



**TRANSMITTED BY FACSIMILE**

Ms. Katie Cairati, M.S.  
Senior Director, Regulatory Affairs  
ChemGenex Pharmaceuticals  
4040 Campbell Avenue, Suite 200  
Menlo Park, CA 94025

**RE:** [REDACTED] (b) (4)  
Omapro™ (omacetaxine mepesuccinate) for Injection  
MACMIS # 19865

Dear Ms. Cairati:

As part of its monitoring and surveillance program, the Division of Drug Marketing, Advertising, and Communications (DDMAC) has reviewed a brochure for Omapro™ (omacetaxine mepesuccinate) for Injection (Omapro) obtained from the ChemGenex exhibit at the American Society for Hematology meeting in Orlando, Florida, in December 2010. This brochure, titled *New therapies for hematological malignancies* (brochure), contains false or misleading statements and promotional claims that represent that Omapro, an investigational new drug, is safe and effective for the purposes for which it is being investigated. As a result, this brochure misbrands Omapro in violation of the Federal Food, Drug, and Cosmetic Act (FD&C Act), 21 U.S.C. 352(a), and violates FDA's regulations, 21 CFR 312.6(b) and 312.7(a).

**Background**

Omapro is an investigational new drug that does not have marketing authorization in the United States. [REDACTED] (b) (4)

[REDACTED] ChemGenex issued a press release on April 12, 2010, addressing the complete response letter. ChemGenex's press release<sup>1</sup>, titled "ChemGenex Receives A Complete Response Letter From The FDA For OMAPRO™", stated that ". . .the principal issues raised by the FDA were similar to those discussed during the 22 March meeting of the Oncology Drug Advisory Committee" (ODAC) . . . ." According

<sup>1</sup> The ChemGenex Press Release announcing the FDA's complete response letter for Omapro is available at: <http://www.chemgenex.com/2010/04/chemgenex-receives-a-complete-response-letter-from-the-fda-for-omapro/> Accessed March 3, 2011.

to the ODAC meeting minutes,<sup>2</sup> (b) (4) discussed at the March 22 ODAC meeting were as follows: safety concerns regarding an overfilled vial size, a single, small (66 patients), and incomplete efficacy study (b) (4) a high number (35%) of ineligible patients were included in the efficacy study, the lack of a uniform in vitro diagnostic test for the Bcr-Abl T315I mutation created uncertainty about patient selection both in the efficacy study and (b) (4) and low response rates were observed in the efficacy study.

### Promotion of an Investigational Drug

Promotion of an investigational new drug is prohibited under FDA regulations at 21 CFR 312.7(a), which states that “A sponsor or investigator, or any person acting on behalf of a sponsor or investigator, shall not represent in a promotional context that an investigational new drug is safe or effective for the purposes for which it is under investigation or otherwise promote the drug. This provision is not intended to restrict the full exchange of scientific information concerning the drug, including dissemination of scientific findings in scientific or lay media. Rather, its intent is to restrict promotional claims of safety or effectiveness of the drug for a use for which it is under investigation and to preclude commercialization of the drug before it is approved for commercial distribution.” In addition, under 21 CFR 312.6(b), the “label or labeling of an investigational new drug shall not bear any statement that is false or misleading in any particular and shall not represent that the investigational new drug is safe or effective for the purposes for which it is being investigated.”

The brochure, titled *New therapies for hematological malignancies* contains claims such as the following that promote Omapro for the treatment of CML and other hematologic malignancies (emphasis original):

#### OMAPRO™ MODE OF ACTION

Protein synthesis is markedly up-regulated in malignant cells and high levels of many ‘short lived proteins’ (oncoproteins) . . . . Drugs that can interfere with the manufacture of these ‘short-lived’ proteins offer a new and unique way of attacking CML, particularly where TKIs have failed to have their desired effect.

OMAPRO™ works by inhibiting protein translation and is particularly active against a number of short lived proteins that are associated with CML, AML and multiple myeloma.

---

<sup>2</sup> The ODAC meeting minutes are available at <http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/%20OncologicDrugsAdvisoryCommittee/UCM207638.pdf> / Accessed March 3, 2011

## GROWING GLOBAL INTEREST IN OMAPRO™

As the positive OMAPRO™ data from clinical trials have become known, the demand for OMAPRO™ under a compassionate use/expanded access or a similar method has increased . . . .

*“Omacetaxine is a valuable option for the treatment of patients with CML, particularly those in two categories where we do not have any available treatment options. These are patients who have failed at least two prior tyrosine kinase inhibitors and those who have a mutation T315I, since we know that with this mutation none of the available tyrosine kinase inhibitors has activity. These results correspond well with the known activity of this compound in CML.”<sup>3</sup>*

The totality of these claims suggests that Omapro, an investigational new drug, is safe and effective for patients with CML when (b) (4) thus violating 21 CFR 312.6(b) and 312.7(a). (b) (4)

### False or Misleading Statements

The brochure also contains false or misleading statements that misbrand Omapro in violation of the FD&C Act, 21 U.S.C. 352(a), and violate FDA’s regulations, 21 CFR 312.6(b). (b) (4)

Therefore, in light of this information, ChemGenex’s dissemination of this brochure, touting the “positive OMAPRO data from clinical trials,” (b) (4) is false or misleading.

In addition, the brochure includes the following statement about expanded access to Omapro: “Meeting the needs of patients is a primary concern of ChemGenex, and the establishment of compassionate use access to OMAPRO™ has been a priority. Now established, this process is providing OMAPRO™ access to physicians and their patients around the world.” Expanded access (also known as compassionate use) is a regulatory pathway through which the FDA may allow patients with serious or immediately life-threatening diseases who have no comparable or satisfactory alternative treatment options to access investigational drugs outside of ongoing clinical trial investigations. See 21 CFR 312.300. We are not aware of any existing U.S. expanded access programs for Omapro. Therefore, the brochure, which

<sup>3</sup> In the brochure, this quote is attributed to “Dr. Jorge Cortes, MD, Professor of Medicine and Deputy Chair in the Department of Leukemia at The University of Texas, MD Anderson Cancer Center. Cancer Institute.”

was distributed at a meeting in the United States, is false or misleading in that it implies that there is an existing U.S. expanded access program for Omapro.

Moreover, expanded access is not intended as a means of circumventing or undermining the drug approval process, and sponsors of expanded access programs, like other sponsors, must comply with 21 CFR 312.6 and 312.7. Regardless of whether there is an expanded access program, ChemGenex, a sponsor, may not promote Omapro, an investigational drug, as safe and effective, or represent Omapro as safe and effective in its labeling. For the reasons noted above, the overall impression conveyed by the brochure misleadingly promotes the safety and efficacy of Omapro, (b) (4) and (b) (4)

(b) (4) (b) (4)

### Conclusion and Requested Action

For the reasons discussed above, the brochure misbrands Omapro in violation of the FD&C Act, 21 U.S.C. 352(a), and violates FDA's regulations, 21 CFR 312.6(b) and 312.7(a).

DDMAC requests that ChemGenex immediately cease the dissemination of violative promotional materials for Omapro such as those described above. Please submit a written response to this letter on or before May 12, 2011, stating whether you intend to comply with this request, listing all promotional materials for Omapro that contain violations such as those described above, and explaining your plan for discontinuing use of such violative materials. Please direct your response to me at the Food and Drug Administration, Center for Drug Evaluation and Research, Division of Drug Marketing, Advertising, and Communications, 5901-B Ammendale Road, Beltsville, MD 20705-1266, facsimile at 301-847-8444. In all future correspondence regarding this matter, please refer to MACMIS # 19865 in addition to the (b) (4). We remind you that only written communications are considered official.

The violations discussed in this letter do not necessarily constitute an exhaustive list. It is your responsibility to ensure that your promotional materials for Omapro comply with each applicable requirement of the FD&C Act and FDA implementing regulations.

Sincerely,

{See appended electronic signature page}

Adam George, Pharm.D.  
Regulatory Review Officer  
Division of Drug Marketing,  
Advertising, and Communications

-----  
**This is a representation of an electronic record that was signed electronically and this page is the manifestation of the electronic signature.**  
-----

/s/  
-----

ADAM GEORGE  
04/28/2011