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pharmacometrics africa

PharmaTrain
MASTERING MEDICINES DEVELOPMENT
CENTRE OF EXCELLENCE

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WORKSHOP

NOVEL CLINICAL TRIAL METHODOLOGIES 2019

25 February 2019

Prof Bernd Rosenkranz, MD PhD (Germany), FFPM

Fakulteit Geneeskunde en Gesondheidswetenskappe
•
Faculty of Medicine and Health Sciences

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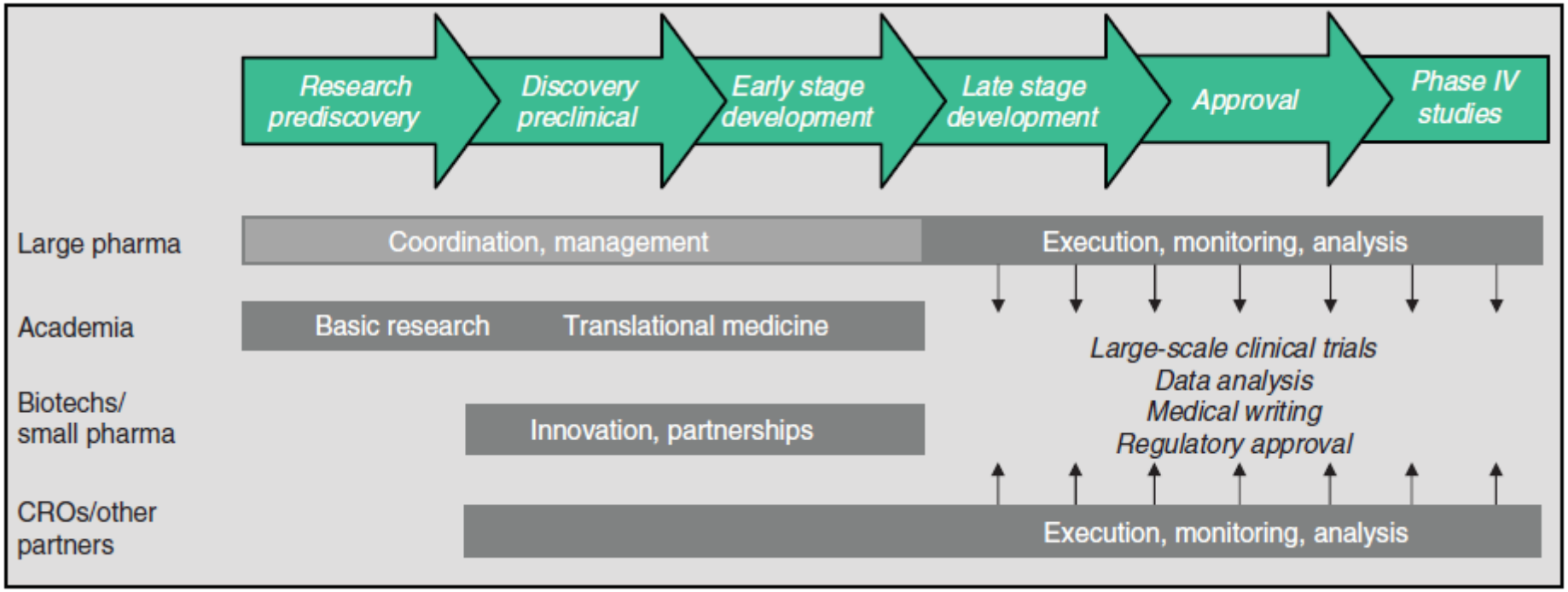
Drug Discovery & Development – Value Chain



Cost: 1 – 2 billion US-$

Kaitin CPT 87: 356, 2010

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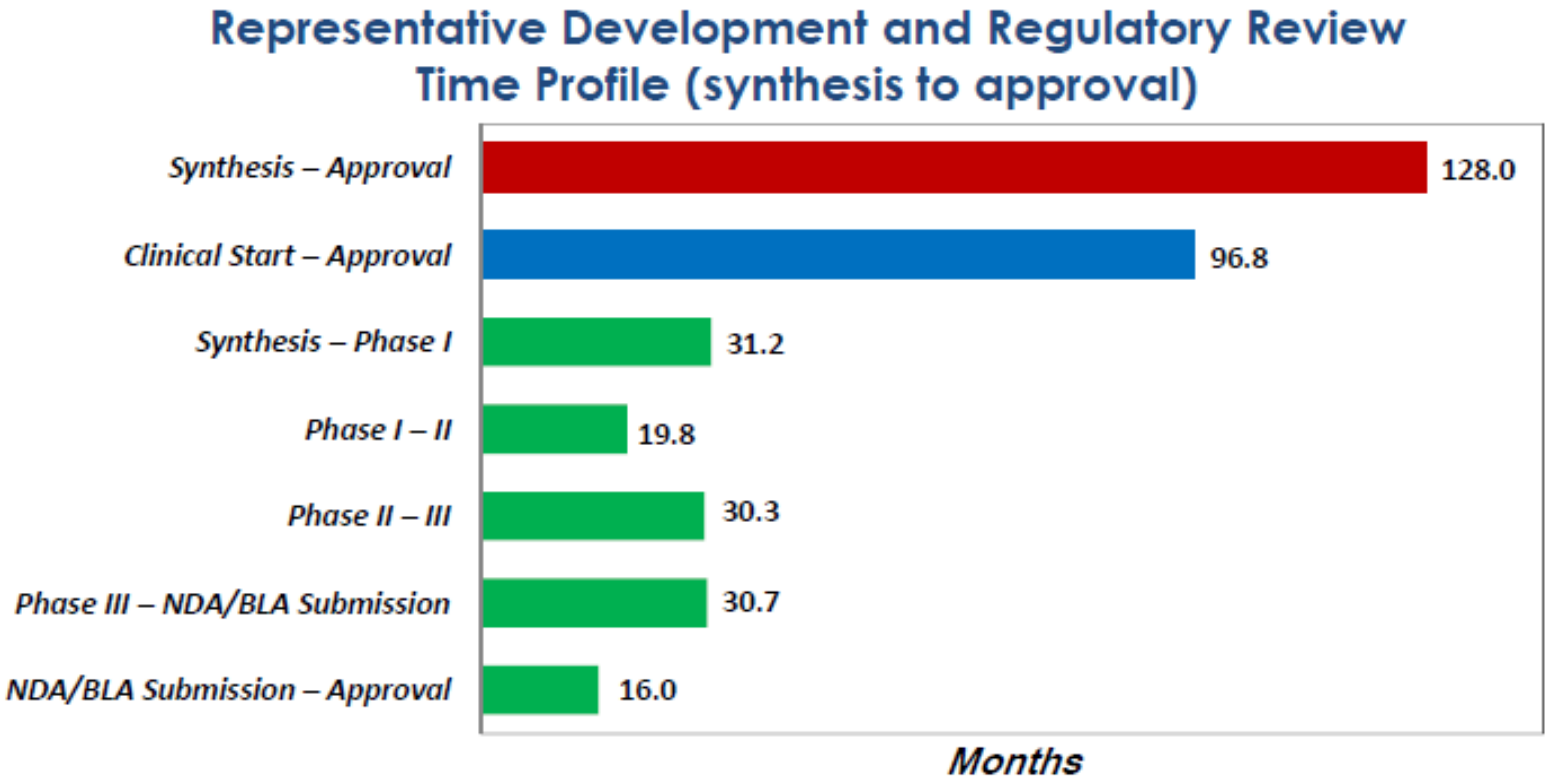
Timelines from Synthesis to Approval



Di Masi 2014: http://csdd.tufts.edu/files/uploads/Tufts_CSDD_briefing_on_RD_cost_study_-_Nov_18,_2014..pdf

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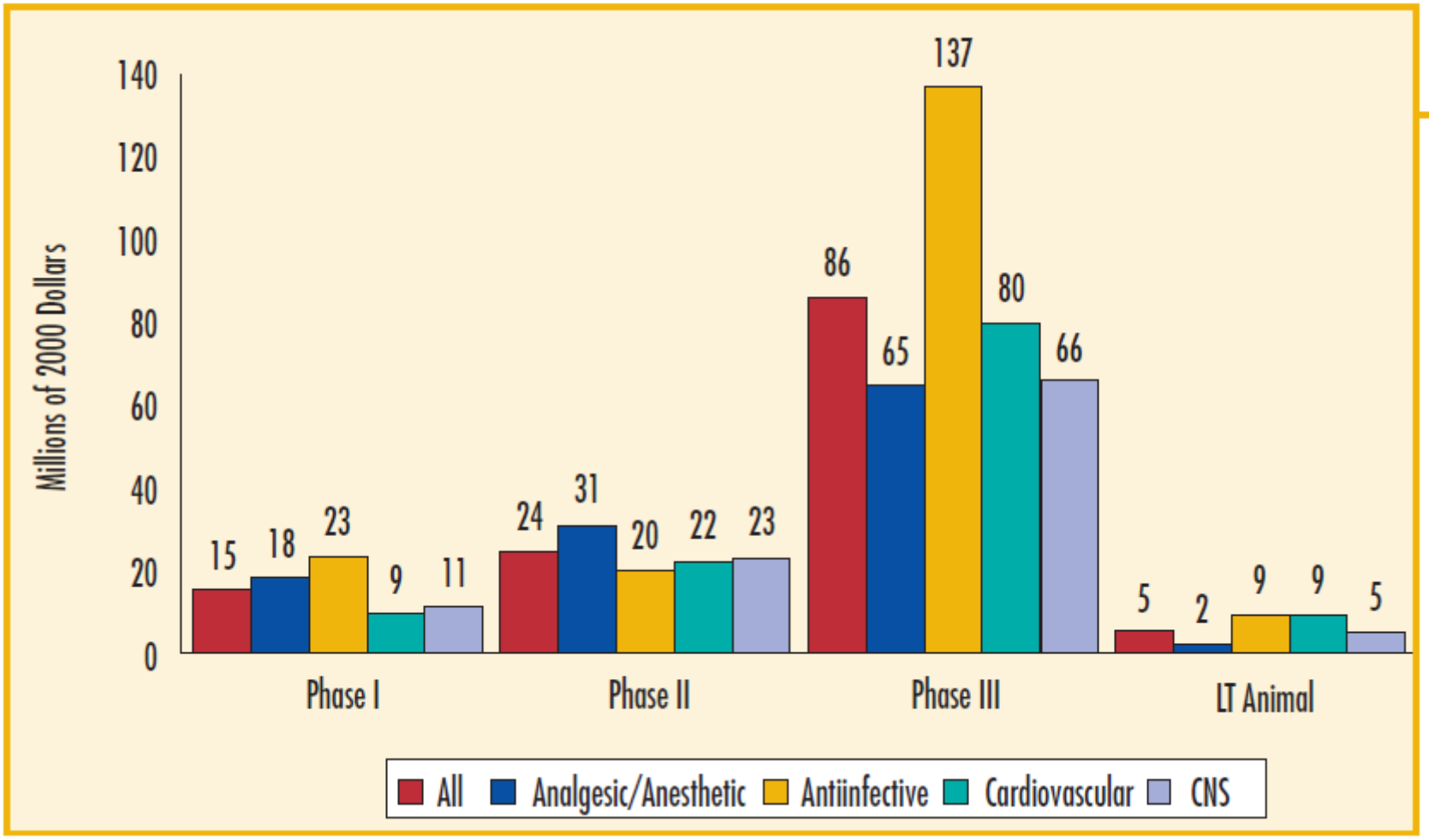
Cost of Clinical Development per Phase



Mean phase cost by therapeutic class for drugs entering a phase
LT animal = Long-term animal testing concurrent with clinical trials

JA DiMasi, HG Grabowski, J Vernon, Drug Information Journal 38: 211-223 (2004)

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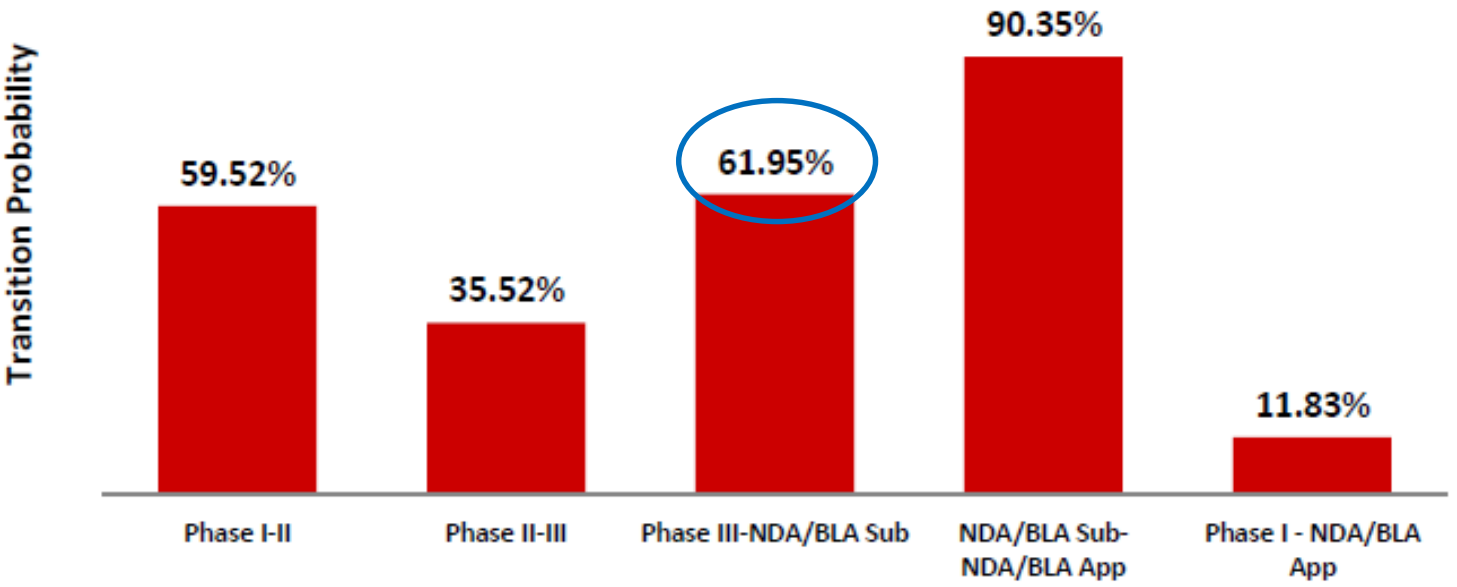
Success Rates During Clinical Development

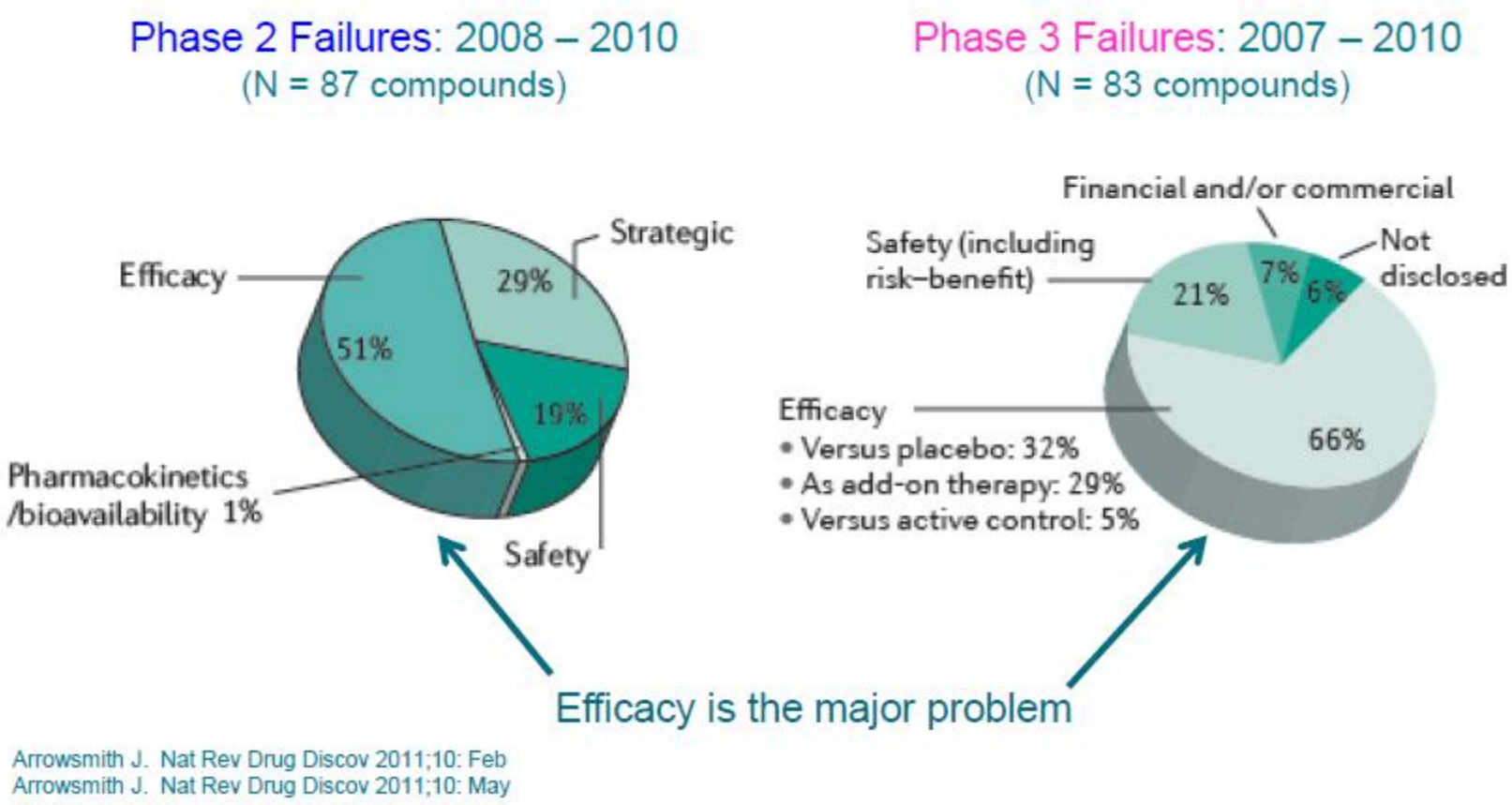
Clinical Phase Transition Probabilities and Overall Clinical Approval Success Rate*



*Therapeutic new molecular entities and new therapeutically significant biologic entities first tested in humans, 1995-2007

Di Masi 2014: http://csdd.tufts.edu/files/uploads/Tufts_CSDD_briefing_on_RD_cost_study_-_Nov_18,_2014..pdf

Causes of Failure in Phases 2 and 3



Arrowsmith J. Nat Rev Drug Discov 2011;10: Feb
Arrowsmith J. Nat Rev Drug Discov 2011;10: May

PA Millihan (Pfizer), presentation

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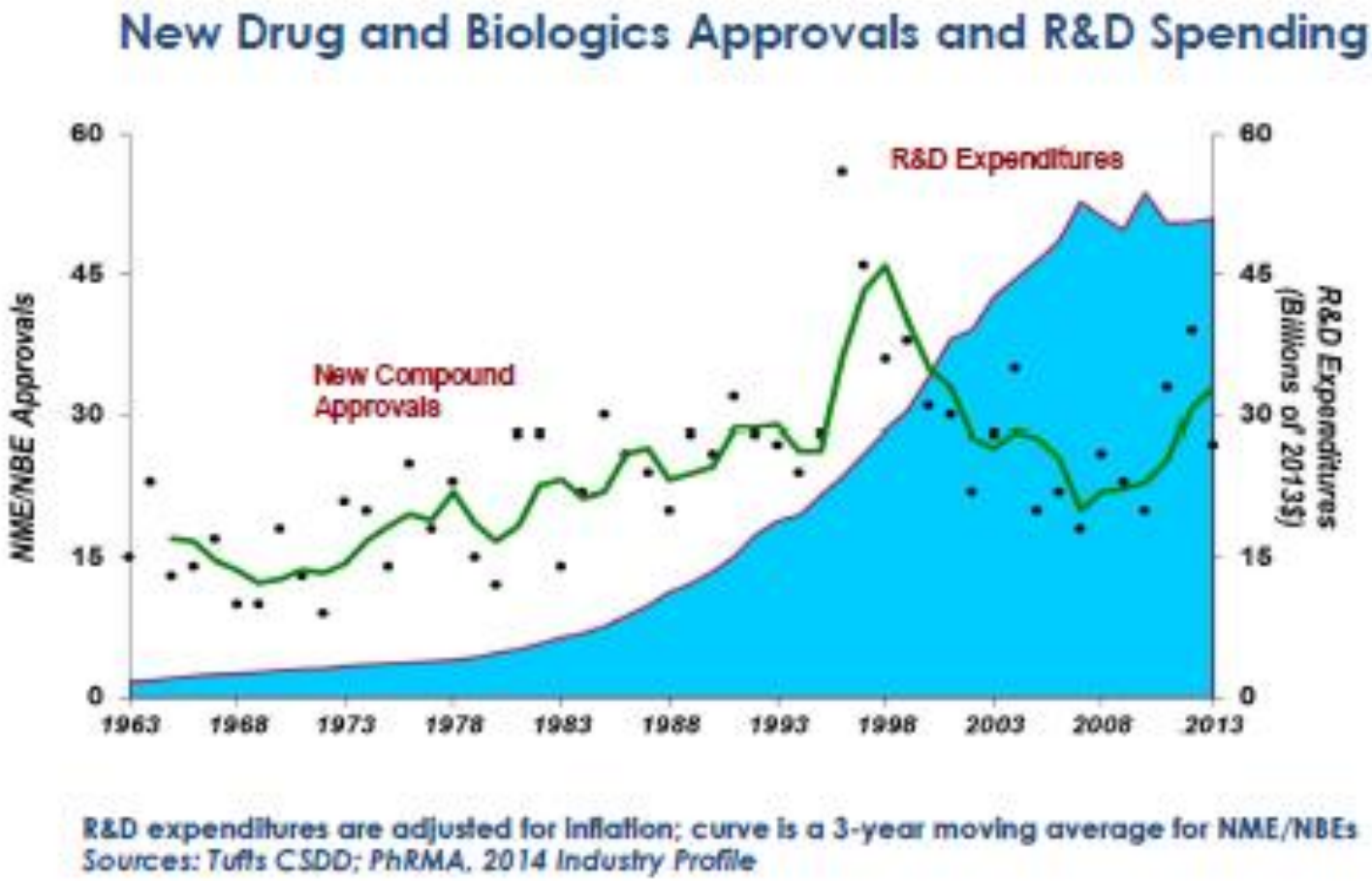
NDAs and Pharmaceutical R&D Expenditures



Di Masi 2014: http://csdd.tufts.edu/files/uploads/Tufts_CSDD_briefing_on_RD_cost_study_-_Nov_18,_2014..pdf

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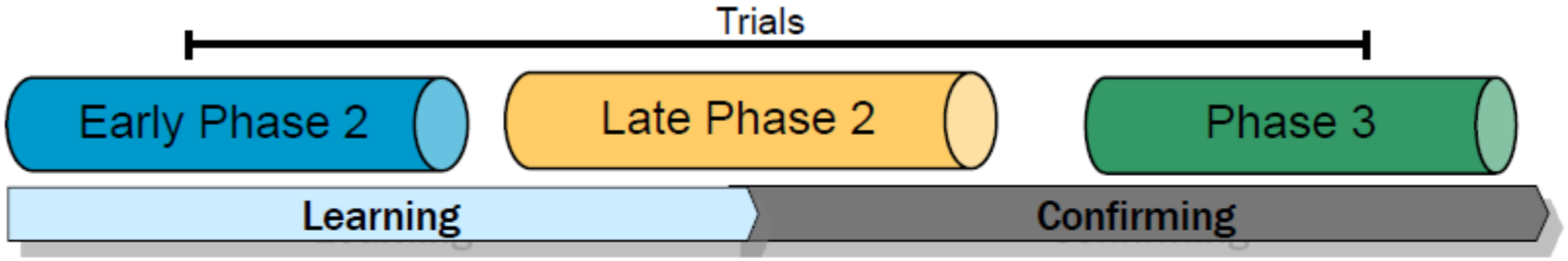
Learn – Confirm Paradigm



- Lean studies for proof of viability
- Fail with low cost
- Learn from failures

fail early

- Confirm activity
- Characterize dose-response
- Understand PK/PD
- Understand totality of data
- Planned futility analyses for inactive doses
- Validate endpoints

- Confirm for registration
- Simple & focused
- Streamlined
- Futility analyses mitigate risk

fail rarely

Lew Sheiner (1940-2004); adapted from PA Millihan (Pfizer)

Pharmacoeconomics

"Wherever health care is provided and used, it is essential to know which interventions work, which do not work, and which are likely to be harmful.

This is especially important in situations where health problems are severe and the scarcity of resources makes it vital that they are not wasted".

Chinnock P, Siegfried N, Clarke M (2005) PLoS Med 2(5): e107

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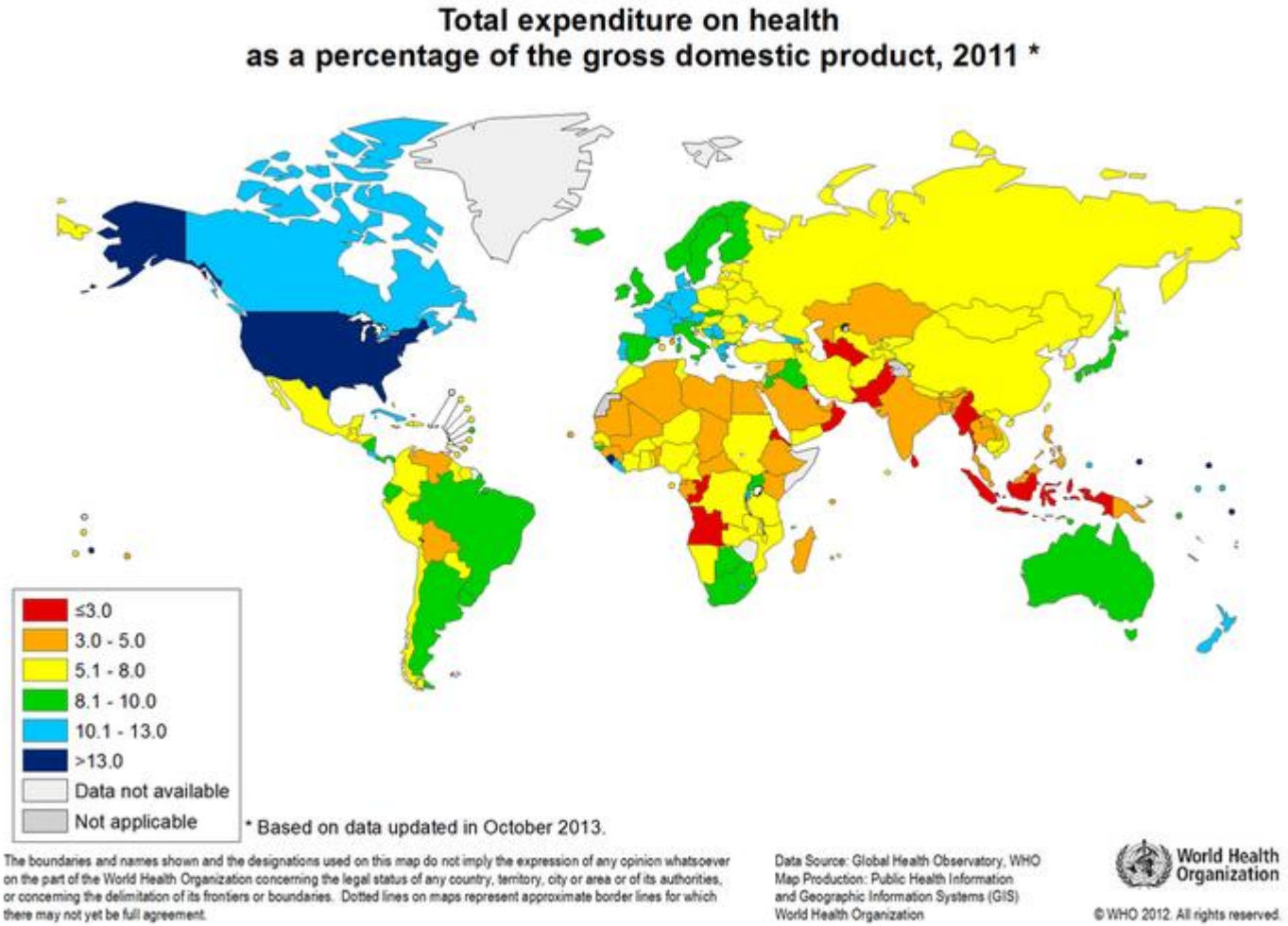
Health Care Expenditure



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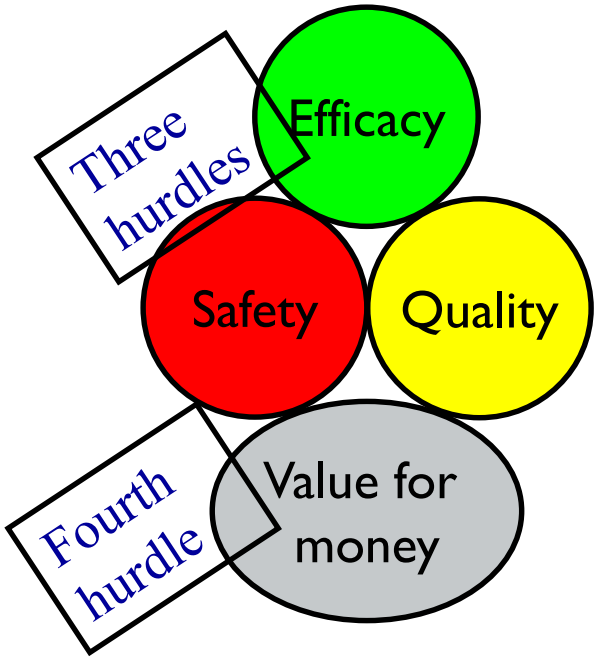
Requirements for New Medicines

Regulatory approval/market authorization –
BENEFIT - RISK

Market access and reimbursement –
COST EFFECTIVENESS

Can we pay for it?
AFFORDABILITY

Fifth hurdle



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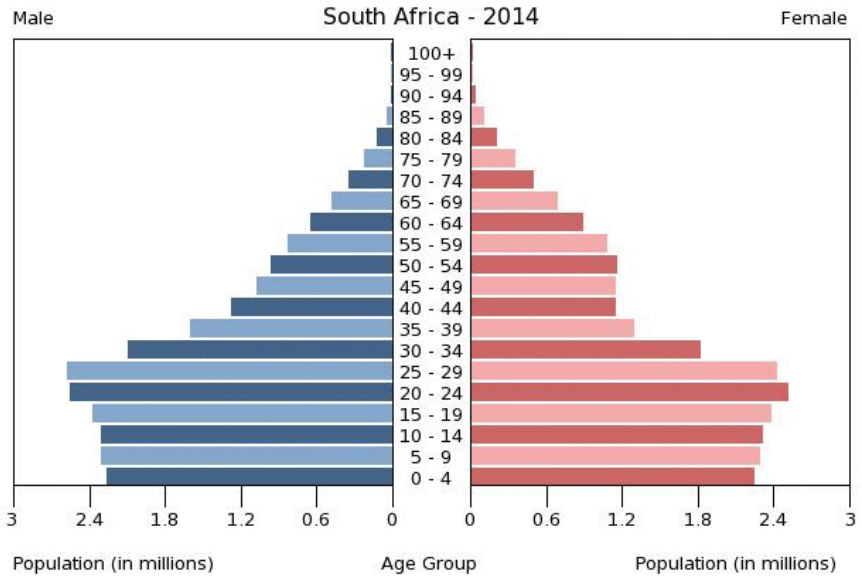
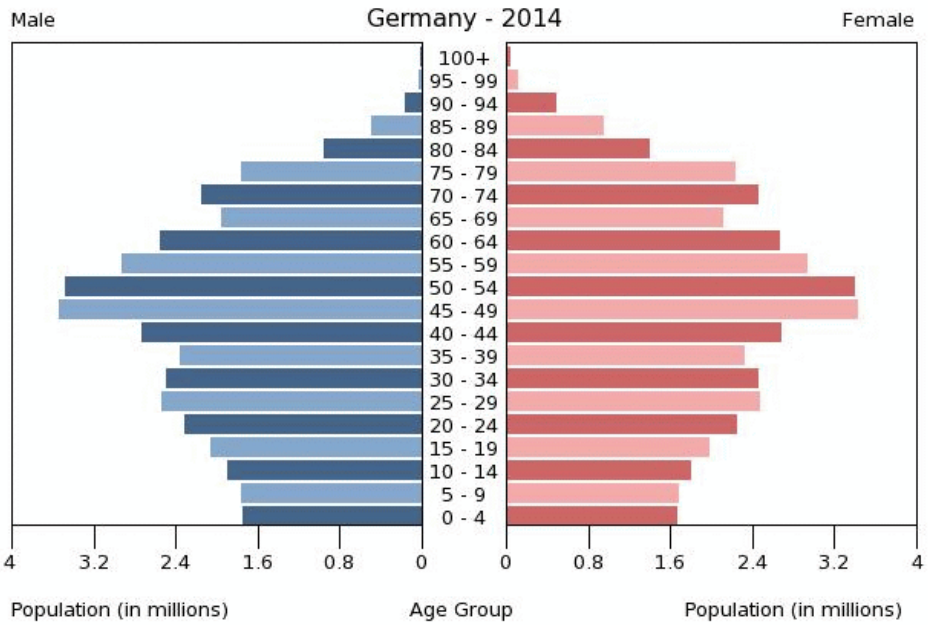
Need for Capacity Building

Population Pyramid: South Africa and Germany





From: Indexmundi.com

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Medicines Development: Competency Development

Medicines Development: Harmonization of Training Programmes

- **Standard syllabus and learning outcomes**
- **Quality control**
- **Examinations**

SMD SPECIALIST IN MEDICINES DEVELOPMENT
MMD MASTER OF MEDICINES DEVELOPMENT
PharmaTrain
MASTERING MEDICINES DEVELOPMENT
CENTRE OF EXCELLENCE
MRA MASTER OF REGULATORY AFFAIRS
CLIC CLINICAL INVESTIGATOR CERTIFICATION

http://www.pharmatrain.eu/

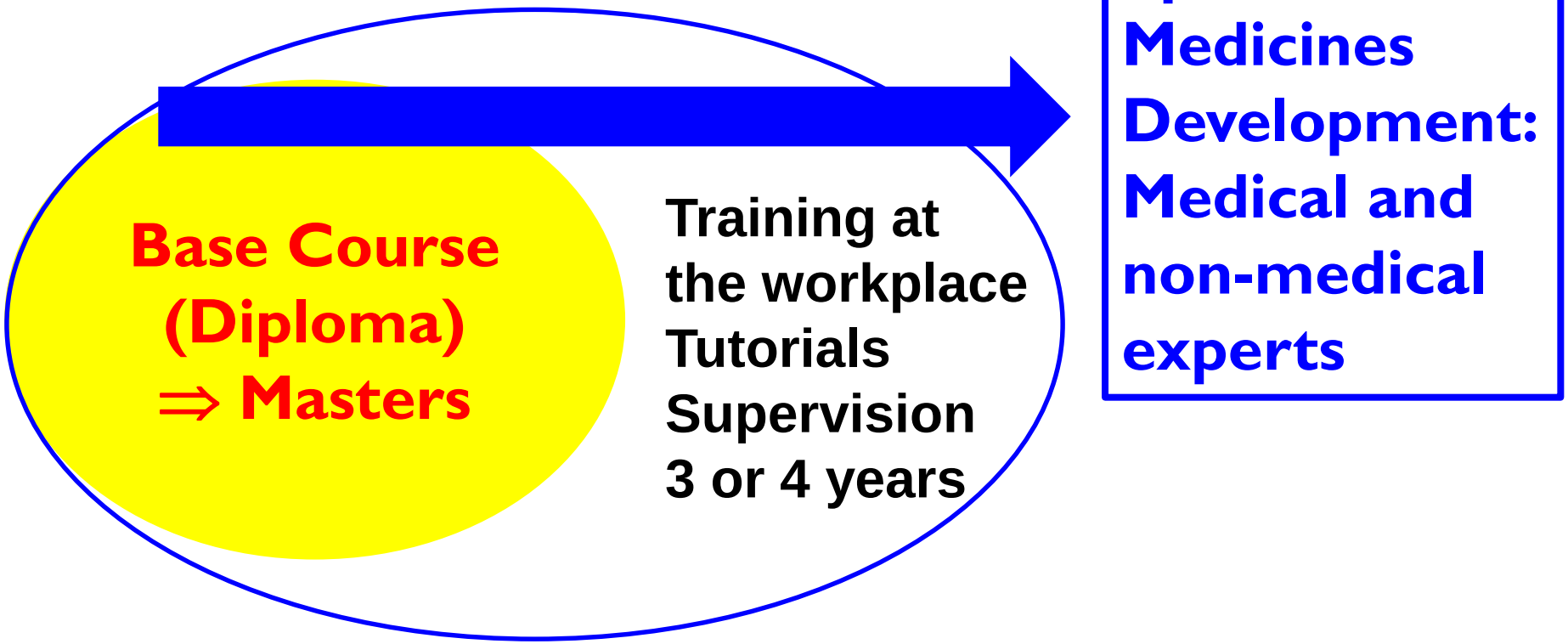
INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS

http://www.ifapp.org/

IFAPP

- UK Faculty of Pharmaceutical Medicine (FPM): Diploma in Pharmaceutical Medicine
- Non-UK activities managed via International Committee

The Path for Specialization: The PharmaTrain View



S. Kerpel-Fronius, 2014

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What About Africa ?

South Africa 'Not Ready For World Cup'

Share 1 retweet Share Comments (2)

5:07pm UK, Tuesday February 23, 2010

Emma Hurd, Africa correspondent

Fifa has admitted that South Africa is not yet ready to host the 2010 World Cup, with just over three months to go before kick off.

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Specific Needs for Africa

- Patient population
  - Age distribution
  - Racial profile
  - Genetic background
- Disease spectrum
- Health system
  - Traditional medicines

- Drug discovery
- Clinical trials expertise
- Manufacturing
- Medicines regulation - SAHPRA

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Pharmaceutical Industry in Africa

- Africa is one of the fastest growing economic regions – value of pharmaceutical industry increased from $ 4.7 billion in 2003 to $ 20.8 billion in 2013
  - Sub-Saharan Africa: mainly in Kenya, Nigeria and SA
  - Still relatively small compared to China and India
- Imports comprising as much as 70-90 % of drugs consumed in most countries in sub-Saharan Africa
- 375 companies with manufacturing sites in Africa to serve a population of around 1.3 billion people
  - Only 6 in sub-Saharan Africa which have achieved WHO pre-qualification

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Pharmaceutical Industry in South Africa

- Clinical trials - Focus of pharmaceutical R & D in South Africa
  - Excellent medical qualification
  - € 1.3 - 2 billion generated annually through clinical trials in South Africa (Kahn, SAMJ 2008; SACRA, estimate 2008)
  - 2480 active trials in the country at any time (May 2018)
- Long and unpredictable review timelines by Regulatory Agencies
  - Establishment of SAHPRA

Need for trained staff in industry,
clinical research groups/CROs and Agencies in Africa

Specialist in Medicines Development

- PharmaTrain Global PharmaTrain Certification Board (gPCB) established

- Regional (country) programmes require:
  - National PharmaTrain Certification Boards (nPCBs)
  - PharmaTrain Regional Advisor
  - Mentors for individual SMD participants

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SMD – First Programmes in Italy and Japan

SMDとは？

SMDは、Specialist in Medicines Developmentの略称です。
SMDプログラム（以下、本プログラム）は、各国の製薬医学教育コースを認証するヨーロッパのNPO法人PharmaTrainにより、2016年から試行的導入が開始された新たな国際認定制度です。今般、2017年度からは正規プログラムとして運用を開始します。

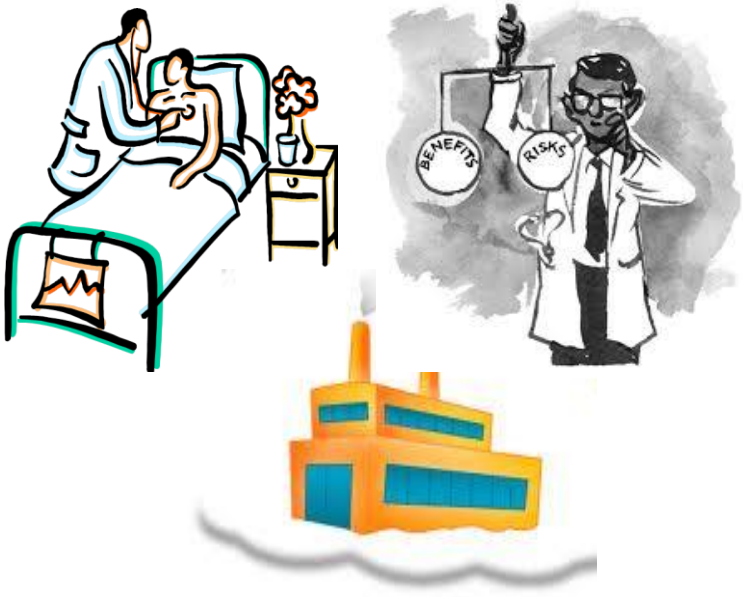
Continuous Professional Development (CPD): Meeting the Challenge

How to develop further my competencies and professional career?

University

Post-graduate courses
Whom to teach ?
What to teach ?

Workplace



How to train competent clinical investigators, regulators, and industry professionals ?

Needs cooperation between academic centers and various work places

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International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine (IFAPP)

http://www.ifapp.org/

Individual membership

Country organizations:
DGPharMed e.V.
SAAPP

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CLINICAL INVESTIGATOR (AND SITE STAFF) CERTIFICATION COURSE

Nitida Wine Farm, Tygerberg Valley Road, Durbanville

11 – 15 MARCH 2019

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Watch the Space: From 2014 to 2020

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WORKSHOP

NOVEL CLINICAL TRIAL METHODOLOGIES 2019

25 February 2019

25 FEBRUARY 2019		
08:30 - 09:00	Bernd Rosenkranz	Welcome and introduction
	Modelling and Simulations	**Chair: Phumla Sinxadi**
09:00 - 09:45	Florian Marx	Modelling the dynamics and control of infectious diseases: The example of tuberculosis
09:45 - 10:30	Leon Aarons	Pharmacokinetic and pharmacodynamic modelling
10:30 - 11:00		TEA BREAK
11:00 - 11:45	Joel S. Owen	Novel clinical trial designs for optimizing parameter estimation in malaria disease-drug models
11:45 - 12:00	Lindi Mathebula & Duduzile Nwandwe	South African clinical trials database - an update
12:00 - 12:15	Elizabeth Allen	Global health working group for the Trials Methodology Research Partnership
12:15 - 13:45		LUNCH BREAK
	Clinical Trial Designs	**Chair: Colin Pillai**
13:45 - 14:30	Jay Park	Improving efficiency and quality in clinical trials in global health research: Adaptive trial designs and master protocols
14:30 - 15:15	Natasha Pillai	Real world data and evidence in clinical trial design
15:15 - 15:45		TEA BREAK
15:45 - 16:30	Helmuth Reuter	Investigator experience: Role in trial design, conduct, analysis and reporting
16:30 - 17:15	Mireille Muller	Innovative trial designs: Engaging with regulators, ethic committees and patients
17:15 - 17:45	All Speakers	General discussion: Novel clinical trial methodologies - International and African perspectives
17:45 - 18:00	Colin Pillai	Closing remarks

„For every human problem, there is always an easy solution that is neat, plausible and wrong.“

(Henry Louis Mencken, 1880 - 1956)