

PATIENT ADVOCACY IN CLINICAL TRIALS

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PREMISE

Patients should be front and centre of clinical trial design, development and outcomes

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WHY?

- ▶ Earlier access to potential new treatments / combinations
 - paediatric/adult paradox
- ▶ Gives patients another option to 'standard of care' (considered as 'unacceptable' by many)

WHY?

- ▶ Reduces the risk for pharma companies / encourages participation
 - ▶ Compassionate use following trial provides ongoing access for patients doing well at minimal cost
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WHY?

- ▶ Patient advocacy in clinical trial development improves the trial experience for patients and may result in better trial delivery through increased participation and reduced drop-out

HOW CAN THIS HAPPEN?

- ▶ Forming coalitions with researchers, clinicians and other patient advocates
 - ▶ Getting other stakeholders to recognise the power of patient advocacy (payors, pharmas, government, regulators)
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HOW CAN THIS HAPPEN?

- ▶ Activism versus advocacy
- ▶ Focus on outcomes

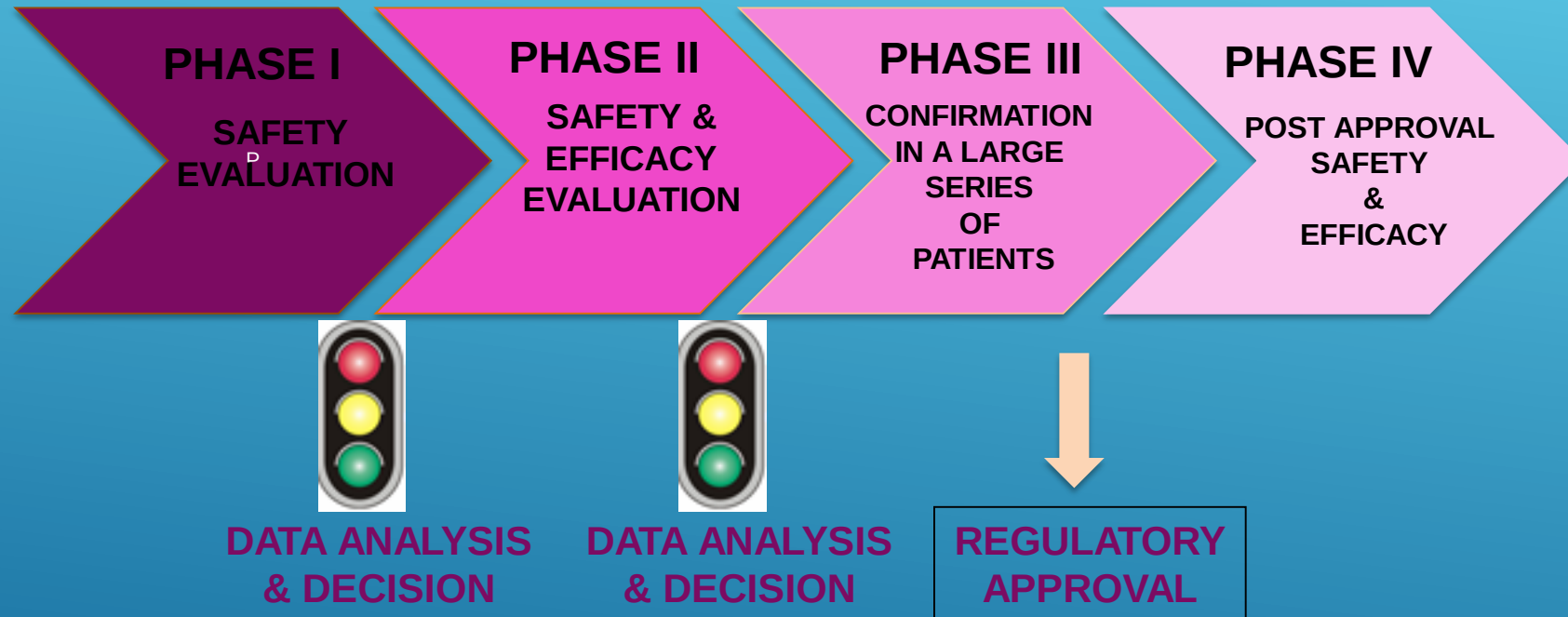


A CURRENT EXAMPLE

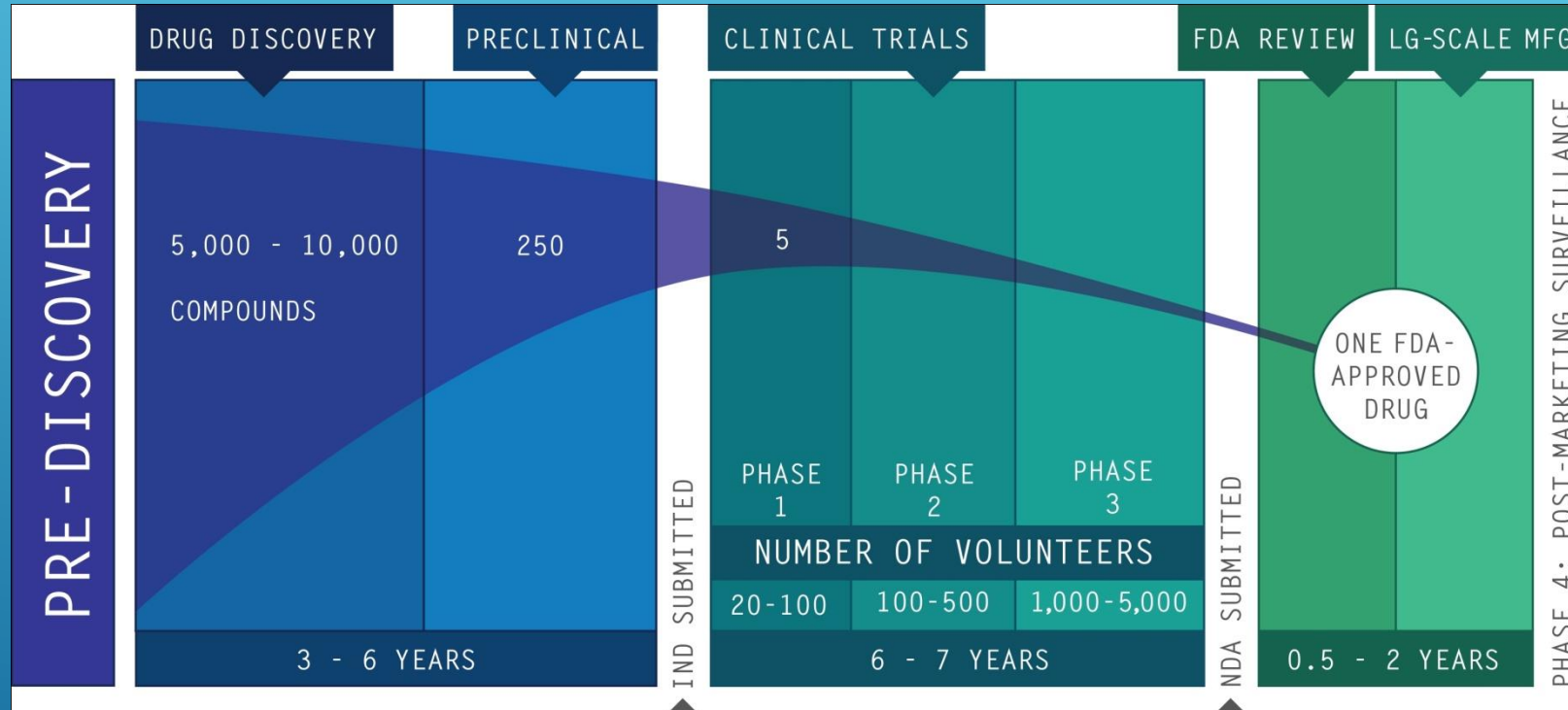
Global Adaptive Learning System (US, Australia, China)

- ▶ Genesis
 - ▶ Opportunities & barriers
 - ▶ Outcomes for patients
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TRADITIONAL CLINICAL DEVELOPMENT PATH



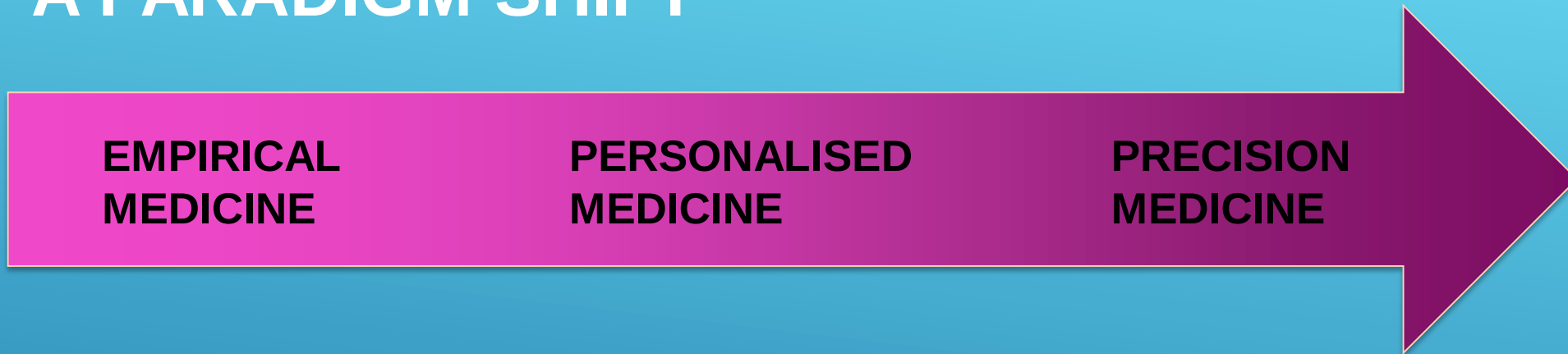
DRUG DEVELOPMENT PIPELINE: MASSIVE ATTRITION, LONG DURATION, HIGH COSTS (NOT SUSTAINABLE)



~ 60% of Oncology Trials Fail in Phase III

~ US\$ 2.0 Billion

A PARADIGM SHIFT



- Blockbuster drugs targeted at broad population segments
- On average, 50% of patients do not have desired therapeutic outcomes
- Significant adverse events

- Drugs targeted at subgroups of patient population
- Genomic profiles determine segmentation and therapy
- Best possible therapeutic outcome with minimal adverse events

- Delivering the right medicine
- To the right patient
- At the right dose
- At the right time

TRADITIONAL VERSUS ADAPTIVE TRIALS COMPARISON

Traditional Clinical Trial	Global Adaptive Learning System
• Tests each intervention using defined study protocols held constant during trial. • Outcomes analyzed at end of trial- years after trial began. Reasons for failure often unknown. **Design amendments can cost \$500,000 in direct costs and 60 days to implement	• Continuous learning system. Outcomes are analyzed throughout the trial to inform next steps. • Flexibility in design helps address uncertainty about original choices for trial parameters.
• Drugs are not deemed ineffective until the conclusion of the study is reached.	• Ineffective drugs can be dropped quickly ** Early study terminations could save \$100-200million
• One new drug is usually tested per trial. • Phase II testing for one drug costs ~\$29 million	• Multiple new drugs tested using only one standard arm for comparison over the course of the trial • In I-SPY2, Phase II testing for multiple drugs cost \$43 million over 6 years
• The success of the study depends on the accuracy of the original assumptions. • Small molecule drugs have a 38%, and large molecules have a 53%, success rate from Phase II to Phase III	• Drugs graduate to Phase III when they meet a threshold of 85% for predicted likelihood of success

YOUR TURN!

- ▶ *Your brief is to challenge, add, subtract, change, improve. Discuss how this works or could work within your country or region.*
 - ▶ *What you would need to make it happen?*
 - ▶ *How could other delegates help?*
 - ▶ *Is there training from which you might benefit.*
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YOUR TURN!

- ▶ *You have 15 minutes in your group*
 - ▶ *One person needs to present back major points to the group (2 minutes)*
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