Clinical Trials aim to

- Confirm the drug works
  (in the case of Gorlin syndrome it could work at treating BCCs some a person already has or prevent future BCCs from coming along.
- Confirm that the drug also meets the individual's needs.
  For example, the trial could confirm that the drug enables an individual to attend school or go to work more often, that they can enjoy day to day activities or simply that they feel happier. These are sometimes called Health Related Quality of Life or Patients Related Outcome Measures (PROMs).
- See how often side effects happen.
  This is most successful for trials that include many people and take place over a long period because some side effects only happen occasionally or after a long time on treatment.
- See how well a drug is 'tolerated'.
  This means – how convenient is a drug to use. For example, if a drug is a cream applied to the face – it won't be use if you can't apply sunscreen on top of it, and If a treatment causes distress: pain, nausea, unsightly inflammation then people are unwilling to continue to use it. Drugs which have to be given in hospital are obviously much less convenient than drugs which you can use at home.
- Work out how much the drug will cost to meet these aims.
  this is called Health Economics. This is obviously affected by the price of the drug. However, it is also affected by how well a drug works. If a drug works really well in 100% of people, it effectively costs less to achieve its aim than a drug which only works a bit, in only 50% of people. It was for this kind of reason that Vismodegib (Erivedge) worked out to be too expensive to be approved by NICE in the UK.

Double Blinded Controlled Randomised Clinical Trials

- **Double blinded** – neither the person in the trial or the trial team can know who is on which. Otherwise, it would be possible for the trial team to cheat and change the results so that the group receiving the new treatment do better.
- **Controlled** – the new drug has to be compared to something. Often, it can be compared to an existing treatment which is widely available. Sometimes, there is no existing treatment available and in this case the trial has to be compared to a placebo. For example, because there are no drugs designed for people with Gorlin Syndrome, most of the trials coming on line now use placebos.
  (A Placebo – this is a substance that looks and feels like the drug being tested, but does not contain the active ingredient, for example, it may be a sugar pill.)
- **Randomised** – the people taking part in the study have to be divided up at random, as to who gets the active treatment and who gets the standard treatment or the placebo. This has to be done at random because otherwise it would be possible for the team to cheat and put all the people on the they think will get better in the treatment group.

Other important things to understand

- **Inclusion criteria**. These define who can join a trial. For example – 'people with confirmed Gorlin Syndrome'
- **Exclusion criteria**. These define who can't join the trial. Sometimes these are obvious, for example women of child bearing age not using contraception are often excluded from a trial, to reduce the risk to an unborn baby. Sometimes the criteria are subtler, for example excluding people with mental illness. This would be a real problem for Gorlin Syndrome because about half of us have a history of depression or anxiety.
- **Informed Consent**. When you have any treatment, you should be asked to give informed consent. Often this is given verbally, for example if you have a urinary infection and your GP recommends antibiotics, the GP should check with you that you agree. The GP should tell you what the common side effects are. Most of us are used to signing consent forms when we go for operations, confirming we understand what the intended benefits and potential risks are. The same applies for any treatment which has not been thoroughly investigated – it should only start after informed consent has been given. The same is true for clinical trials.
- **Ethics Committee**. These are committees run by hospitals which oversee clinical trials. They include doctors and nurses, but also lay people. A trial can only go ahead if the Ethics Committee approves.