College of Medicine
Department of Internal Medicine

Pulmonary and Critical Care Medicine
University of Cincinnati Medical Center
PO Box 670564
Cincinnati OH 45267-0564

Phone (513) 558-4831
Fax (513)558-4858

April 30, 2012

To whom it may concern:

I am writing to support the off label use of everolimus (Certican) for the treatment of patients with moderate to severe, progressive Lymphangioleiomyomatosis (LAM). LAM is a slowly progressive neoplasm of young and middle aged women that destroys the lungs. It occurs in patients with tuberous sclerosis complex and also in people who do not have a transmissable genetic disease.

I was the principal investigator on a double blind randomized clinical trial of the very closely related drug, sirolimus, in patients with moderately severe LAM. We found that sirolimus stabilizes lung function and improves quality of life. We participated in a open label, nonrandomized trial of everolimus in patients with LAM and, together with other trial investigators, found that the risks and benefits were very similar to those with sirolimus (attached). Lung function and six minute walk distance improved.

Everolimus is already FDA approved for two other indications in tuberous sclerosis patients, for the treatment of angiomyolipomas and for subependymal giant cell astrocytomas. I understand that siroilmus is difficult to obtain in Israel.

In the United States, many clinicians use sirolimus and everolimus interchangeably for the treatment of patients with LAM. I believe that the effects and side effects of these two drug are extremely similar, if not identical. Please consider approving the use of everolimus for the treatment of LAM. We believe that use of mTOR inhibitors such as sirolimus and everolimus can prevent lung function decline. It is imperative, therefore, that we provide access for LAM patients to this class of drugs as soon as possible.

Sincerely,

Francis X. McCormack, M.D.
Taylor Professor and Director
Pulmonary, Critical Care and Sleep Medicine

FXM:cpchAA49431