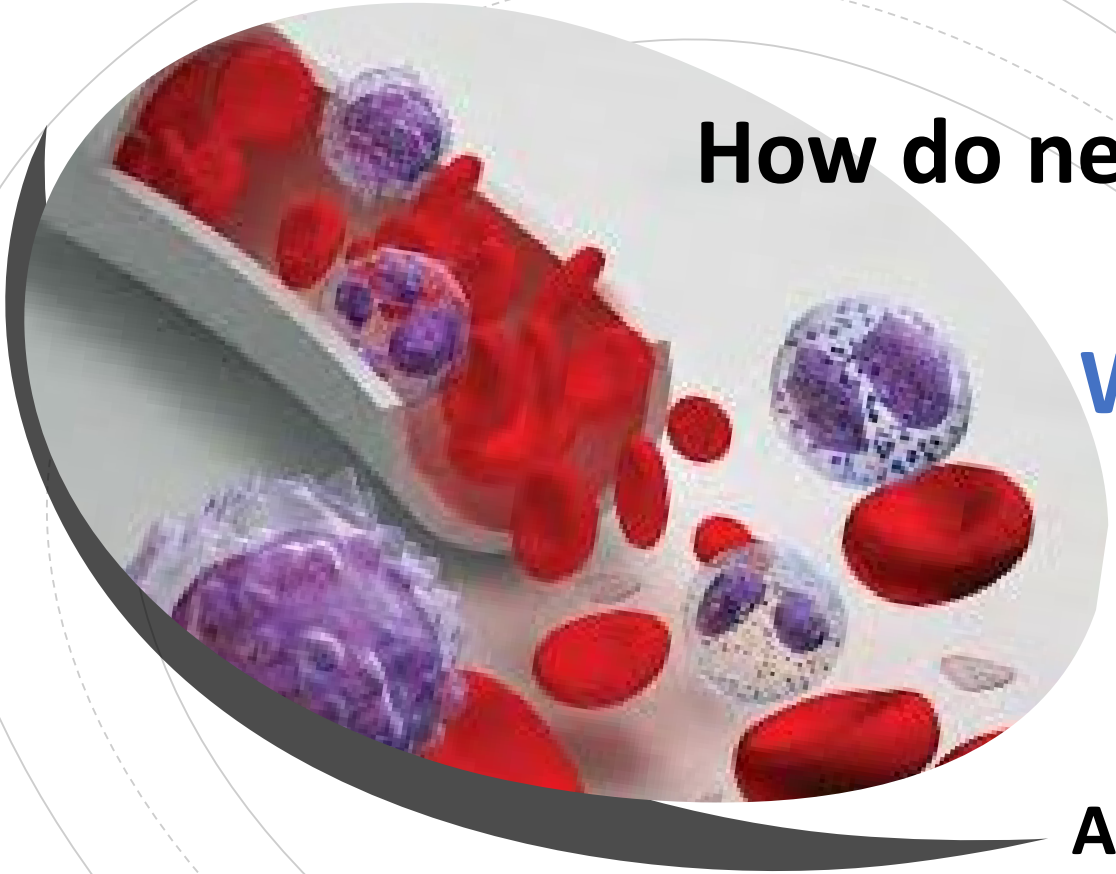
Centre for BLOOD RESEARCH

UNIVERSITY of York



How do new medicines become available?

Why/ how CBR needs to work with clinicians

Adele K. Fielding
Professor of Haematology

HULL YORK MEDICAL SCHOOL

What I am going to talk about:

- What are clinician-scientists?
- Clinical Trials 101 – a primer
- How positive results from clinical trials lead to medicines being licensed
- How the UK decides which licensed medications it will fund for the population

Clinician-scientist: Common misunderstandings?

Random woman taking payment
from me in shop

Proud, newly qualified doctor
Circa 1988, with new bank card

Clinician-scientists/academic clinicians: dually qualified "MD, PhD"

Great clinicians and great scientists?

Focused on one clinical area, understand the full depth and breadth of that area clinically and scientifically, can 'translate' metaphorically and literally – often involved in designing and leading clinical trials

Dodgy clinicians and underpar scientists?

see fewer patients than full time clinicians

less time to focus on the research lab

sometimes less well trained than non-clinical scientists

pompous, self important and

A well-known UK (now US-based!) clinician-scientist talking to a very famous UK scientist at dinner

"Oh, I just do clinical trials, its easier than doing basic science…"

Response to Dr ……..

- If you were planning the most complicated expensive experiment in your life, where every single cell culture or blots (and all of their relatives) could talk to you and ask you for a detailed rationale of what you planned to do and then take their time to decide whether to consent……..

- Would you think it was something to invest less thought, preparation or scientific insight into ?

What is a clinical trial?

Any medical research study involving people

Observational

No intervention – people receive usual/no treatment

Can collect data, blood, tissue samples etc

Types of studies

registry/

pos veillance/real world

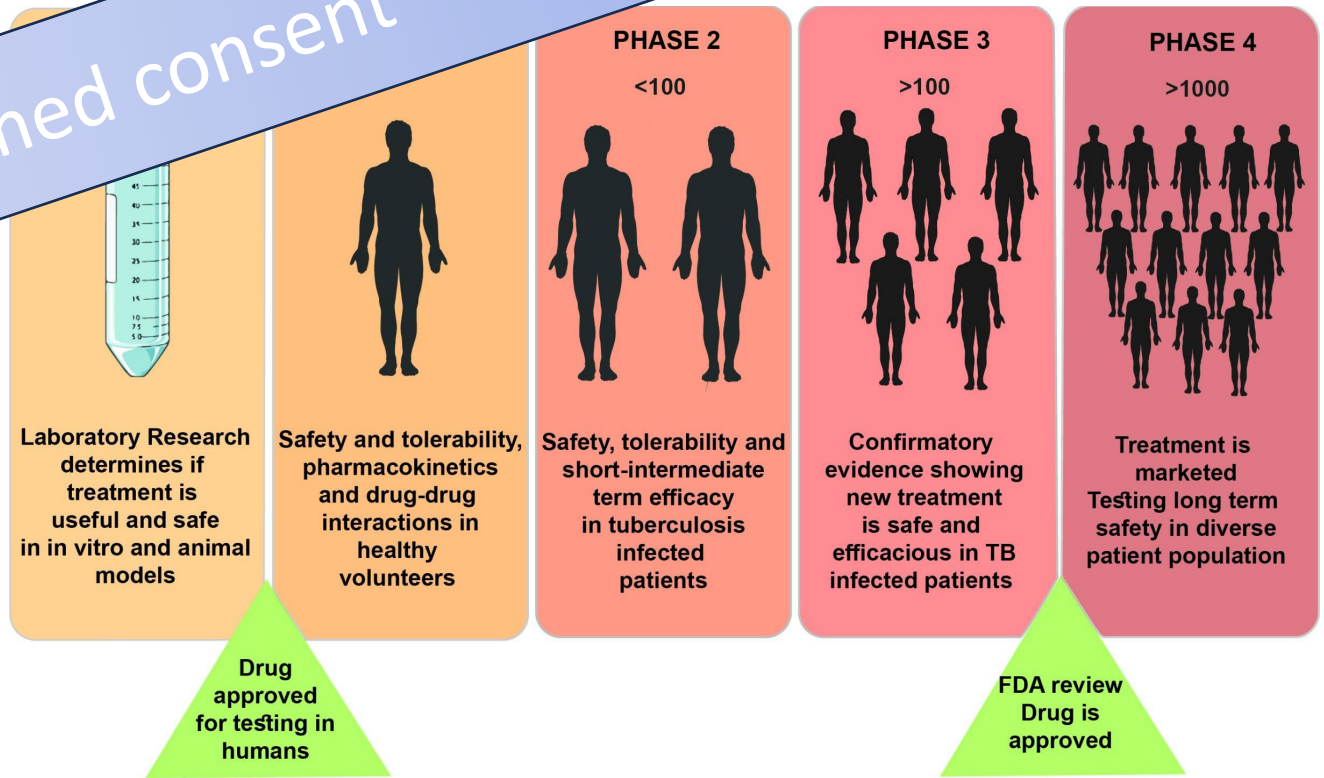
obse ional studies

cohort studies

Inter

Te exercise regimen

Informed consent



ICH Good Clinical Practice (GCP) - more rigid standards than normal medical care

International Conference on Harmonisation of technical requirements for registration of pharmaceuticals for human use

Tight guidelines on ethical and practical conduct of a clinical study.

The rights, safety and wellbeing of trial subjects are paramount

The clinical trial data are authentic and credible and internationally valid

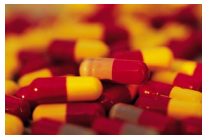
Comprehensive documentation: record keeping according to defined standards not just of data but exhaustive documentation of training, reporting of events,…..

Constant monitoring and inspections ensure that these standards are achieved.

What do you need to do a clinical trial?

SPONSOR (legally responsible for conduct under GCP)

Chief Investigator



Drug, device or intervention worth testing

Rare condition - collect data and specimens

ETHICAL APPROVAL

NATIONAL REGULATORY BODY APPROVAL
*MHRA for UK

Tissue collection/storage/ access rules

Genetic modification rules

Radiation exposure rules

SITE(s) approval, willingness and ability
- recruit and care for patients
- provide data

Principal Investigator needed as a lead for each site

Role of a clinical trial investigator

Chief Investigator

The named Chief Investigator (CI) takes overall responsibility for the conduct of the proposed research.

Can supervise the entire research effectively; communicates with the Research Ethics Committees (REC) and other review bodies during the application process and during the conduct of the research.

Principal Investigator

The principal investigator is responsible for a single research site There should be one PI for each research site.

How do trial results lead to medicines being available for patients ?

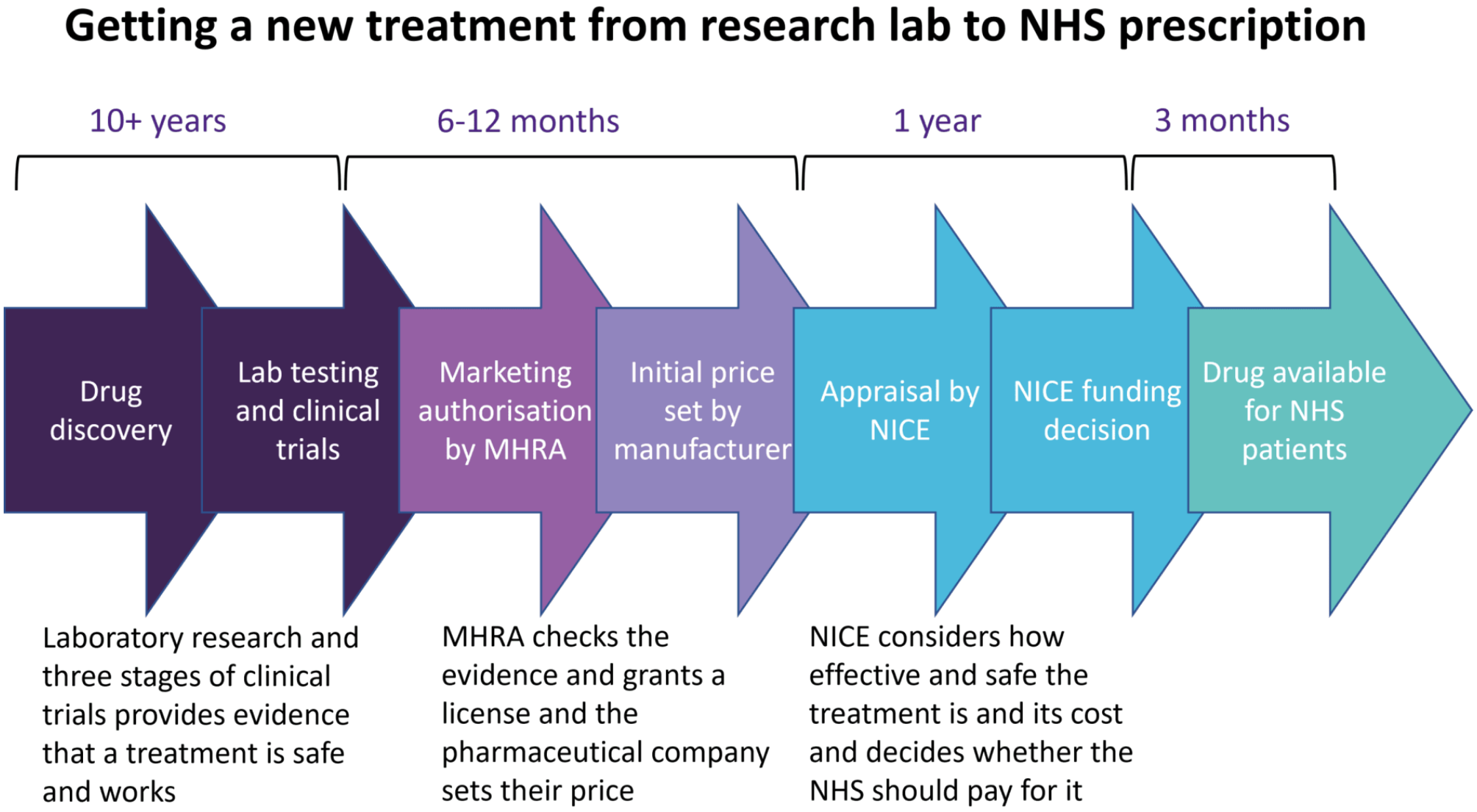
Regulatory approval -> licence

Marketing authorisation is **the process of reviewing and assessing the evidence to support a medicinal product, such as a drug, in relation to its marketing, finalised by granting of a licence to be sold**.

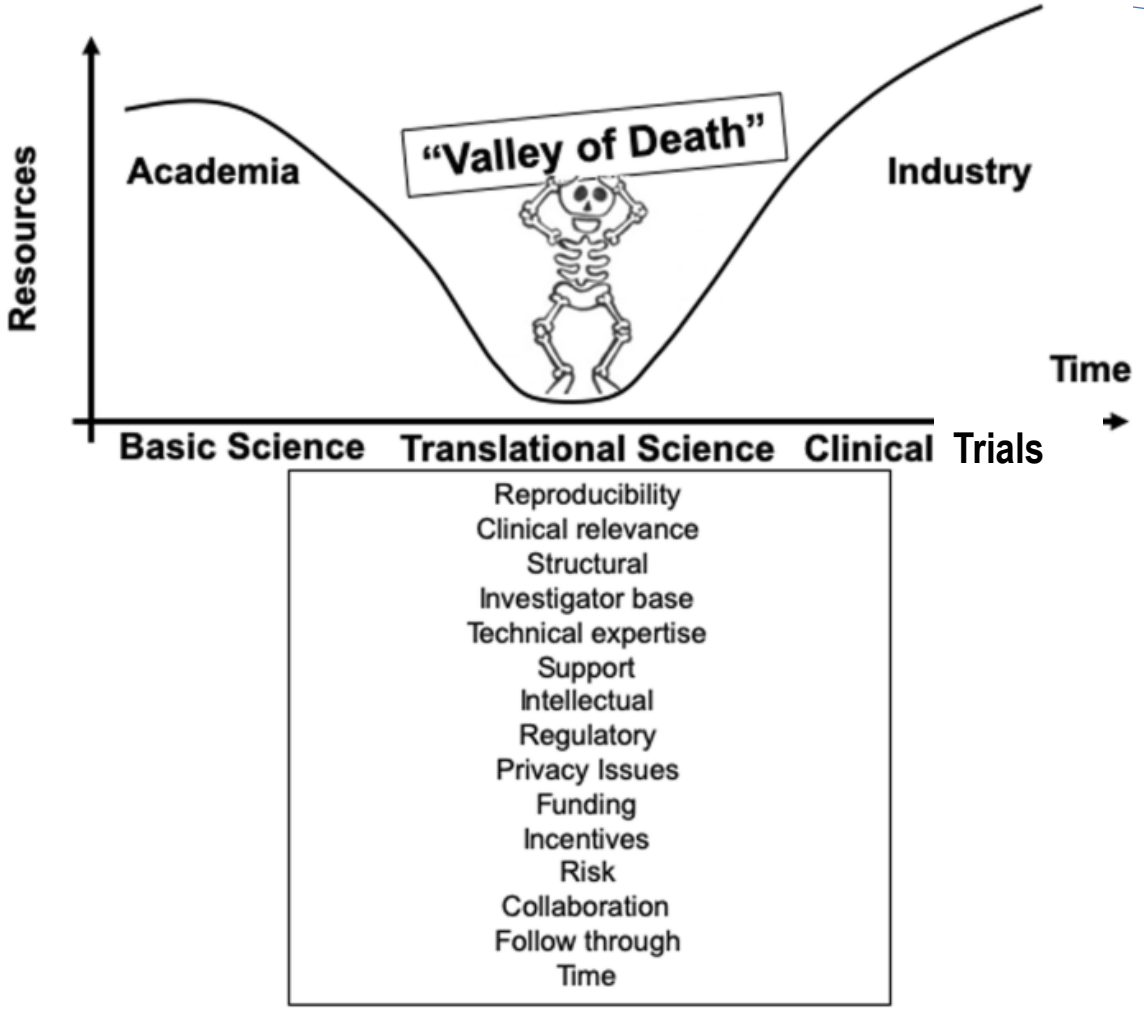
The licence **states which illness the medicine can be used for how much can be used how to give the medicine which group of patients it can be given to**. The licence is provided by a government organisation called the Medicines and Healthcare products Regulatory Agency, also known as MHRA.

it requires careful orchestration across R&D and commercial functions over the year—or longer—that it takes to develop a filing strategy and prepare the dossier for a regulatory agency.

How do trial results lead to medicines being available for patients ?



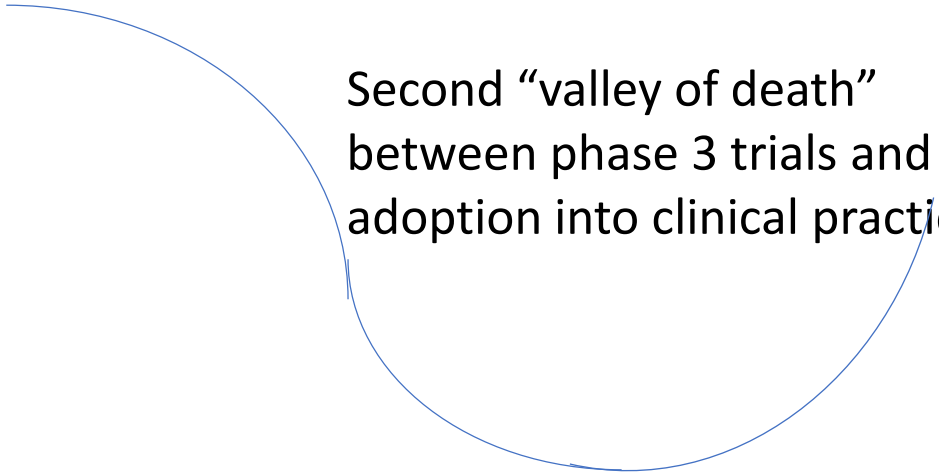
Why does it take so long for medicines to become available for patients ?



Second "valley of death" between phase 3 trials and adoption into clinical practice

Medicines & Healthcare products Regulatory Agency

NICE
National Institute for Health and Care Excellence



An academic investigator cannot change the licence as they don’t own the drug

Some companies don’t want to pursue licence change as it costs a lot of work/money

Many countries - incl UK - do not allow “off-licence” prescribing

A drug with a positive trial outcome may not be cost effective

Who is footing the bill?
Licensing vs. ‘reimbursement’

NICE
National Institute for
Health and Care Excellence

reviews each treatment bases their decision on the best available evidence

Quality Adjusted Life Years (QALYs) used to assess the benefit and quality of life a treatment will provide

input from experts - lay members and members from clinical practice, public health, social care and industry

public involvement - patients, carers, service users and the general public

Centre for BLOOD RESEARCH

UNIVERSITY of York

Committed to working together as a team to learn about the science of blood cancers, develop and test new treatments and improve outcomes for patients

Epidemiology, basic science, clinical science, patients

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Thank you

UKALL14 and 60+
Trial Management Groups
David Marks
Clare Rowntree
Anthony Moorman
Nick Morley
Andrew McMillan
Tobias Menne
Bela Patel

Emily Cutler

Aditi Dey

Jenny Chatzerigou

Rosie Amerikanou

Hermione Allen

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Past AKF lab members

My lovely clinical colleagues over many years

UCL CTC

Amy Kirkwoood senior statistician
Paul Smith
Emma Lawrie
Amy Douglas
Pip Patrick
Laura Clifton Hadley
Zaynab Rana

Infrastructure and trial coordination

UCL Cancer Trial Centre

- UK National Cancer Research Institute
- National Health Service Hospitals and staff 69 centres