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Clinical Trials

- Phase One
- Phase Two
- ➤ Phase Three

Clinical Trials-Challenges

Patients Concept

- Fearful of clinical trials
- Feel they are daunting
- Myths & Pre- conceived ideas Guinea pig

How I deal with the challenges

- Find out the patients views on trials in general.
- Discover any personal issues that may exist.
- Discuss the Pro's and Con's of the trial.
- Allow patient the time to discuss with family friend and staff.

Benefits to Clinical Trials

- Gain Access to the newest drugs
- May give better Quality of life
- May not necessarily benefit you but will help people in the future.
- Being observed by another specialist team

Possible Disadvantages

- Extra Visits to the hospital
- More Tests (blood tests / ECG / Biopsies)

Luspatercept (ACE-536-MDS-001)

Medalist Trial

This is a phase 3, Double blind, randomised, placebo controlled, multicentre study.

Inclusion

- Diagnosed MDS patients with RARS disease (very low, low, intermediate risk disease).
- Requiring red blood cells Transfusions at least
 units / 8 weeks of red cells.
- Intolerant to erythropoietin.

Exclusion

- Prior Treatment with disease modifying agents (IMiDs such as lenalidamide)
- Previously treated with either Luspatercept (ACE-536) or sotatercept (ACE-011)
- MDS associated with del 5q cytogenetic abnormality
- History of stroke, DVT, PE within the last 6 months
- Uncontrolled hypertension
- Prior histories of malignancies

Visits

- Screening Visit (may need multiple visits to include ECG, blood & bone marrow tests.) (1)
- Treatment phase 24 weeks (8 treatment cycles)
- Initially Weekly for first cycle of treatment (3)
- Once every 3 weeks apart from cycle 5 which has a day 8 visit. (8)
- Up to another 9 visits post treatment (1 x 6 wks, 8 x 12 wks)

Total of around 21 visits over 2 years.

Case Study

Patient A

- MDS patient requiring regular blood transfusions
- Low platelets
- Offered Azacitadine Trial

Any Questions?

