

## **Niiki Pharma Announces Positive Interim Data from Ongoing Clinical Trial of Novel Anti-Cancer Agent NKP-1339**

Hoboken, NJ and Philadelphia, PA, September 20, 2011 – Niiki Pharma Inc. announced positive interim results from the ongoing Phase I clinical trial of its lead product, NKP-1339 in patients with metastatic solid tumors resistant to standard therapies. NKP-1339 is a first-in-class transferrin targeted small molecule that down-regulates GRP78, a key regulator of mis-folded protein processing and a tumor survival factor.

### **NKP-1339 Phase I Interim Data**

Previous to enrollment in the Phase I trial, all patients had received multiple standard therapies and exhibited disease progression on their last treatment. Of the first 24 patients enrolled, six patients (25%) exhibited anti-tumor activity, demonstrated by disease stability for at least 12 weeks and/or tumor regression. “We are pleased by the Phase I interim results demonstrating the single agent anti-tumor activity of NKP-1339 in heavily pretreated patients with advanced cancer for which there are no other treatment options,” said Dr. Angela Ogden, Chief Medical Officer for Niiki Pharma Inc.

One patient continues on NKP-1339 therapy after more than 70 weeks with shrinkage of a non-pancreatic neuroendocrine (non-pNET) “It is gratifying to see someone whose tumor has been resistant to other therapies do well with this promising investigational therapy,” said Dr. Howard Burris, M.D., F.A.C.P., Director of Drug Development at the Sarah Cannon Research Institute and trial investigator. “The patient has received NKP-1339 for more than a year and continues to benefit.”

Five other patients have experienced stable disease of up to 24 weeks with NKP-1339 treatment, including one with gastrinoma non-pNET, one with colorectal cancer, two with non-small cell lung cancer and one with cancer of unknown primary. “We need new treatments for patients with non-pNET,” said Dr. Von Hoff, Dr. Daniel Von Hoff, Translational Genomics Research Institute and a trial investigator. “The NKP-1339 anti-tumor activity observed in these two non-pNET patients is significant. This activity, together with mild-side effect profile, makes NKP-1339 a potentially promising new treatment for this disease.”

NKP-1339 treatment has been very well tolerated to date with mild manageable side effects. The most common drug-related side effects are grade 1-2 fever and mild flu-like symptoms, which can be prevented with standard medications. The maximum tolerated dose (MTD) has not been reached and NKP-1339 dose escalation continues.

### **About NKP-1339**

NKP-1339 is a first-in-class transferrin targeted ruthenium-based anti-cancer compound. Transferrin receptor is significantly overexpressed in many tumor types. The intracellular target pathways of NKP-1339 include down-regulation of GRP78, a key regulator of mis-folded protein processing and a tumor survival factor. GRP78 is the cause of resistance in many tumor types. In preclinical studies, NKP-1339 has demonstrated activity against multiple tumor types, including those resistant to other anti-cancer agents, including platinum-containing agent, anthracyclines and anti-tubulins.

NKP-1339 was invented by Professor Bernard K. Keppler, currently the Dean of the Faculty of Chemistry at University of Vienna, Austria, and Head of the Research Platform "Translational Cancer Therapy Research", a multi-disciplinary collaboration between University of Vienna and the Medical University of Vienna. Professor Kepler is a member of the Vienna Board of Trustees for innovative and interdisciplinary Cancer Research, the current President of the Austrian Association of University Professors, a founding member of the Central European Society of Anticancer-Drug Research, and the first Vice-Chairman of this Society. He is the recipient of several prestigious scientific awards.

“The preliminary results of this ongoing trial showing the anti-tumor activity and the safety profile of NKP-1339 supports our preclinical studies that the drug targets tumors selectively and is active

against different tumors,” said Professor Bernhard K. Keppler, University of Vienna. “We are delighted to see years of research come to fruition in collaboration with Niiki Pharma’s team.”

## **About the NKP-1339 Phase I Trial**

The NKP-1339 Phase I trial is a dose-ascending trial to determine the safety, tolerability, maximum tolerated dose, and pharmacokinetics of NKP-1339. The trial is being led by Senior Principal Investigators Dr. Daniel Von Hoff, Translational Genomics Research Institute (TGen), and Dr. Howard Burris, Sarah Cannon Research Institute (SCRI).

Dr. Daniel Von Hoff, M.D., F.A.C.P., is the Physician-in-Chief and Distinguished Professor at TGen in Phoenix, Arizona, and the Chief Scientific Officer for US Oncology and for Scottsdale Healthcare. He was appointed to and served six years on President Bush’s National Cancer Advisory Board and was past President of the American Association for Cancer Research (AACR,) a former Board member of the American Society of Clinical Oncology (ASCO), and the 2010 recipient of the David A. Karnofsky Memorial Award by ASCO for his outstanding achievements in cancer research.

Dr. Howard Burris, M.D., F.A.C.P., is the Director of Drug Development for SCRI, a Nashville, Tennessee, global clinical research organization focusing on advancing therapies and accelerating drug development in areas such as oncology, cardiology and gastroenterology and a member of Tennessee Oncology. He is also a member of the ASCO Ethics, Clinical Practice and Program Committees, Board member of Gilda's Club, Past-President and Trustee for the Southern Association of Oncology, and Editor of the Oncology Report.

## **About Niiki Pharma Inc.**

Niiki Pharma Inc. is an oncology therapeutics development company which was founded with the mission to acquire and develop targeted first-in-class treatments for cancer. The company is actively developing a pipeline of novel first-in-class anti-cancer agents. For more information, visit

## **Links**

Niiki Pharma Inc.  
Dr. Daniel Von Hoff, TGEN  
Dr. Howard Burris, SCRI  
Professor Bernhard K. Keppler, University of Vienna

[www.niikipharma.com](http://www.niikipharma.com)  
[www.tgen.org](http://www.tgen.org)  
[www.sarahcannonresearch.com](http://www.sarahcannonresearch.com)  
[www.anorg-chemie.univie.ac.at](http://www.anorg-chemie.univie.ac.at)

## **CONTACTS**

For media relations and other inquiries: Carolyn Rhinebarger, [carolyn@luminovas.com](mailto:carolyn@luminovas.com), +1-919-604-4053. For investor relations: Ali Ardakani, [ardakani@niikipharma.com](mailto:ardakani@niikipharma.com), +1-201-238-2885