

## **BioMed Valley Discoveries announces presentation of early clinical activity of first-in-class cancer therapy ulixertinib at 2017 ASCO annual meeting**

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Kansas City, MO. — BioMed Valley Discoveries announced today that new clinical research of first-in-class ERK-targeted cancer therapy ulixertinib (BVD-523) will be presented at the annual meeting of the [American Society of Clinical Oncology](#) (ASCO) June 2-6, 2017, in Chicago, Illinois.

Bob Li, MD, MPH, a medical oncologist at Memorial Sloan Kettering Cancer Center, will present scientific data from a multicenter Phase I/IIa clinical trial supporting further clinical development of ulixertinib. The clinical trial, sponsored by BioMed Valley Discoveries, was designed to assess the safety of ulixertinib and preliminary anti-tumor activity in patients with advanced solid tumors.

The research abstract was awarded "[Best of ASCO](#)" status, a designation reserved for studies that reflect the foremost research and strategies in oncology that will directly impact patient care.

Ulixertinib targets ERK, the terminal kinase of the mitogen-activated protein kinase (MAPK) signaling pathway, which is involved in cell growth, proliferation, and survival. Mutations in components of the MAPK pathway are present in more than a third of all human cancers.

"This is the first ERK inhibitor to advance this far in the clinic," said Dean Welsch, PhD, project leader and Executive Director of Translational Sciences and Head of Pharmacology at BioMed Valley Discoveries. "We have reached a key milestone in the development of this compound as a single agent. In the future, we plan to test this agent alone as well as in combination with drugs that target the same and other pathways."

The MAPK signaling pathway is one of the most well-studied pathways in human biology. The MAPK pathway acts like an intracellular relay race: the first component, RAS, activates RAF, which activates MEK, which activates ERK, which finally flips the switches that enable a cell to grow, proliferate, and survive. In many types of cancer, this race never ends, causing cells to grow out of control. Because of its importance in cancer, the MAPK pathway has been the focus of drug discovery for more than 15 years.

While no compounds have effectively targeted RAS in the clinic, drugs designed to inhibit RAF or MEK have improved clinical outcomes for metastatic melanoma and other tumor types. However, the majority of patients given these targeted therapies eventually develop resistance to the drugs and a reoccurrence of disease. Because ERK resides at the end of the relay, ERK inhibition may provide an effective treatment that is less susceptible to acquired drug resistance.

The first part of the clinical trial entailed a dose escalation study in 27 patients. This part established a recommended Phase II dose of 600 milligrams twice-daily. The second part of the trial involved a cohort expansion in 108 patients with specific mutations and tumor types, including *BRAF* or *NRAS* mutant melanoma and other *BRAF* or *MEK* mutant cancers.

"Previous work targeting the MAPK pathway has largely centered around the most common alteration to the *BRAF* gene known as the V600 mutation," said Welsch. "Our study was unique because we didn't just include patients who had that one mutation, but also those who harbored other atypical *BRAF* mutations that activate that same pathway. Our plan is to help patients whose tumors contain these mutations and who do not currently have any approved treatments. We are also excited to follow up this successful trial with additional ones testing our ERK inhibitor in combination with other agents."

Overall, the treatment appeared to be safe and well-tolerated. The most common adverse events were rash (49%), diarrhea (47%), fatigue (41%), and nausea (37%), side effects comparable to those seen with MEK inhibitors targeting an upstream part of the pathway.

In addition to three patients with partial responses (11%) to ulixertinib during dose escalation, an additional eleven partial responses (13%) were observed during the expansion part of the trial: one patient with melanoma resistant to BRAF/MEK inhibitors, three patients with *NRAS* mutant melanoma, two patients with *BRAF* mutant lung cancers, one patient with *BRAF* mutant glioblastoma multiforme, and four patients with atypical *BRAF* mutations.

BioMed Valley Discoveries already has several investigator-initiated trials underway to look at new indications. It also plans to assess whether ulixertinib may be effective in novel combination therapy regimens, and in specific genetic backgrounds that accompany acquired resistance.

Ulixertinib received FDA Fast Track designation in September 2015.

Members of BioMed Valley Discoveries will attend the ASCO meeting. To contact a company representative at the meeting about a scientific or business inquiry, email Dean Welsch at [erk@biomed-valley.com](mailto:erk@biomed-valley.com). The abstract (number 2508) is available online at [abstracts.asco.org](http://abstracts.asco.org).

### **About BioMed Valley Discoveries**

[BioMed Valley Discoveries](http://www.biomed-valley.com) is a clinical stage biotechnology company focused on addressing unmet medical needs in a variety of therapeutic and diagnostic areas. In addition to the ERK inhibitor, BVD's portfolio includes an oncolytic bacteria that has completed enrollment for Phase I, a selective phosphoinositide 3-kinase gamma inhibitor in late preclinical testing, and two early-stage antibodies targeting the tumor microenvironment.

Operating since 2007, BioMed Valley Discoveries was established by Jim Stowers Jr., founder of the asset management firm American Century Investments, and his wife Virginia, to advance new medical innovations to improve the lives of patients with difficult-to-treat diseases. BVD is owned by a supporting organization of the Stowers Institute for Medical Research, a non-profit, basic biomedical research organization. Profits from BioMed Valley Discoveries accrue to the benefit of the Stowers Institute.

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