



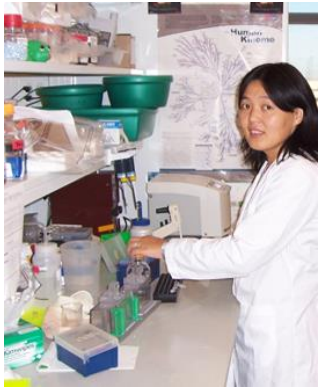
A Cancer Center Designated by the
National Cancer Institute

John L. Villano, MD, PhD

October 17, 2018

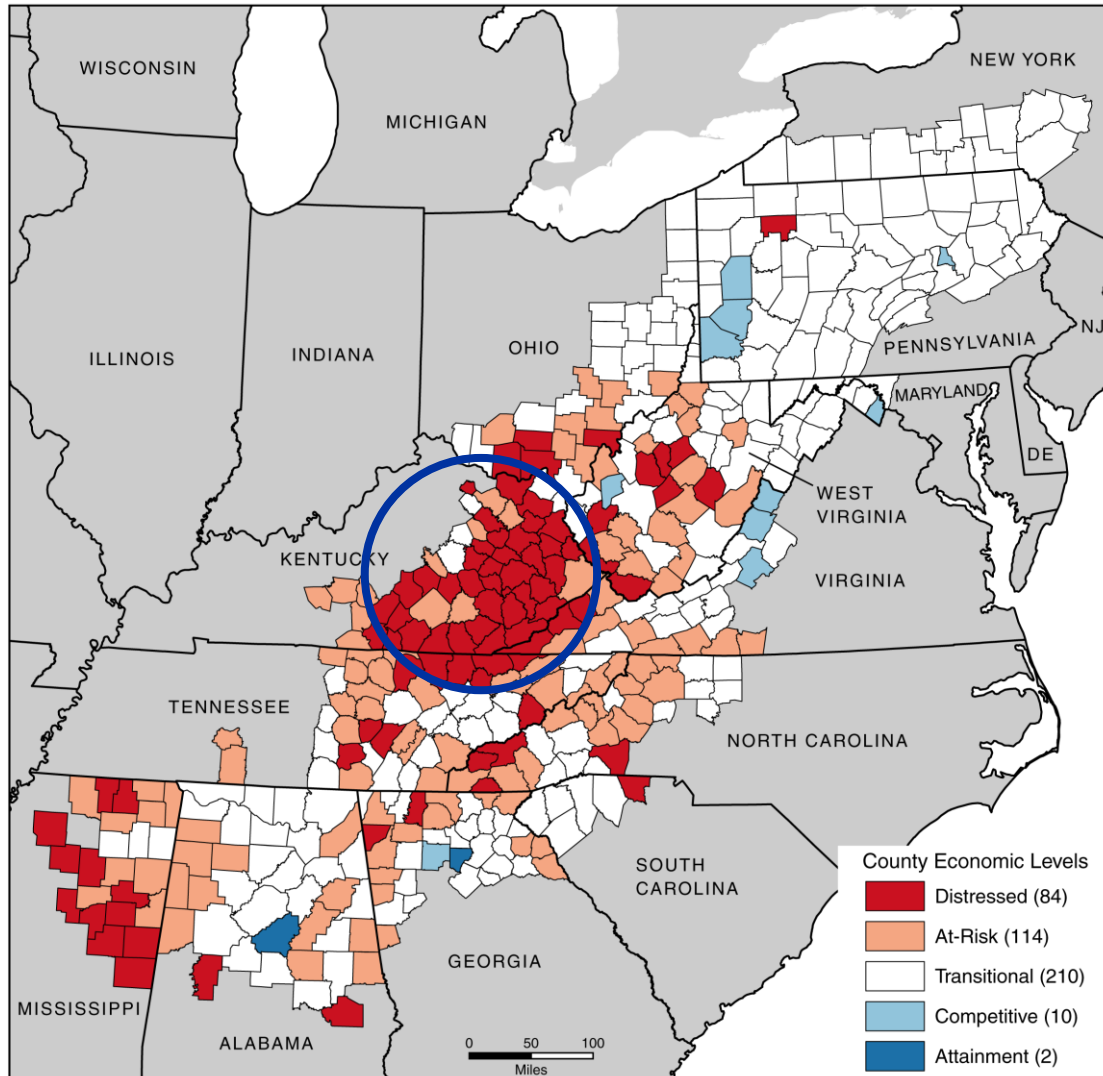
Our Mission

- To reduce cancer mortality in our state through a comprehensive program of cancer research, treatment, education, and community engagement with a particular focus on the underserved population of Appalachian Kentucky.



Conquering Cancer in the Commonwealth

Appalachian Kentucky



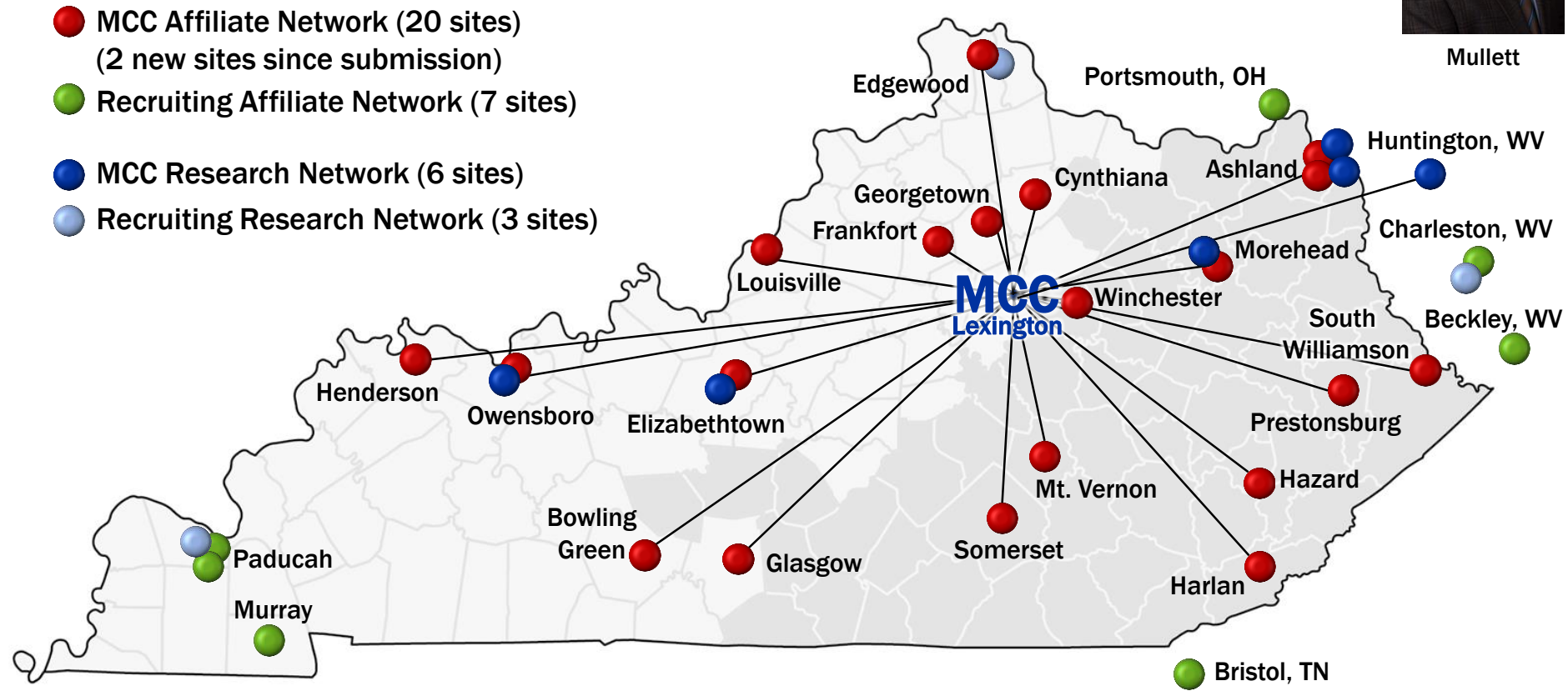
- ↑ Cancer
- ↑ Smoking
- ↑ Obesity
- ↑ Chronic Infection (HPV & HCV)
- ↑ Poverty

Appalachian county economic status (2016-17)

MCC Network: Expanding Access Throughout Kentucky and Beyond



Mullett



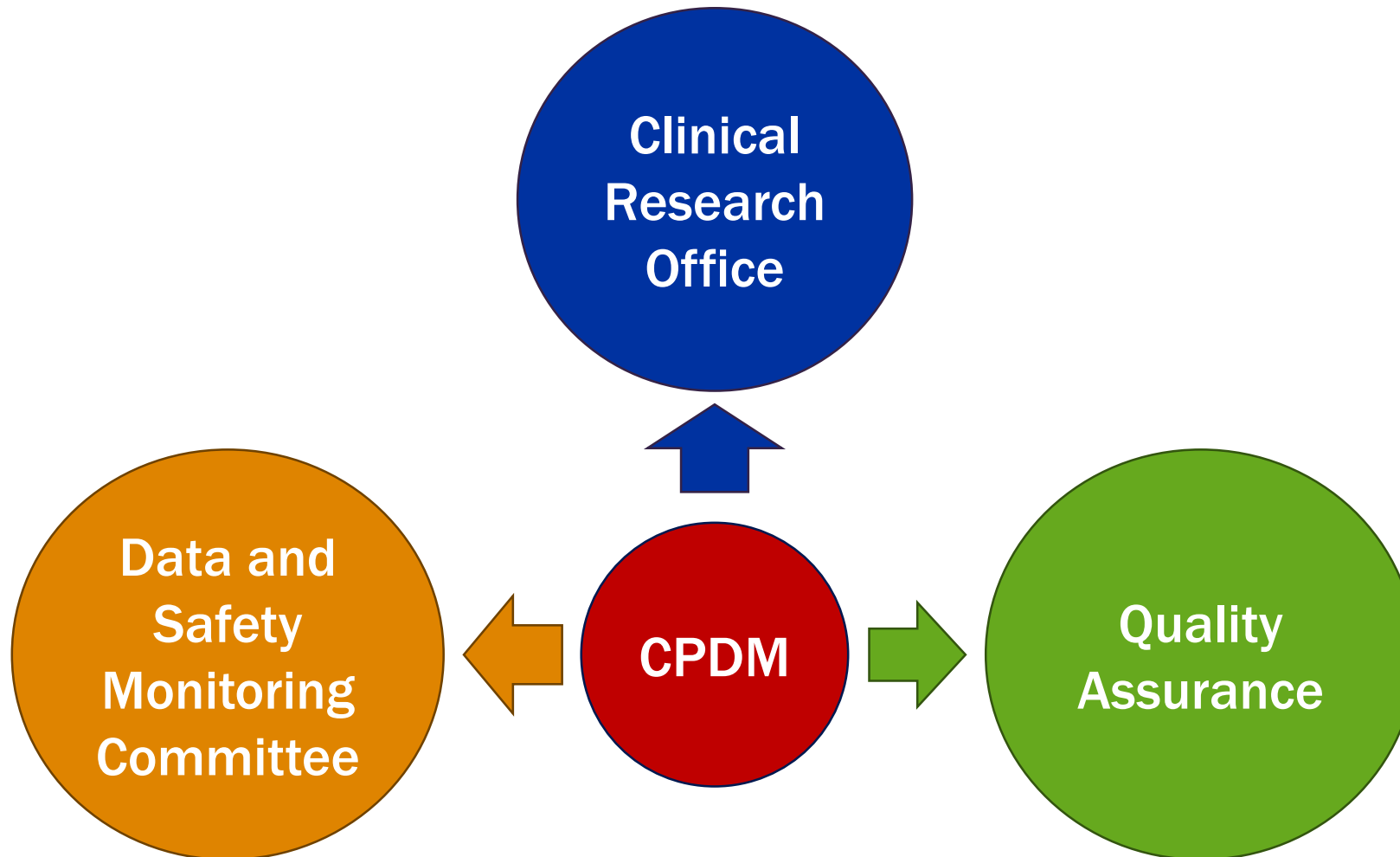
59% of all new cancer cases in Kentucky are directly or indirectly cared for by MCC

Markey At-A-Glance



- 120 members (73 research members)
- \$41.9M total cancer research funding (48% increase)
- 10,084 interventional accruals (44% Appalachian)
- >1,400 students, trainees and junior faculty mentored since 2013
- >5,700 health care professionals trained since 2013

MCC Clinical Protocol and Data Management



Primary goal:
Maintain high quality clinical cancer research

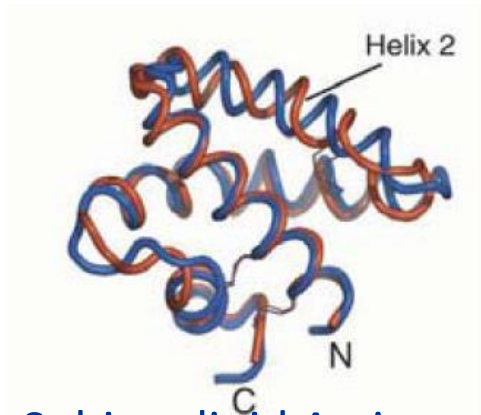
BXQ-350 CLINICAL TRIAL

- **Part 1: Dose escalation scheme**
 - Sequential cohort were treated with escalating doses until the maximum tolerated dose is established or the highest planned dose level (2.4 mg/kg) is reached
- **Part 2: BXQ-350 will be administered at maximum tolerated dose determined in part 1**
 - Primary objective: To assess preliminary antitumor activity

Current enrollment						
Consent signed	On study	On treatment	Off treatment	On follow up	Off study	Expired
11	11	11	7	0	3	2

BXQ-350 (“SapC-DOPS”)

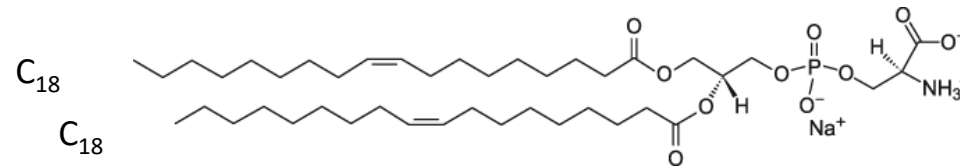
Protein



Sphingolipid Activator
Protein, Saposin C (SapC)

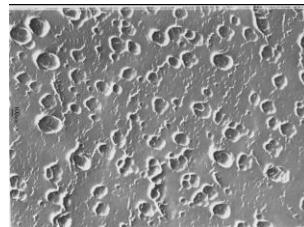
+

Lipid (aminophospholipid)



1,2-Dioleoyl-*sn*-Glycero-3-Phospho-L-
Serine (Sodium Salt) (DOPS)

=



BXQ-350 Protein/Lipid nanovesicle

Vesicle diameter, ca. 60 nm

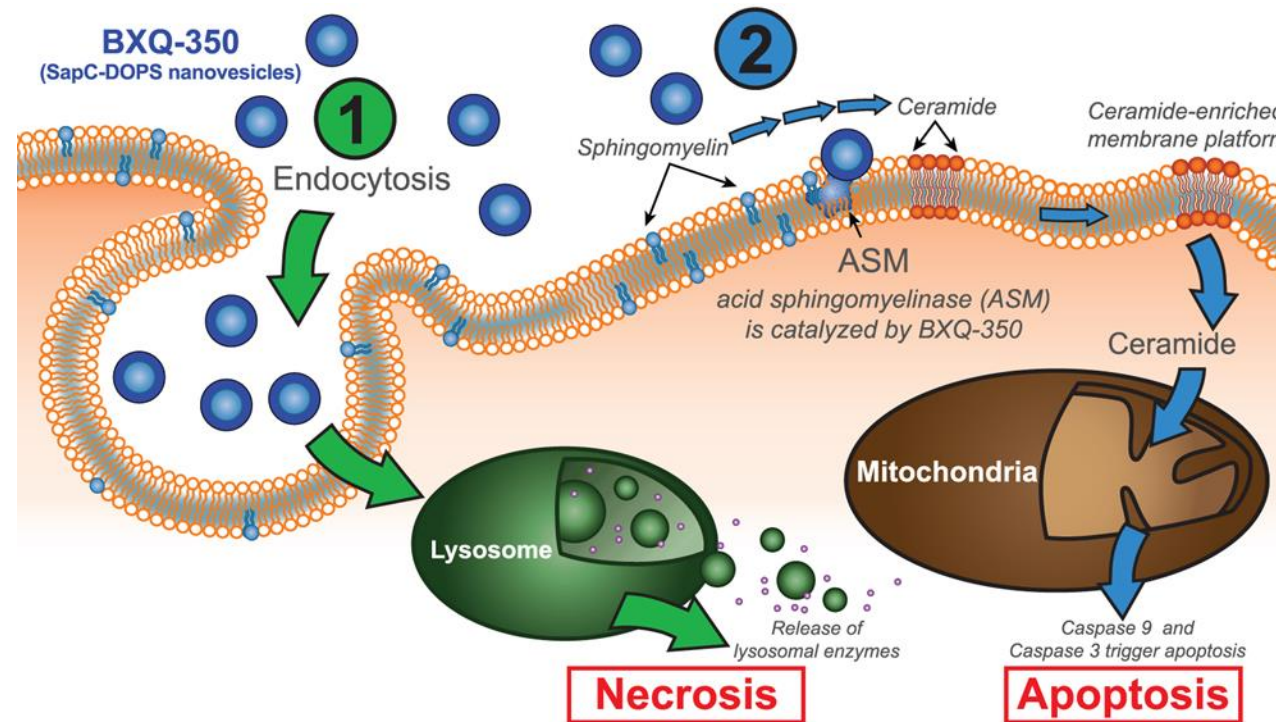


BXQ-350 Mechanism of Action

Two mechanisms of action have been characterized:

Mechanism #1 - Endocytosis of BXQ-350 leading to lysosomal degradation and necrosis

Mechanism #2 - Catalysis of acid sphingomyelinase leading to ceramide elevation and apoptosis



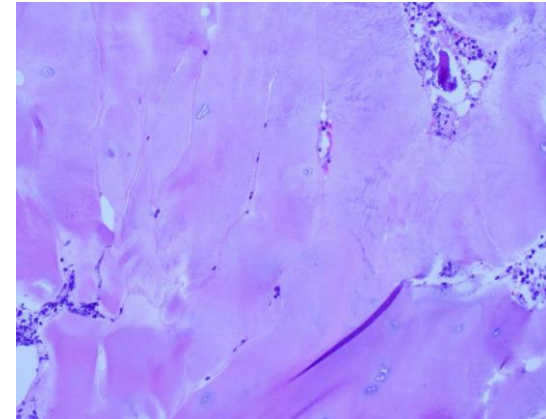
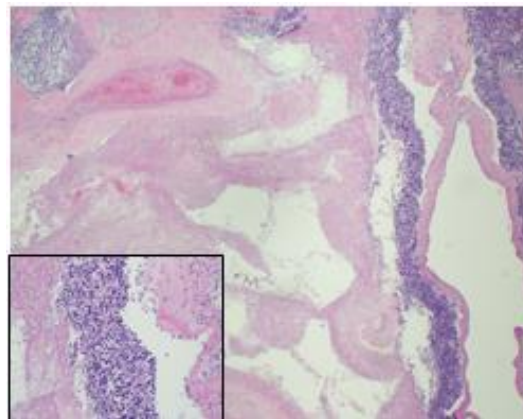
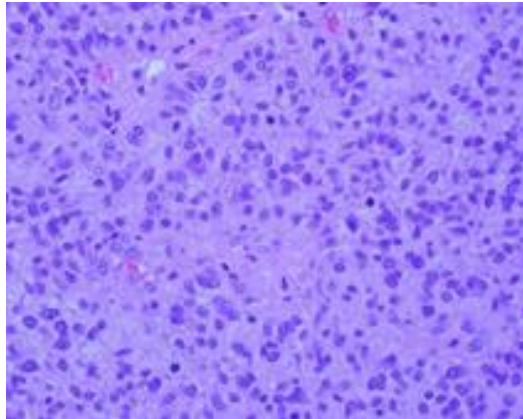
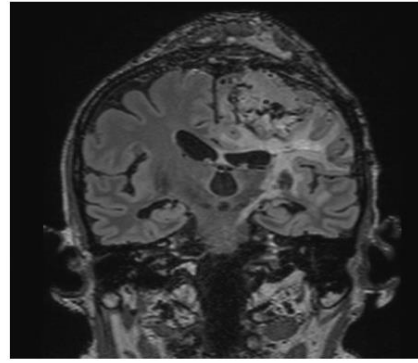
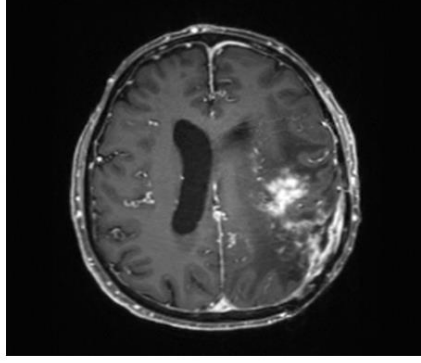
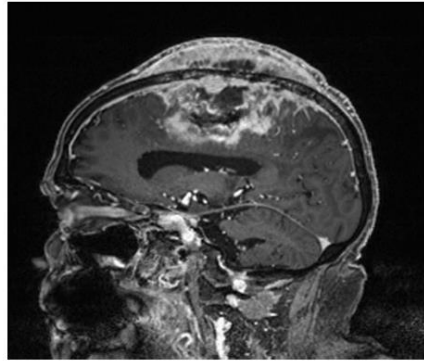
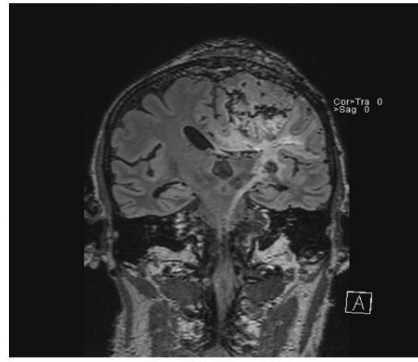
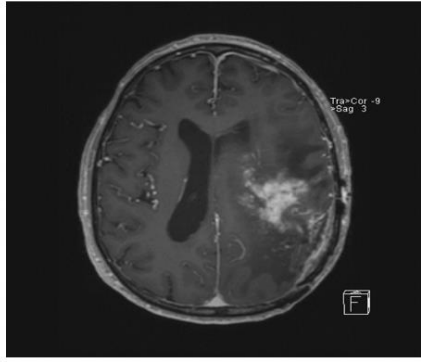
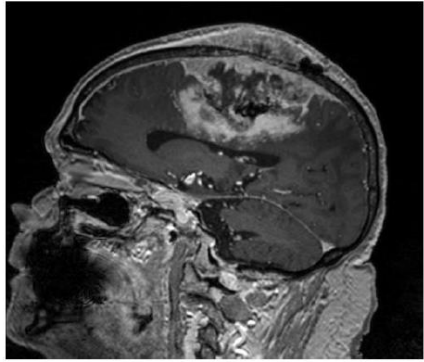
BXQ-350 for ependymoma

A 67 y/o gentleman with a history of prostate cancer was diagnosed in October 2014 with a left parietal anaplastic ependymoma.

He underwent gross total resection followed by adjuvant radiation. Repeat brain MRI in April 2017 demonstrated a local recurrence. He received 3 cycles of temozolomide but continued to progress.

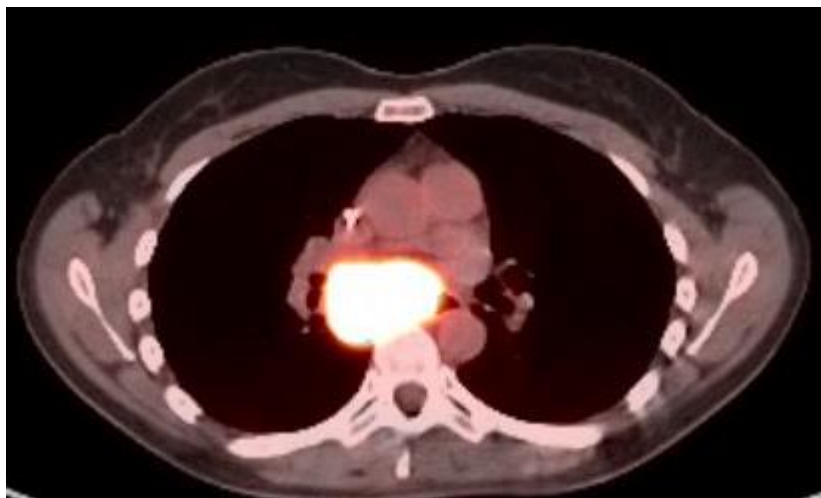
He was enrolled in BXQ-350 in September 2017.

The patient received C1 (BXQ-350 2/4 mg/kg IV infusion at day 1-5, 8, 10, 12,15,22 and 3 additional cycles (1x28 days), and was followed until death for safety, response, RANO, and ECOG.



Patient 1 at UK

October 2016

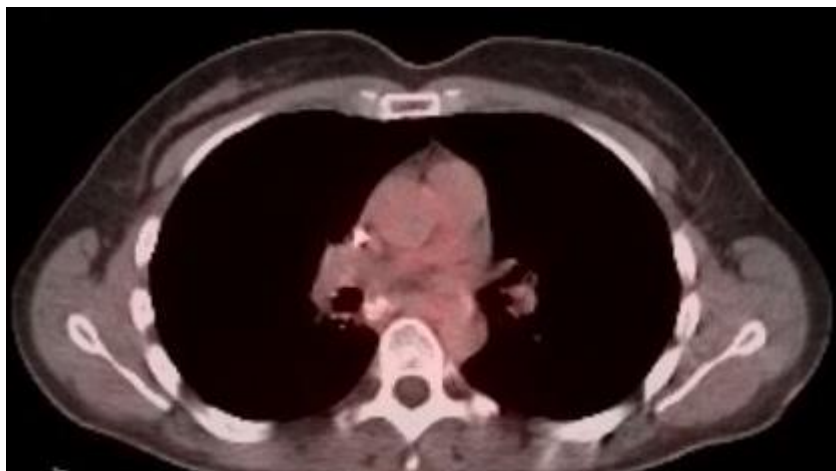


39 y/o woman with metastatic (stage IV) rectal adenocarcinoma

Enrolled in BXQ-350 in April 2017

She has received 20 cycles; currently NED

April 2018



Improving Cancer Care Across Kentucky



- Strong track record for Investigator-Initiated Trials
- Engagement of key populations
 - 68% of treatment intervention accruals are women
 - 65% of pediatric patients receive treatment on a COG trial
 - 54% of treatment intervention accruals are from Appalachia
- Comprehensive strategy for expansion of the Markey Cancer Center Network across the entire state and Central Appalachia