



Bexion Pharmaceuticals

Power by Nature; *Precision* by Science™

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Patient No. 1 and the Cancer Trial that Just Might Change Everything

THE CINCINNATI ENQUIRER
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SAVE UP TO

\$113

IN COUPON SAVINGS

Coordinators' plans were put in place in Bengals' win
Sports, 1C

Patient No. 1 and the cancer trial that may mean hope

BXQ-350, developed locally, helps man fight disease, keep tee time

Anne Sakor
Cincinnati Enquirer
USA TODAY NETWORK

Properly mixed, the medicine came out as a pale white to clear liquid. A nurse brought the intravenous bag to the side of a recliner, where a man with brain cancer sat. The nurse hung the plump bag on a stand and prepared the treatment for delivery. The moment had arrived for BXQ-350 to meet its first human patient.

For the man, and for the medicine, the stakes could not have been higher.

BXQ-350, by Bexion Pharmaceuticals of Covington, is a first-of-its-kind cancer treatment, a new approach discovered, developed and tested in Greater Cincinnati. The innovative drug is made from a human protein and does something unlike any other: It weaponizes the special mechanics of cancer to destroy it, without affecting healthy cells.

That the treatment killed nothing but cancer so jarred federal regulators that they had demanded more re-

See **CANCER**, Page 10A



Bob Rulli undergoes an infusion of the experimental drug BXQ-350, which lasted about an hour, July 10 at the University of Cincinnati's Barrett Cancer Center in Cincinnati. BXQ-350 is a first-of-its-kind cancer treatment, a new approach discovered, developed and tested in Greater Cincinnati. KAREEM ELGAZZAR/THE ENQUIRER

Bare-knuckle boxer escaped abuse at home
Box: Bawlings finds her happy place in the ring after surviving physical abuse at hands of her ex-husband. In Sports

NEWSLINE
Volcano calms down as Hawaii braces for Lane
Experts believe islands will avoid a natural-disaster double-whammy

IN MONEY
Would you pay \$60M for a used car?
1962 Ferrari GTO, one of just 26 made, is up for auction this weekend

IN LIFE
Great Lakes museum is deep dive into history
But Ohio attraction is much more than the wreck of the Edmund Fitzgerald

Ohio State drops the ball on Meyer
The sudden part of watching Urban Meyer's Hall of Fame coaching career devolve into a 23-page report that depicts him as a serial liar, sipping co-op wine and reckoning personal manager who provided a risky employee to that human being actually read what was on paper and concluded he should remain on the bench at Ohio State.

Patient No. 1 and the cancer trial that may mean hope
Bob, center, and Elise Rulli, far right, meet with doctors in preparation for Bob's procedure in Cincinnati. July, names are pseudonyms.

Trump team in high anxiety
Many still processing week's revelations

KILLING CANCER

Inside
The debate over cancer's new weapon

Overview of Bexion Pharmaceuticals

- Phase I oncology trials initiated in late 2016
- \$40MM+ invested (Series A and ongoing Series B)
- \$6MM+ in non-dilutive grants (SBIR, KY and others)
 - Phase II Bridge Award (Harold Varmus, MD – NCI Director)
 - Tibbett's Award
- Orphan Drug Status (GBM); others planned
- Planning Phase I pediatric trial in 2019
 - Rare Disease Voucher
- Planning Phase II GBM in 2019

Why Are We In Covington?

- Technology originated at Cincinnati Children's Hospital
- SBIR/STTR Matching Funds Program
 - \$1.1MM match to NCI funds of \$5MM funding
- Kentucky Enterprise Fund
 - \$400,000 in 2009
 - \$350,000 in 2018
- Reasonable cost of living
 - We built our own life sciences incubator
- Access to talent, but must grow
- Access to capital, but must grow

Phase 1a Dose-Escalation Trial Summary

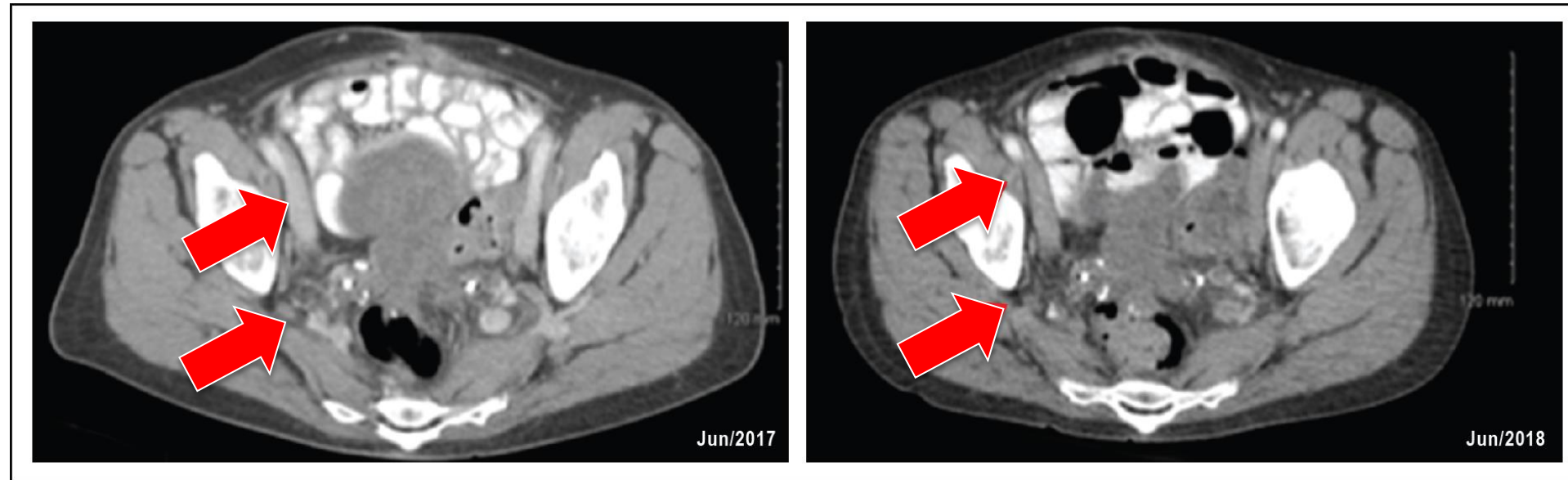
- 17 Patients (age 24-67)
- Median 7 (range 2-12) prior systemic therapies
- Completed median 2 (of up to 6 planned) cycles (2 cycles= 8 weeks)
- No DLTs
- No treatment-related serious adverse events (SAEs)
- Most common treatment-related moderate AEs were transient fatigue
- Three (3) patients are still on trial

Best response in 7 Pt completing to day 113 was 1 PR (appendiceal carcinoma) at 2.4 mg/kg, and 6 SD (1 HGG)

Pt at 0.7 mg/kg had stable disease >24+ months, and 6 had improved day 113 RANO/RECIST)

Case Data: Adenocarcinoma of the Appendix

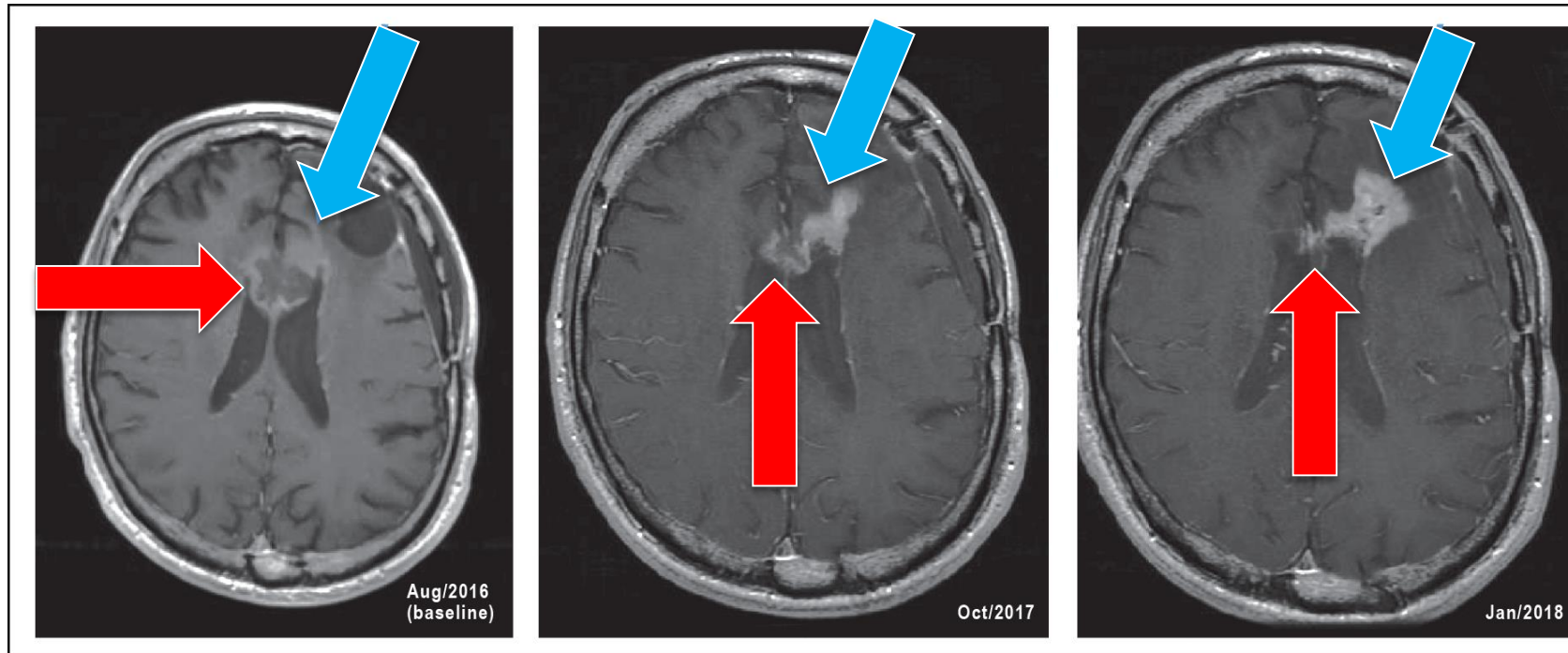
Axial CT in 62-yr old female with locally advanced mucinous adenocarcinoma of the appendix showing partial response after approximately 6 months of BXQ-350 2.4 mg/kg i.v. treatment, indicated by reduced hyperintense tumor margins apparent in CT imaging with biopsy confirmation. Partial Response was observed via improved RECIST score at Day 113, including changes in measurable lesions at the vaginal apex, anterior aspect of the small bowel loop, and lateral segment of the hepatic lobe.



Red Arrows indicate the initial target lesion that decreased in size while on study (Scan in Sept, 2018 shows continued reduction of lesion)

Case Data: Glioblastoma (GBM)

Axial T1-post contrast (Ax SE T1 POST FC) 1.5T MRI of the brain of a 64-yr old male patient with glioblastoma (GBM) treated with BXQ-350 i.v. 0.7 mg/kg in Phase 1a. GBM was first diagnosed in 2013 and previously treated with surgery, chemotherapy and radiation. The patient remained on study for approximately 1 year with stable disease, as assessed by RANO score, and completed all six cycles of part 1a of the study before being moved to the subsequent 2.4 mg/kg dose for subsequent cycles in Part 1b. Measurable lesion reduction over time is apparent in the anterior corpus callosum and frontal lobe, with those bordering the ventricle.



Red Arrows indicate the initial target lesion that decreased in size while on study

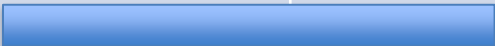

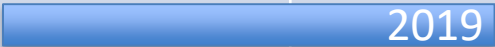
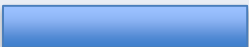
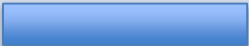


Blue Arrows indicate new area of enhancement (resection in May, 2018 revealed very few tumor cells)

Pipeline

SapC has receptor-mediated activities beyond its catalytic properties

Applications in Cancer and other therapeutic areas:

- *Orphan and Rare Tumors*
- *Lysosomal storage disease*
- *CNS diseases*

Drug	Indication	Preclinical	I	II	III
BXQ-350	GBM+Solid Tumors				
BXQ-350	GBM				
BXQ-350	Pediatric/GBM+Solid Tumors				
BXQ-350	Rare tumors				
BXQ-350	GI tumors				
BXQ-400	Lysosomal Storage Diseases		Opportunity for significant improvement of ERT		
	Undisclosed CNS		First-in-class for disease modification		
	Undisclosed CNS	Clinical evidence led opportunity			