

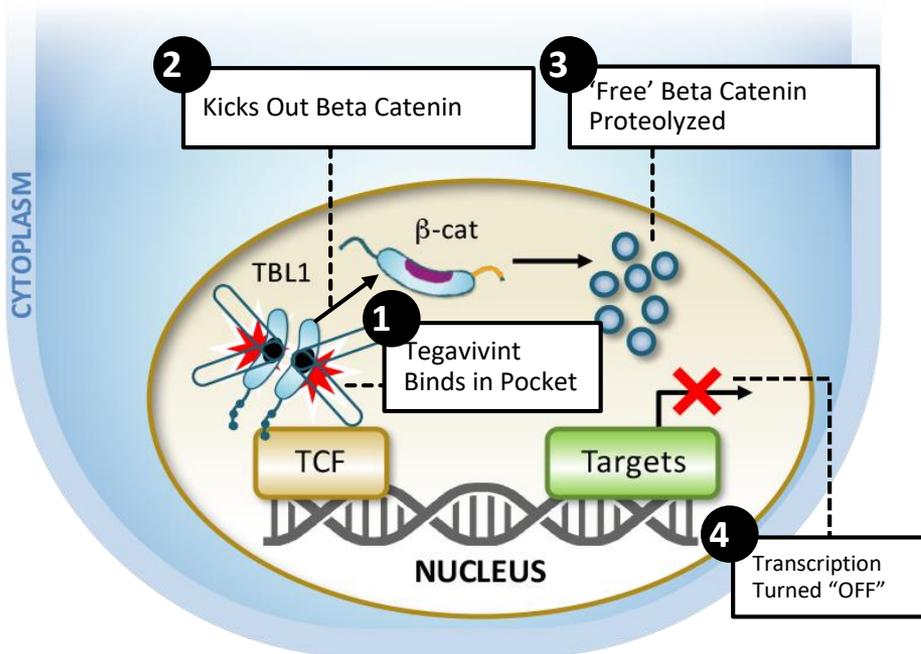


A Phase 1 Trial of BC2059 (Tegavivint) in Desmoid

A brief outline of the trial

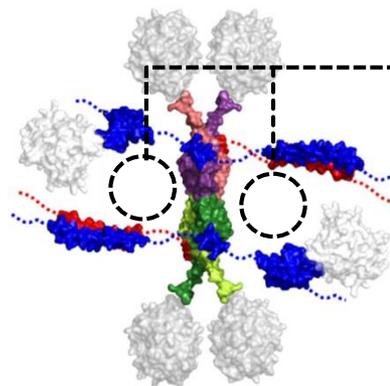
BC2059 (Tegavivint) is a small molecule inhibitor of beta catenin activity, the main driver of desmoid tumors.

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 is just now being tested in people.
- 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 is 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 - Sites

- The trial is now open at:
 - U Washington/Fred Hutchinson (Dr. Lee Cranmer)
 - Princess Margaret/ U Toronto (Dr. Albiruni Razak)
 - Partners (MGH/DFCI) (Dr. Edwin Choy)
- Later this fall it will be open at:
 - Memorial Sloan Kettering (Dr. Mrinal Gounder)
 - MD Anderson (Dr. Vinod Ravi)
 - UCLA Cancer Center (Dr. Noah Federman)
 - James Cancer Center/OSU (Dr. David Liebner)
- We will work with the DTRF to communicate with the desmoid tumor the exact dates and locations where the trial will be available.

Current status of the trial - Progress

- The first patient has been enrolled and has received two cycles (months) of drug without any problems.
- The second patient will begin at the next higher dose level shortly.
- Once the second patient has completed one cycle (month), we will enroll 3 more patients at the next higher dose level. This is still a lower dose than we think will be eventually used.

Thank you!