Discovery and development of drugs

4.3.1.9 · Biology · revision notes

Where medicines come from (plants, microorganisms and chemists' laboratories) and how a new drug is tested before it can be used: preclinical testing, clinical trials, placebos, double blind trials and peer review. A common 4 to 6 mark 'describe' question, and a good source of data questions.

Key facts

- Digitalis (heart drug) ← foxgloves
- Penicillin ← *Penicillium* mould, discovered by Alexander Fleming
- Preclinical testing: cells, tissues and live animals
- Placebo = looks like the drug but contains no drug
- Peer review: other experts check the results before they are published
- Aspirin (painkiller) ← willow
- New drugs are tested for toxicity, efficacy and dose
- Clinical trials: healthy volunteers and patients; very low doses first, then find the optimum dose
- Double blind: neither doctors nor patients know who gets the drug or the placebo

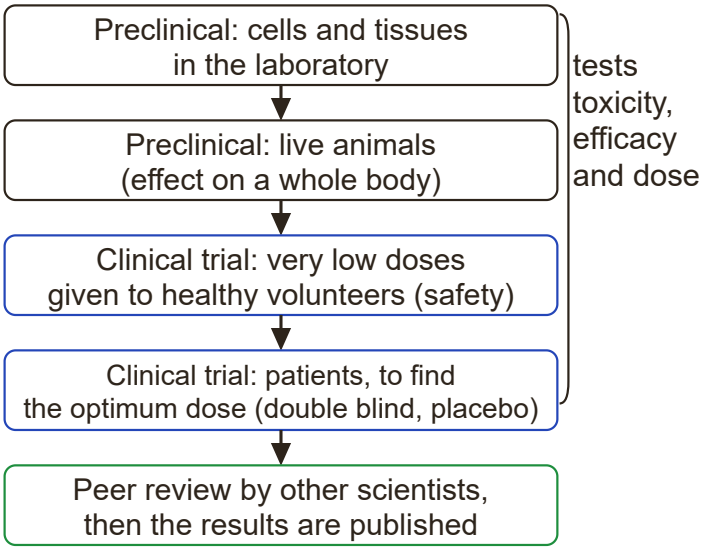
Notes

Where drugs come from

- Traditionally, drugs were extracted from **plants** and **microorganisms**.
- The heart drug **digitalis** comes from **foxgloves**. The painkiller **aspirin** comes from **willow**.
- **Penicillin** was discovered by **Alexander Fleming** from the *Penicillium* mould. He noticed that bacteria did not grow around some mould that had got onto one of his culture plates.
- Most new drugs are now **synthesised by chemists** in the pharmaceutical industry, but the starting point may still be a chemical extracted from a plant.

Testing a new drug

- New drugs must be tested and trialled to check that they are safe and effective. They are tested for **toxicity** (harm), **efficacy** (how well they work) and **dose**.
- **Preclinical testing** is done in a laboratory using **cells**, **tissues** and **live animals**. Animals show the effect of the drug on a whole body.



The stages of testing a new drug, in order.

- **Clinical trials** use **healthy volunteers** and **patients**.
- Very **low doses** are given to healthy volunteers at the start of a clinical trial, to check that the drug is safe.
- If the drug is safe, further clinical trials find the **optimum dose**: the dose that works best with the fewest side effects.

Placebos and double blind trials

- A **placebo** looks like the drug but does not contain the drug. In a trial, some patients get the drug and some get the placebo.
- Some patients feel better just because they believe they are being treated, so comparing the two groups shows whether the drug has a real effect.
- In a **double blind trial**, neither the doctors nor the patients know who has received the drug and who has received the placebo until the trial is complete. This stops their expectations affecting the results.

Publishing the results

- The results are published only after **peer review**: other scientists who are experts in the same field check the method, results and conclusions.
- This makes it less likely that false or biased claims are published.

How to answer each type of question

Recall: sources of drugs

1 mark each | Grade 3

1. Learn the three pairs: foxglove → digitalis, willow → aspirin, *Penicillium* → penicillin.
2. Remember who discovered penicillin: Alexander Fleming.

Example. Give the original source of each drug.
(a) Aspirin
(b) Digitalis
(c) Penicillin

Model answer.

(a) Willow (1)
(b) Foxgloves (1)
(c) *Penicillium* mould (1)

6-mark: describe how a new drug is tested

4 to 6 marks | Grade 5

1. Start with preclinical testing: cells and tissues, then live animals.
2. Clinical trials: very low doses first to check safety, then patients to find efficacy and the optimum dose.
3. Mention the placebo and the double blind design.
4. Finish with peer review before the results are published.

Example. A company has developed a new drug to lower blood pressure.
Describe how the drug would be tested before doctors could use it. (6 marks)

Model answer.

The drug is first tested in a laboratory on cells and tissues, to check that it is not toxic. It is then tested on live animals, to see its effect on a whole body. If it seems safe, clinical trials start: very low doses are given to healthy volunteers to check for harmful side effects. The drug is then given to patients with high blood pressure to find out whether it works and to find the optimum dose. Some patients are given a placebo instead, and the trial is double blind, so neither the doctors nor the patients know who has the drug. Other scientists check the results (peer review) before they are published.

This is a Level 3 answer (5 to 6 marks): the stages are in a logical order and linked to what each one tests. A list of stages with no detail would be Level 1 or 2.

Explain placebos and double blind trials

2 to 3 marks | **Grade 7**

1. Placebo: looks like the drug but contains no drug.
2. Double blind: neither the doctors nor the patients know who has the drug.
3. Give the reason: so expectations cannot bias the results.

Example. In a trial of a new painkiller, half the patients were given a placebo. The trial was double blind.
(a) What is a placebo?
(b) Explain why the trial was double blind.

Model answer.

(a) A substance that looks like the drug but does not contain the drug (1)

(b) Neither the doctors nor the patients knew who had the drug (1), so the doctors' and patients' expectations could not affect (bias) the results (1).

Data: results of a clinical trial

2 to 3 marks | **Grade 7**

1. Percentage = number improved ÷ number in the group × 100.
2. Compare the drug group with the placebo group.
3. Explain any improvement in the placebo group.

Example. In a trial, 200 patients took a new drug and 200 patients took a placebo. The table shows the results.

Group	Number of patients	Number whose symptoms improved
New drug	200	138
Placebo	200	46

(a) Calculate the percentage of patients whose symptoms improved in each group.
(b) Suggest why some patients who took the placebo improved.

Model answer.

(a) Drug: 138 ÷ 200 × 100 = 69% (1); placebo: 46 ÷ 200 × 100 = 23% (1)

(b) They believed they were being treated, so they felt better (the placebo effect), or they would have recovered anyway (1).

Don’t lose marks

- Saying the full dose is given to patients straight away. Very low doses are given first, to check the drug is safe.
- Describing a trial as double blind when only the patients don't know. In a double blind trial the doctors don't know either.

- ✗ Mixing up the sources: aspirin comes from willow, digitalis from foxgloves.
- ✗ Saying peer review is done by the company that made the drug. It is done by other scientists who are experts in the field.
- ✗ Mixing up efficacy (how well the drug works) and toxicity (whether it is harmful).

More tips

Memory tricks

- Sources: foxglove → digitalis, willow → aspirin, *Penicillium* → penicillin.
- Testing checks 'TED': Toxicity, Efficacy, Dose.
- Order: cells and tissues → animals → very low dose in people → patients (optimum dose) → peer review.
- Double blind = two groups kept in the dark: the doctors AND the patients.

Exam technique

- In 6-mark 'describe the testing' answers, keep the stages in order and say what each stage checks.
- Learn the exact terms: toxicity, efficacy, dose, preclinical, clinical, placebo, double blind, peer review.
- In data questions, compare the drug group with the placebo group, and use percentages if the groups are different sizes.

What each grade needs

What you need to be able to do, from the first marks up to the top grade.

Grade 3 — **Match drugs to their original sources**
Digitalis from foxgloves, aspirin from willow, and penicillin from *Penicillium* mould (discovered by Alexander Fleming).

Grade 4 — **State what new drugs are tested for**
Toxicity (is it harmful?), efficacy (does it work?) and dose (how much should be given?).

Grade 5 — **Describe preclinical testing**
The drug is tested in a laboratory on cells, tissues and live animals before any people take it.

Grade 5 — **Describe the stages of clinical trials**
Very low doses are given first to check safety; further trials on patients find the optimum dose.

Grade 5 — **Explain why results are peer reviewed**
Other experts check the method, results and conclusions before publication, so false or biased claims are less likely to be published.

Grade 6 — **Explain why a placebo is used**
A placebo contains no drug, so comparing the two groups shows whether the drug itself has an effect.

Grade 7 — **Explain why double blind trials are used**
Neither doctors nor patients know who has the drug, so their expectations cannot bias the results.

Free notes, Full packs, practice papers and mock exams at chhetriacademy.com. Written for this site; not taken from past papers. Stuck? 1-to-1 tutoring with a free 30-minute trial lesson.