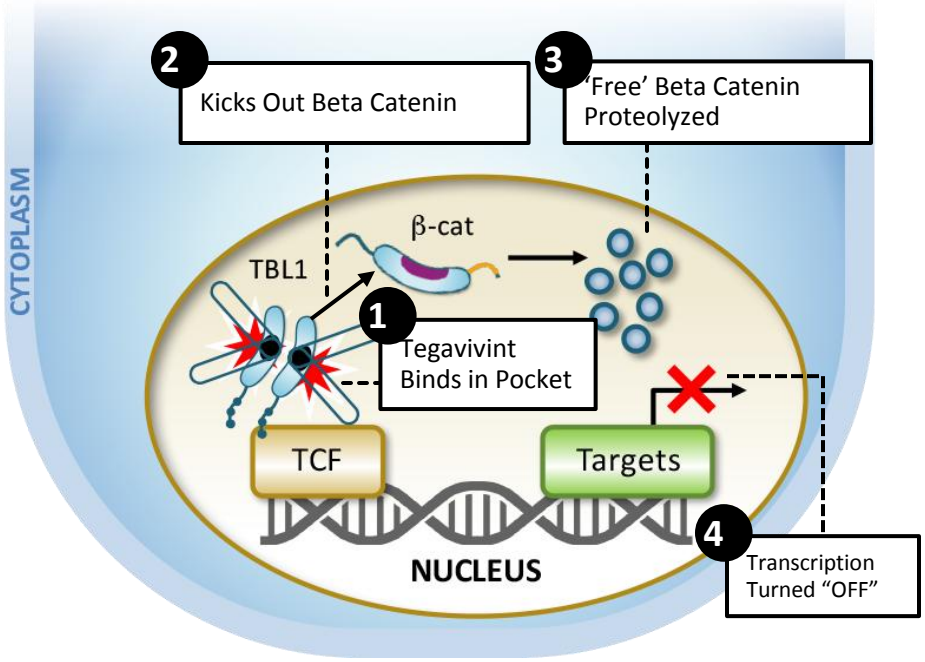
βCat Pharma

A Phase 1 Trial of BC2059 (Tegavivint) in Desmoid

A brief outline of the trial

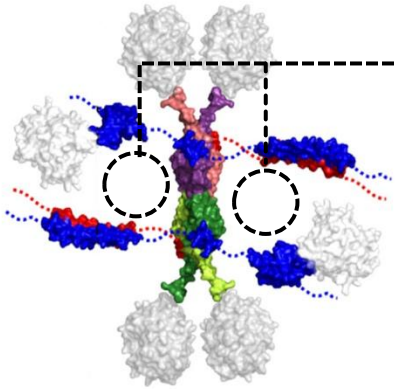
BC2059 (Tegavivint) is a small molecule inhibitor of beta catenin activity, the main driver of desmoid tumors.

Tegavivint–TREATED CELL



ADVANTAGES

TBL1 Tetramer



Hydrophobic Pockets Allow BC2059 to Bind Directly to TBL1 and Displace Beta Catenin; Free Beta Catenin is Proteolyzed

- Actually kills desmoid tumor cells
- Potent and specific
- No effect on membrane bound beta catenin in preclinical testing.
- No effect on normal stem cells repopulating the GI tract in preclinical testing

- KEY WORD IS PRECLINICAL.

This is a Phase 1 trial. What does that mean?

- Phase 1 trials are often the first time the drug has been tested in people.
- The primary purpose is to define safety and determine the proper dose.
- BC2059 (Tegavivint) has been extensively tested in a variety of animals, but not in people. So, this will be a Phase 1 trial.
- Because of that, patients on this trial will be monitored closely and frequently
  - Only a few patients at a time are treated initially
  - Doses start low and then are increased with each succeeding group of patients

- It's important to understand that, while there is a lot of biologic rationale for the drug, we don't know if it will work.
- We want to work closely with the DTRF in this process so that we can learn as much about Tegavivint in desmoid as possible.

FAQs about the trial

- Who will be eligible?
  - Patients with desmoid tumors “in need of treatment”
    - Tumor is progressing OR
    - Causing quality of life issues

    Note: FAP patients will not be eligible initially.
- What will we measure?
  - Safety, safety, safety….then,
  - Size of the tumor
  - Quality of life as measured by a questionnaire
- How will the drug be given?
  - BC2059 (Tegavivint) is a drug given intravenously for a 4 hour infusion once a week for three out of four weeks
- What about patients in the first groups that got a low dose of the drug?
  - If they are doing well, they can have their dose increased later

Current status of the trial

- Study protocol has been completed
- Initial discussions for possible trial sites:
  - U Washington/Fred Hutchinson (Dr. Lee Cranmer)
  - Memorial Sloan Kettering (Dr. Mrinal Gounder)
  - Princess Margaret/ U Toronto (Dr. Albiruni Razak)
  - Partners (MGH/DFCI) (Dr. Edwin Choy)
  - MD Anderson (Dr. Shreyaskumar Patel)
  - Norwell Health/Cold Spring Harbor (Dr. Robert Maki)
  - James Cancer Center/OSU (expansion phase only; Dr. David Liebner)
- Estimated trial opening January 2018
- We will work with the DTRF to communicate with the desmoid tumor the exact dates and locations where the trial will be available.

Thank you!