



## **CHIMERIX ANTIVIRAL COMPOUND, CMX001, MEETS CMV PHASE 2 PRIMARY ENDPOINT IN ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANT RECIPIENTS**

### **Positive Phase 2 Study Results Presented in ‘Best Abstracts’ Oral Session at the 2012 BMT Tandem Meetings**

**RESEARCH TRIANGLE PARK, NC, February 6, 2012** – Chimerix, Inc., a biotechnology company developing novel antiviral therapeutics, today announced positive results from CMX001 Study 201, a Phase 2 study evaluating CMX001 for the prevention of cytomegalovirus (CMV) disease in hematopoietic stem cell transplant (HCT) recipients. CMX001 is a broad spectrum Lipid-Antiviral-Conjugate completing Phase 2 clinical development for the prevention of CMV in HCT recipients. In CMX001 Study 201, a double-blind, placebo-controlled trial which enrolled 230 HCT recipients, CMX001 had a statistically significant benefit versus placebo in preventing CMV viremia and/or CMV disease 13 weeks post-transplant.

Francisco Marty, MD, Assistant Professor of Medicine at Dana-Farber Cancer Institute and Brigham and Women’s Hospital’s Division of Infectious Disease, and a lead investigator in Chimerix’s CMX001 Phase 2 CMV study, presented the data during the “Best Abstracts Plenary Session” at the 2012 BMT Tandem Meetings on February 3, 2012 in San Diego, California. “This study provides positive data supporting the antiviral activity of CMX001 at different dose levels, and a better understanding of CMX001’s safety and tolerability as a prophylactic agent against CMV infection, a major cause of morbidity and mortality in bone marrow transplant recipients,” said Dr. Marty. “There is a substantial unmet medical need for safer and effective therapies against CMV. If approved, many patients have the potential to benefit from the future availability of CMX001.”

“These results exceeded our high expectations, and we are thrilled to share such positive CMX001 data with the transplant community,” said Wendy P. Painter, MD, MPH, Chimerix’s Chief Medical Officer. “We look forward to initiating the Phase 3 CMV program later this year. This study reinforces our belief that CMX001’s broad spectrum application against multiple viral infections, its safety profile and convenient oral dosing will enable it to become a new standard of care for transplant recipients.”

### **CMX001 Study 201 Results Presented at BMT Tandem Meetings**

Results from subjects receiving CMX001 100 mg twice weekly met the primary endpoint, a statistically significant reduction in CMV viremia (CMV > 200 copies/mL) or disease at the end of treatment in CMX001-treated subjects versus those who received placebo (p=0.001). Moreover, CMX001 Study 201 showed that three different doses of CMX001 demonstrated statistically significant reductions in the proportion of subjects with CMV viremia  $\geq$  1000 copies/mL at any time during treatment when compared to placebo (p=0.002, <0.001, <0.001, respectively; see Table 1 below). In subjects who were CMV viremia negative prior to treatment, four different CMX001 dose regimens demonstrated statistically significant reduction versus placebo (see Table 2 below).

**Table 1**  
**Subjects with Clinically Relevant CMV Viremia**  
**(≥ 1,000 copies/mL at any time during treatment)**

Dose	Enrolled (N)	CMV Viremia (N)	%	P
40 mg QW <sup>1</sup>	25	10	40%	0.43
100 mg QW	27	6	22%	0.06
200 mg QW	39	7	18%	0.002
200 mg BIW <sup>2</sup>	30	2	7%	< 0.001
100 mg BIW	50	4	8%	< 0.001
Pooled Placebo	59	25	42%	-

<sup>1</sup>QW: Once weekly. <sup>2</sup>BIW: Twice weekly.

**Table 2**  
**Subjects with Clinically Relevant CMV Viremia – CMV Negative Strata**  
**(≥ 1,000 copies/mL at any time during treatment)**

Dose	Enrolled (N)	CMV Viremia (N)	%	P
40 mg QW	18	4	22%	0.55
100 mg QW	23	2	9%	0.04
200 mg QW	29	2	7%	0.02
200 mg BIW	22	0	0	0.002
100 mg BIW	41	0	0	< 0.001
Pooled Placebo	48	15	31%	-

There was no difference versus placebo across CMX001 treatment groups in measurements of renal function and hematologic parameters. Diarrhea was the most common adverse event seen in the CMX001 treatment groups and was dose-limiting at the highest dose of CMX001 (200 mg twice weekly).

### **CMX001 Study 201 Design**

CMX001-201 was a randomized, double-blind, placebo-controlled, dose-escalation, multi-center trial evaluating the safety, tolerability, and ability of CMX001 to prevent or control CMV disease in 230 evaluable CMV seropositive allogeneic stem cell transplant recipients. Following engraftment (Days 14-30 post-transplant), subjects were stratified based on the presence or absence of acute GVHD requiring systemic therapy and the presence or absence of CMV DNA in plasma and randomized (3:1, CMX001 versus placebo) into five sequential, dose-escalating cohorts. Subjects were treated once weekly or twice weekly for 9 to 11 weeks through post-transplant Week 13, after which subjects were followed for an additional 4 to 8 weeks. Placebo patient results were pooled for endpoint analysis.

### **About CMX001**

CMX001 is a Lipid-Antiviral-Conjugate that delivers high intracellular levels of the active antiviral agent cidofovir-diphosphate and has broad spectrum in vitro activity against double-stranded DNA (dsDNA) viruses. CMX001 is completing Phase 2 clinical development for the prophylaxis of CMV and is in Phase 2 development for the preemption and treatment of adenovirus infection in HCT recipients. Antiviral activity results from completed and ongoing studies, coupled with the lack of myelotoxicity and

nephrotoxicity seen in currently available therapies, indicate that CMX001 has the potential to improve outcome for immunosuppressed patients.

To date, more than 700 patients have been dosed with CMX001 in placebo-controlled clinical trials and open-label treatment protocols. As part of Chimerix's open-label treatment protocols, data were recently presented at ICAAC 2011<sup>1</sup> in an oral presentation entitled "CMX001 is not nephrotoxic or myelosuppressive in 183 patients with life threatening dsDNA infections including refractory Cytomegalovirus, Adenovirus, and BK Virus".

### **About Cytomegalovirus**

CMV is a member of the herpesvirus group of dsDNA viruses. Like other herpesviruses, CMV has the ability to remain dormant in the body for long periods of time. In immunocompromised individuals, including transplant recipients, cancer patients and children born with primary CMV infection, CMV can lead to serious disease or death. At least 65% of transplant recipients are at moderate-to-high risk of CMV due to reactivation of latent virus from donor or recipient tissues. In these patients, CMV disease can lead to severe and potentially life-threatening conditions such as nephritis, pneumonitis or hepatitis, or complications such as acute or chronic rejection of a transplanted organ. While currently available systemic anti-CMV agents can be effective against the virus, their use is limited by significant toxicities, including myelotoxicity and nephrotoxicity.

### **About Chimerix**

Chimerix is developing novel antiviral therapeutics with the potential to transform patient care in multiple settings, including transplant, oncology, acute care and global health. Utilizing proprietary lipid conjugate technology, the company's two clinical stage compounds have demonstrated the potential for enhanced activity, bioavailability and safety compared to currently approved drugs.

In addition to the company's development of its lead candidate, CMX001, for transplant recipients, CMX001 is also being developed as a medical countermeasure in the event of a smallpox release, with the potential to provide an important therapeutic option for the 80 million people in the U.S. currently estimated to be immunocompromised, or a household contact of a contraindicated individual, and thus not candidates to receive a smallpox vaccine (for additional information, please see <http://www.bt.cdc.gov/agent/smallpox/vaccination/contraindications-clinic.asp>). Chimerix has received federal funding for the development of CMX001 as a medical countermeasure against smallpox from the National Institute of Allergy and Infectious Diseases under Grant No. U01-A1057233 and from the Biomedical Advanced Research and Development Authority (BARDA), Office of the Assistant Secretary for Preparedness and Response, Office of the Secretary, Department of Health and Human Services, under Contract No. HHSO100201100013C.

Chimerix's second clinical-stage antiviral compound, CMX157, is a Lipid-Antiviral-Conjugate that delivers high intracellular levels of the active antiviral agent tenofovir-diphosphate. CMX157 is in development as a potent nucleoside analogue against HIV and HBV infections, and has the potential to directly address several limitations of current therapies. CMX157 has completed a Phase 1 clinical trial in healthy volunteers, providing pharmacokinetic data which support the compound's enhanced characteristics.

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<sup>1</sup> Genovefa Papanicolau, MD, Associate Member of Infectious Diseases Service at Memorial Sloan-Kettering Cancer Center, at the 51<sup>st</sup> Interscience Conference on Antimicrobial Agents and Chemotherapy (ICAAC) Annual Meeting, 2011.

Led by an experienced antiviral drug development team, Chimerix is also leveraging its lipid conjugate technology and extensive chemical library to pursue new treatments for hepatitis C virus, influenza, and other areas of high unmet medical need. For additional information on Chimerix, please visit <http://www.chimerix.com>.

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