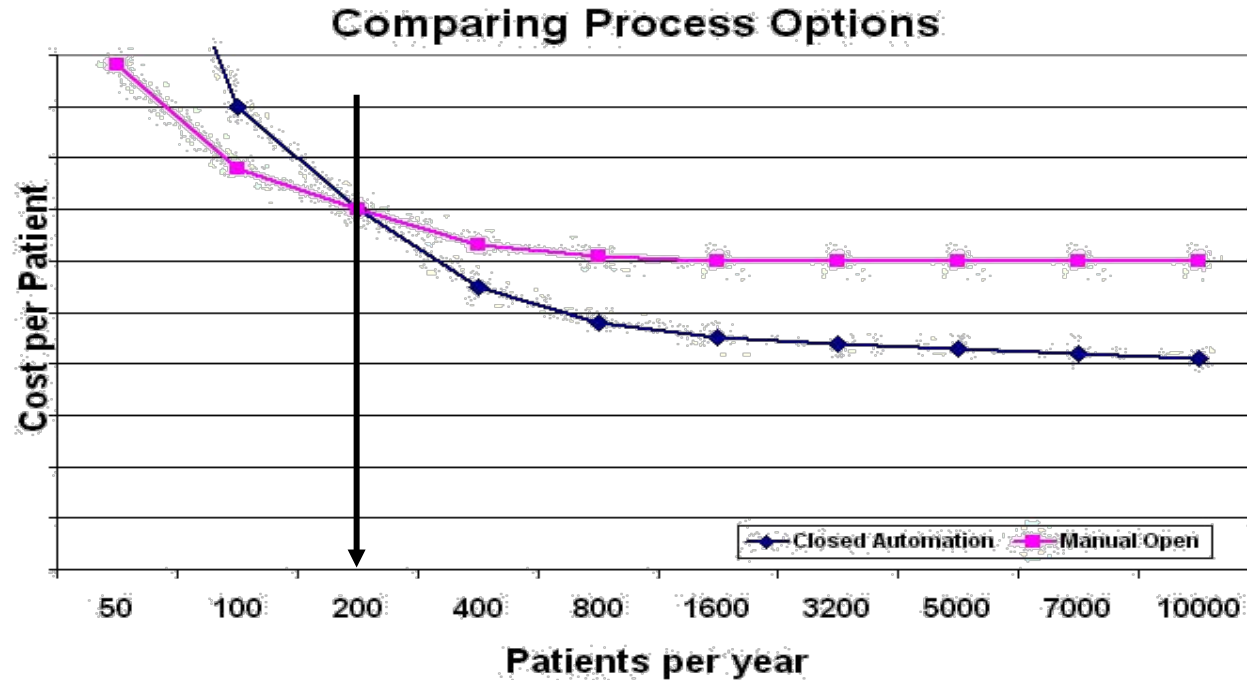
vs.

Automated & Closed

- Reducing the opportunity for human error.
- Significant reduction in # of quality events
- Satisfy TGA, FDA & EMA requirements.



Benefits of automation - Cost



Whilst no two therapies are the same, we are typically seeing:

- **30% - 50%** reduction in capital cost
- **>50%** reduction in the cost of labour
- **25% - 90%** reduction in cost per patient
- Significant reduction in the # of quality events

Benefits of automation – Time to market

Scale Up

Match demand by replication of processing equipment

Scale Out

Duplicate therapy in new geographic locations.
Introduce therapy into new regulatory jurisdictions.

Expansion strategies

Duplicate your own GMP facilities

OR

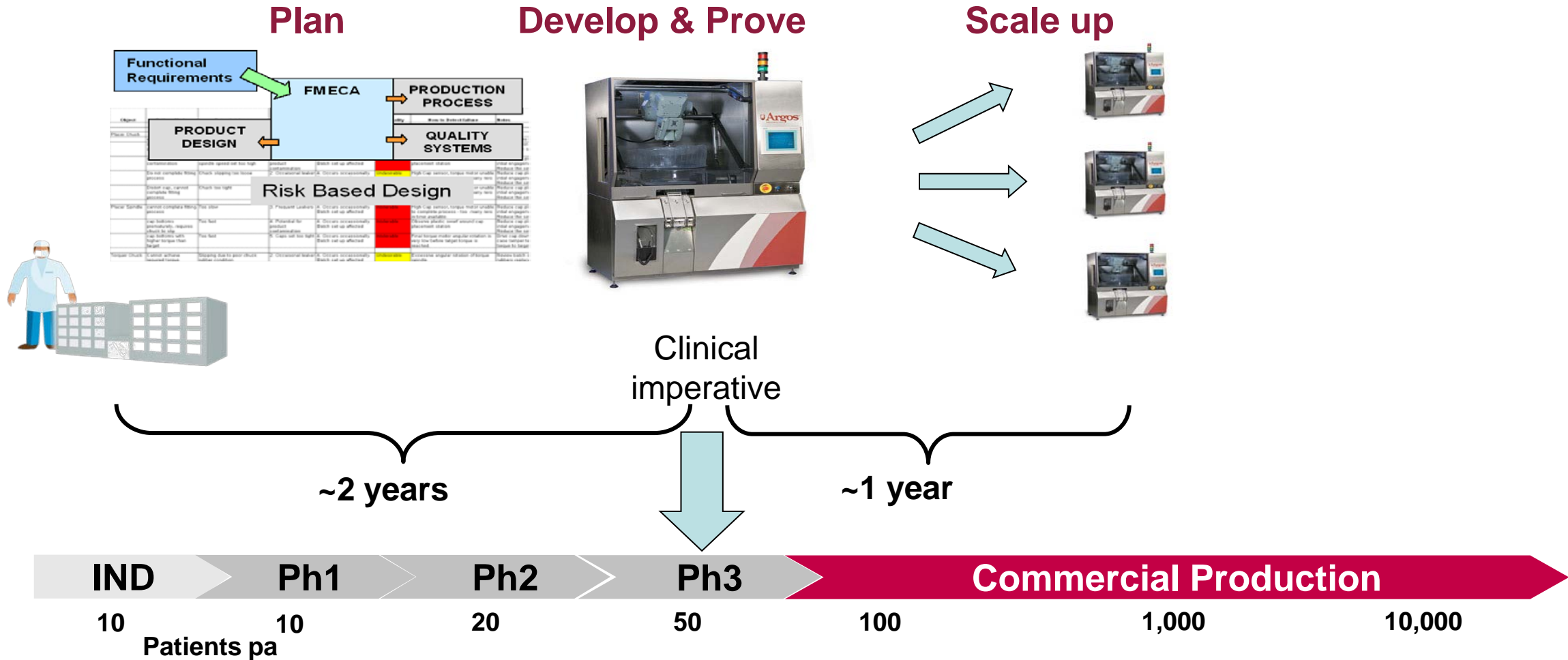
Locate the equipment in a GMP facility eg CMO

Centralised monitoring of quality, material supply & equipment

These strategies can realise a franchise model

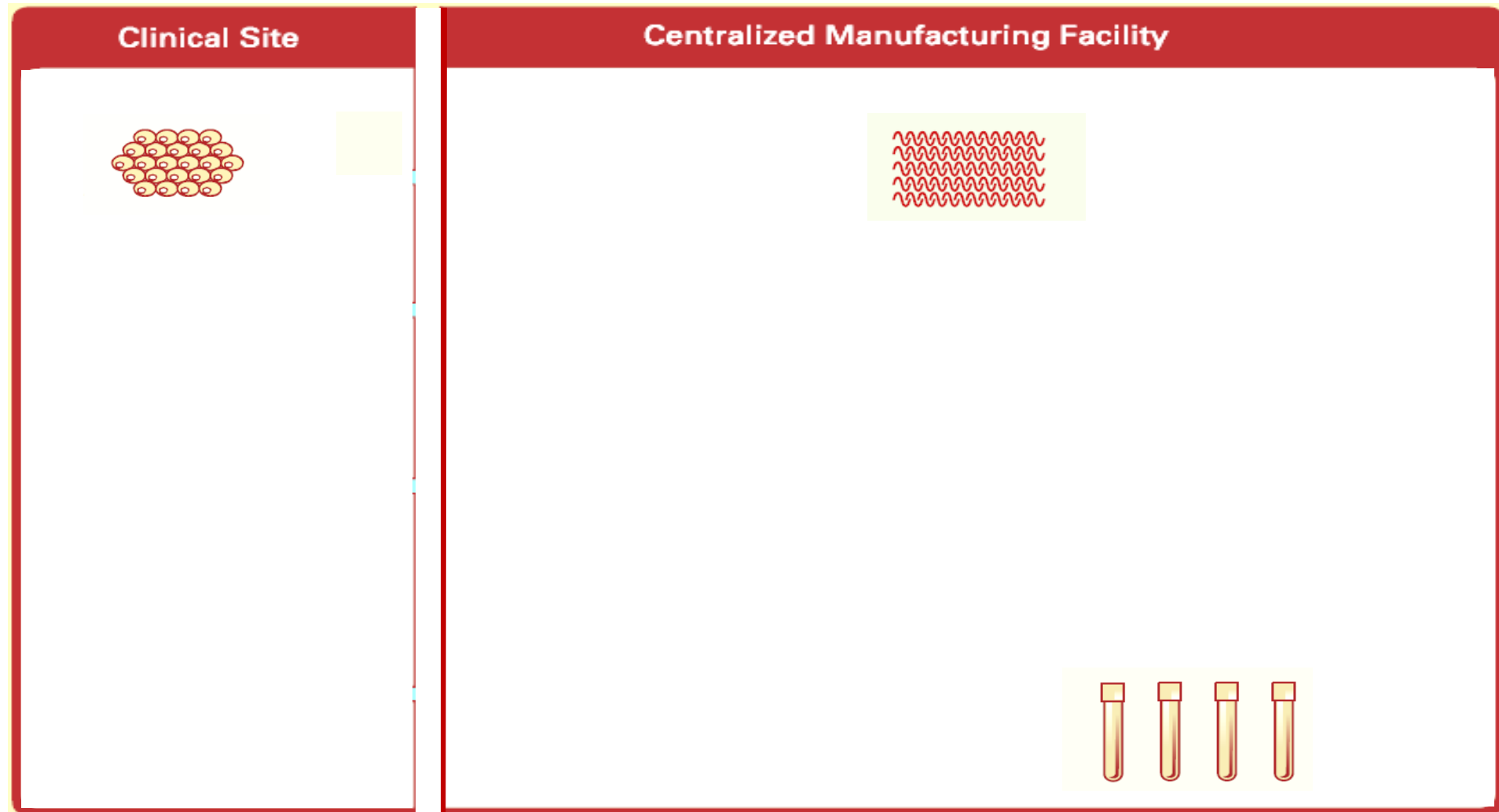


When is the right time to consider automation?



Start as early as possible to align clinical process to manufacture

Argos Therapeutics – A Closed and Automated Process



The automation of cell therapies;

- **Delivers robust quality**
- **Enables scale up**
- **Reduces cost**

and

Enables new models for commercialisation